UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2025

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM TO**

Commission File Number 001-37471

Palvella Therapeutics, Inc.

(Exact name of Registrant as specified in its Charter)

Nevada	**30-0784346**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
353 W. Lancaster Ave, Suite 200	
Wayne, Pennsylvania	**19087**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (484) 253-1461

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	**PVLA**	**The Nasdaq Capital Market**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☐ NO ☒

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. YES ☐ NO ☒

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☒ NO ☐

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit such files). YES ☒ NO ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES ☐ NO ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant, based on the closing price of the Registrant's Common Stock, as reported on the Nasdaq Capital Market on June 30, 2025, was $196.7 million.

The number of shares of Registrant's Common Stock outstanding as of March 25, 2026 was 14,313,659.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant intends to file a definitive proxy statement pursuant to Regulation 14A within 120 days of the end of the fiscal year ended December 31, 2025. Portions of such definitive proxy statement are incorporated by reference into Part III of this Annual Report on Form 10-K.

TABLE OF CONTENTS

GENERAL INFORMATION

Unless otherwise stated or the context requires otherwise, references in this Annual Report on Form 10-K to "Palvella," the "company," the "Company," "we," "us," "our" or similar designations refer to Palvella Therapeutics, Inc. (formerly Pieris Pharmaceuticals, Inc.) and its subsidiaries, taken together. All trademarks, service marks, trade names and registered marks used in this report are trademarks, trade names or registered marks of their respective owners.

References to "Pieris" refer to Pieris Pharmaceuticals, Inc., our predecessor company prior to the Merger (as defined below) and references to "Legacy Palvella" or "Palvella" refer to Palvella Therapeutics, Inc. prior to the Merger and our wholly owned subsidiary upon the consummation of the Merger (as defined below).

On December 13, 2024 (the "Closing Date"), Palvella Therapeutics, Inc., a Nevada corporation (the "Company" or "Palvella") (previously named Pieris Pharmaceuticals, Inc. and our predecessor company ("Pieris")), consummated the previously announced merger pursuant to the terms of that certain Agreement and Plan of Merger, dated as of July 23, 2024 (the "Merger Agreement"), by and among the Company, Polo Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Pieris (the "Merger Sub"), and Palvella Therapeutics, Inc., a Delaware corporation ("Legacy Palvella"). Pursuant to the Merger Agreement, on the Closing Date, (i) Merger Sub merged with and into Legacy Palvella, with Legacy Palvella as the surviving company in the merger and, after giving effect to such merger, continuing as a wholly owned subsidiary of the Company (the "Merger") and (ii) the Company's name was changed from Pieris Pharmaceuticals, Inc. to Palvella Therapeutics, Inc.

Website addresses referenced in this Annual Report on Form 10-K are provided for convenience only, and the content on the referenced websites does not constitute a part of, and are specifically not incorporated by reference into, this Annual Report on Form 10-K.

Statements made in this Annual Report on Form 10-K concerning the contents of any agreement, contract or other document are summaries of such agreements, contracts or documents and are not complete descriptions of all their terms. If we filed any of these agreements, contracts or documents as exhibits to this Annual Report on Form 10-K or to any previous filing with the U.S. Securities and Exchange Commission ("SEC"), you may read the original document for a complete understanding of its terms.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K ("Form 10-K") and the documents we incorporate by reference contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements, other than statements of historical fact, included or incorporated in this report regarding, among other things, our management team's expectations, hopes, beliefs, intentions or strategies regarding the future, are forward-looking statements. In addition, any statements that refer to projections, forecasts or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking statements. The words "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intends," "may," "might," "plan," "possible," "potential," "predict," "project," "should," "will," "would" and similar expressions may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking.

Forward-looking statements contained in this Form 10-K include, but are not limited to, statements about:

- the strategies, prospects, plans, expectations and objectives of management of our future operations;
- the potential of, and expectations regarding our programs, including QTORIN™ rapamycin, QTORIN™ pitavastatin, and research-stage opportunities, including their expected therapeutic potential and market opportunity;
- the need to hire additional personnel and our ability to attract and retain such personnel;
- the ability to protect and enhance our products, proprietary technologies and intellectual property, including the extensions of existing patent terms where available, the validity of intellectual property rights held by third parties, and our ability not to infringe, misappropriate or otherwise violate any third-party intellectual property rights;
- developments and projections relating to our competitors or industry;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- our financial performance;
- expectations concerning our relationships and actions with third parties, including any licenses and collaborations with such third parties;
- future regulatory, judicial and legislative changes or uncertainties in our industry in the United States, Europe, and other jurisdictions, including due to uncertainty resulting from the recent change in U.S. administration and shift in government policy and evolving regulatory environment;
- the ability of our clinical trials to demonstrate safety and efficacy of our product candidates;
- our ability to utilize our proprietary drug development platform to develop a pipeline of product candidates to address unmet needs in rare skin disease and vascular malformation indications;
- the outcome of clinical trials of our product candidates, including the ability of those trials to satisfy relevant governmental and regulatory requirements;
- the timing of availability of data from our clinical trials;
- our plans to research, develop and commercialize our current and future product candidates;
- our reliance on contract manufacturers, contract research organizations and other third parties;
- our ability to develop and advance current product candidates and programs into clinical studies and to successfully complete those studies;

- our manufacturing, commercialization, and marketing capabilities and strategy;
- the size of the market opportunity for our product candidates, including estimates of the number of patients who suffer from the diseases we are targeting;
- expectations regarding potential for accelerated approval or other expedited regulatory designations;
- degree of market acceptance of our product candidates, as well as the pricing and reimbursement of our product candidates, if approved;
- our competitive position and the success of competing therapies that are or may become available;
- estimates of the number of patients that we will enroll in our clinical trials;
- the beneficial characteristics, and the potential safety, efficacy and therapeutic effects of our product candidates;
- our ability to obtain and maintain regulatory approval of our product candidates and our expectations regarding various types of therapy;
- the potential impact of healthcare reform in the U.S., including the Inflation Reduction Act of 2022, and measures being taken worldwide designed to reduce healthcare costs; and
- the volatility of capital markets and other macroeconomic factors, including those due to inflationary pressures, tariffs and other trade restrictions or the threat of such actions, interest rate and currency rate fluctuations, economic slowdown or recession, banking instability, monetary policy changes, geopolitical tensions or the outbreak of hostilities or war, including from the ongoing Russia-Ukraine war, the current conflicts in Venezuela and the Middle East (including any escalation or expansion) and increasing tensions between China and Taiwan.

There are a number of important factors that could cause our actual results to differ materially from those indicated or implied by forward-looking statements. These important factors include those set forth below under Part I, Item 1A “Risk Factors” and in our other disclosures and filings with the SEC. These factors and the other cautionary statements made in this Form 10-K and the documents we incorporate by reference should be read as being applicable to all related forward-looking statements whenever they appear in this Form 10-K and the documents we incorporate by reference.

In addition, any forward-looking statements represent our estimates only as of the date that this Form 10-K is filed with the SEC and should not be relied upon as representing our estimates as of any subsequent date. All forward-looking statements included in this Form 10-K are made as of the date hereof and are expressly qualified in their entirety by this cautionary notice. We disclaim any intention or obligation to update or revise any forward-looking statement, whether as a result of new information, future events, or otherwise, except as may be required by law.

SUMMARY OF PRINCIPAL RISK FACTORS

Below is a summary of material factors that make an investment in our common stock speculative or risky. Importantly, this summary does not address all the risks and uncertainties that we face. Additional discussion of the risks and uncertainties summarized in this risk factor summary, as well as other risks and uncertainties that we face, can be found within Part I, Item 1A, "Risk Factors" in this Annual Report on Form 10-K. The below summary is qualified in its entirety by those more complete discussions of such risks and uncertainties. You should consider carefully the risks and uncertainties described under Part I, Item 1A, "Risk Factors" in this Annual Report on Form 10-K as part of your evaluation of an investment in our common stock.

- We have historically incurred significant operating losses and anticipate that we will continue to incur significant operating losses for at least the next several years. We may never achieve or maintain profitability.
- We have never generated revenue from product sales and may never achieve or maintain profitability.
- We will likely require additional funding to finance our operations, which may cause dilution to our stockholders. Failure to obtain this necessary funding when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development, commercialization efforts or other operations.
- Our future success is substantially dependent on the successful clinical development, regulatory approval and commercialization of QTORIN rapamycin, which is in later stages of development than our other product candidates.
- We may be unable to obtain regulatory approval for our product candidates under applicable regulatory requirements. The denial or delay of any such approval would adversely impact our potential to generate revenue, our business and our results of operations.
- The rare skin diseases and vascular malformations we are currently targeting have no U.S. Food and Drug Administration ("FDA")-approved therapies, which subjects the design and execution of our clinical development programs to complexities and known as well as unknown risks, including those related to novel and/or subjective clinical endpoints and varying patient population characteristics.
- Our lead product candidates are based on our QTORIN platform and we are consequentially highly dependent on the successful development of this novel and unproven technology.
- Orphan Drug Designation has been granted for QTORIN rapamycin for the treatment of microcystic lymphatic malformation (microcystic LM), but we may be unable to obtain such designation for other product candidates or to realize the benefits of Orphan Drug Designation, including potential marketing exclusivity, even if such designation is obtained.
- Our development and commercialization strategy for our product candidates depends, in part, on published scientific literature and the FDA's prior findings regarding the safety and efficacy of rapamycin, pitavastatin, and other planned or future product candidates. If we are not able to pursue this strategy, we may be delayed in receiving regulatory authority approval.
- Breakthrough Therapy Designation has been granted for QTORIN rapamycin for the treatment of microcystic LM but we may never be able to realize the benefits of such designation for QTORIN rapamycin or any other indications or future product candidates, if granted, and such designation may not lead to a faster development, regulatory review or approval process or increase the likelihood that our product candidates will receive marketing approval.
- Even if QTORIN rapamycin, QTORIN pitavastatin or any future product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.
- We currently rely on contract manufacturer organizations ("CMOs") to manufacture preclinical and clinical supplies of our product candidates and expect to rely on CMOs for the commercial supplies of any approved

product candidates. The loss of these manufacturers, or their failure to provide us with sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.

- We may not be able to obtain, maintain or enforce patent rights that cover our product candidates and technologies that are of sufficient breadth to prevent third parties from competing against us.

PART I

Item 1. Business.

On December 13, 2024, we completed the previously announced business combination with Legacy Palvella in accordance with the terms of the Merger Agreement, pursuant to which, among other matters, Merger Sub merged with and into Legacy Palvella, with Legacy Palvella surviving as our wholly owned subsidiary (such business combination, the Merger). In connection with the completion of the Merger, we changed our name from "Pieris Pharmaceuticals, Inc." to "Palvella Therapeutics, Inc.," and our business became primarily the business conducted by Legacy Palvella. We are now a clinical-stage biopharmaceutical company focused on developing and, if approved, commercializing novel therapies to treat patients suffering from serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies.

Unless the context indicates otherwise, references in this section to the "Company," "we," "our," "us" and "Palvella" refer, collectively, to Palvella Therapeutics, Inc. and its consolidated subsidiaries following completion of the Merger.

Overview

We are a clinical-stage biopharmaceutical company whose vision is to become the leading rare disease biopharmaceutical company focused on developing and, if approved, commercializing novel therapies to treat patients suffering from serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies. We envision a future treatment paradigm in which individuals suffering from serious, rare skin diseases and vascular malformations, and the physicians treating those diseases, have significantly improved treatment options which address the underlying causes of those diseases. We intend to leverage our versatile QTORIN platform to minimize the challenges and timelines typically associated with generating novel topical product candidates. The QTORIN platform is specifically designed to reproducibly generate novel topical product candidates that penetrate the deep layers of the skin to locally treat a broad spectrum of rare skin diseases and vascular malformations. Our lead product candidate, QTORIN 3.9% rapamycin anhydrous gel ("QTORIN rapamycin"), is currently in clinical development for microcystic lymphatic malformations ("microcystic LMs") and cutaneous venous malformations ("cutaneous VMs"). QTORIN rapamycin contains the active pharmaceutical ingredient ("API") rapamycin, also known as sirolimus, which is an inhibitor of mTOR, a kinase that has been known to play a key role in cell growth and proliferation.

In February 2026, we announced positive topline results from SELVA, a Phase 3, single-arm, baseline-controlled study, which evaluated the safety and efficacy of QTORIN rapamycin for the treatment of microcystic LMs in patients 3 years and older. The study met the pre-specified primary endpoint, the mLM Investigator Global Assessment ("mLM-IGA"), with a +2.13 ($p<0.001$) improvement. The study also met its pre-specified key secondary and all four additional secondary endpoints with statistical significance (all $p<0.001$). In December 2025, we announced positive topline efficacy results from TOIVA, a Phase 2, single-arm, baseline-controlled study, which evaluated the safety and efficacy of QTORIN rapamycin for the treatment of cutaneous VMs in patients 6 years and older, which achieved nominal statistical significance ($p<0.001$) on multiple pre-specified clinician-reported and patient-reported efficacy endpoints, including dynamic change endpoints and static severity endpoints at Week 12.

In September 2025, we announced the expansion of our QTORIN rapamycin development program into clinically significant angiokeratomas.

In November 2025, we announced a new QTORIN product candidate, QTORIN pitavastatin, for the treatment of disseminated superficial actinic porokeratosis. QTORIN pitavastatin leverages our proprietary QTORIN platform and is designed to be the first pathogenesis-directed therapy for disseminated superficial actinic porokeratosis
("DSAP") by directly inhibiting the causal mevalonate pathway.

QTORIN Rapamycin for the Treatment of Microcystic LMs

Microcystic LM is a serious, chronically debilitating, and lifelong genetic disease of the lymphatic system characterized by lymphorrhea and acute cellulitis. It is estimated that there are more than 30,000 diagnosed patients in the United States with microcystic LMs. The specific pathophysiology of microcystic LMs is primarily the result of somatic activating mutations in the PIK3CA pathway that result in increased activation of the PI3K/mTOR pathway and subsequent lymphatic hyperplasia. Because microcystic LMs have a well-understood pathophysiology and a well-defined disease course, we believe an appropriate clinical study for this rare disease is a baseline-controlled study using clinician assessments.

We recently completed the SELVA trial, a Phase 3, single-arm, baseline-controlled clinical trial evaluating once-daily QTORIN rapamycin in individuals aged ≥ 3 years with microcystic LMs. Of the 51 participants enrolled, 50 initiated treatment, including 49 participants aged ≥ 6 years and 1 participant in the exploratory 3- to 5-year-old cohort. In accordance with the statistical analysis plan, efficacy results were reported for participants aged ≥ 6 years, which constituted the Intent-to-Treat ("ITT") population. The study was originally designed to enroll 40 participants across leading U.S. vascular anomaly centers and exceeded its target enrollment.

The primary endpoint, the mLM-IGA, is a 7-point clinician-assessed dynamic scale measuring change in disease severity from baseline ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the mLM-IGA at Week 24 in the ITT population (n=49), QTORIN rapamycin demonstrated a mean improvement of +2.13 points, meeting the study's primary endpoint ($p<0.001$). Of the participants aged ≥ 6 who completed the efficacy evaluation period, 95% (41/43) demonstrated at least a 1-point improvement, and 86% (37/43) were either "Much Improved" (+2) or "Very Much Improved" (+3). In the 3- to 5-year-old cohort, one participant enrolled and was "Very Much Improved" (+3) on the mLM-IGA at Week 24.

The key secondary endpoint, the blinded mLM Multi-Component Static Scale ("mLM-MCSS"), a clinician-assessed static scale scored as the total of three sub-scales (minimum score: 3; maximum score: 15) capturing lesion height, leaking/bleeding, and vesicle appearance, improved by a mean of 3.36 points ($p<0.001$), based on a blinded independent review of randomized Baseline and Week 24 photographs evaluated by a committee of clinician experts.

Similar to previous clinical trials of QTORIN rapamycin, in the Phase 3 SELVA study, QTORIN rapamycin was well-tolerated. Amongst the 50 participants who initiated treatment, 35 participants (70%) experienced treatment-emergent adverse events ("TEAEs"). Four experienced serious adverse events, of which one experienced a severe TEAE; all were deemed unrelated to study drug by investigators. Amongst the TEAEs, a total of 17 participants experienced treatment-related adverse events ("TRAEs"), all of which were rated mild or moderate. The most common TRAEs included application site acne, application site discoloration, and application site pruritus (all n=3, 6%). Rapamycin levels were below 2 ng/mL in systemic circulation for all participants at all timepoints in the study.

We previously announced topline Phase 2 clinical trial results from our multi-center, open-label, baseline-controlled study of 12 subjects receiving QTORIN rapamycin administered once daily for 12 weeks for the treatment of microcystic LMs. The Phase 2 clinical trial featured multiple pre-specified efficacy assessments, including clinician and patient global impression assessments as well as assessments of individual clinical manifestations that are important disease burdens for individuals living with microcystic LMs. All participants in the Phase 2 clinical trial demonstrated improvements on the Clinician Global Impression of Change ("CGI-C") scale, a 7-point clinician-rated change scale, with all participants in the study rated as either "Much Improved" (n=7, 58%) or "Very Much Improved" (n=5, 42%) after 12-weeks of treatment compared to the pre-treatment baseline period.

In the first quarter of 2026, we submitted a pre-NDA meeting request to the FDA. We anticipate the meeting to occur during the second quarter of 2026. We have received Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation from the FDA for QTORIN rapamycin for the treatment of microcystic LMs. In addition, we have been awarded an FDA Orphan Products Clinical Trials Grant for up to $2.6 million supporting the SELVA Phase 3 study and have received $1.1 million of proceeds to date.

QTORIN Rapamycin for the Treatment of Cutaneous VMs

Cutaneous VM is a serious disease with a high unmet need characterized by dysregulated growth of malformed veins impacting the skin, causing functional impairment and deformity. It is estimated that there are more than 75,000 diagnosed patients in the United States with cutaneous VMs.

In December 2025, we announced positive topline results from TOIVA, a multicenter, single-arm, open-label, baseline-controlled, Phase 2 clinical trial designed to evaluate the safety and efficacy of QTORIN rapamycin for the treatment of cutaneous VMs. The study enrolled 16 participants ≥ 6 years at leading vascular anomaly centers across the U.S. Key findings from the study's pre-specified efficacy endpoints at Week 12 demonstrated nominally statistically significant ($p<0.001$) improvements at Week 12 on several of the clinically relevant and important efficacy endpoints evaluated when compared to pre-treatment (baseline), including many of the static and impression of change global instruments evaluated, including the Overall Cutaneous VM Investigator Global Assessment ("Overall cVM-IGA") (Table 5). The Overall cVM-IGA is a 7-point, clinician-assessed, single-item efficacy endpoint measuring change in severity from baseline, with the numeric rating scale ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the Overall cVM-IGA at Week 12, 73% (11/15) participants improved, with 67% (10/15) either "Much Improved" (+2) or "Very Much Improved" (+3). No trial participants (0/15) were "Minimally Worse" (-1), "Much Worse" (-2), or "Very Much Worse" (-3).

Similar to previous clinical trials of QTORIN rapamycin, in the Phase 2 TOIVA study QTORIN rapamycin was generally well-tolerated, with the most common TEAEs being application site reactions (erythema, 25%). All TRAEs were moderate or mild, with no unexpected adverse events reported. Rapamycin levels were below 2 ng/mL in systemic circulation for all participants at all timepoints in the study.

In January 2026, we completed a Preliminary Breakthrough Therapy Designation Advice meeting with the FDA. Based on that meeting, our intent is to submit an application to the FDA for Breakthrough Therapy Designation in the second quarter of 2026. We plan to commence a Phase 3 pivotal study in the second half of 2026. We have received Fast Track Designation from the FDA for QTORIN rapamycin for the treatment of cutaneous VMs.

QTORIN Rapamycin for the Treatment of Clinically Significant Angiokeratomas

In September 2025, we announced the expansion of our QTORIN rapamycin development program into treating clinically significant angiokeratomas. No FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.

Clinically significant angiokeratomas are superficial vascular malformations of lymphatic origin which can cause bleeding, pain, functional impairment, and risk of infection, with no tendency for spontaneous regression. Angiokeratomas were recently classified as an isolated lymphatic malformation in 2025 by the International Society for the Study of Vascular Anomalies ("ISSVA"). Current treatment options include potentially destructive procedural interventions that carry significant risks of pain, scarring, and recurrence. Despite the substantial disease burden, there are currently no FDA-approved treatments available for clinically significant angiokeratomas.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study of approximately 10-20 patients to evaluate QTORIN rapamycin for the treatment of clinically significant angiokeratomas. Study initiation is anticipated in the second quarter of 2026. We received Fast Track Designation for QTORIN rapamycin for the treatment of angiokeratomas.

There are no FDA-approved therapies currently indicated for microcystic LMs, cutaneous VMs, or clinically significant angiokeratomas. If approved for the treatment of microcystic LMs, cutaneous VMs, or clinically significant angiokeratomas, we believe QTORIN rapamycin has the potential to become first line therapy and the standard of care for these indications.

Issued Patents and Patent Applications for Anhydrous Gel Formulations of Rapamycin

We have multiple patents and patent applications directed to anhydrous gel formulations of rapamycin, including QTORIN rapamycin, and the use of such anhydrous gel formulations for the treatment of certain skin disorders, including microcystic LMs and cutaneous VMs. Our issued U.S. patents with claims directed to certain anhydrous gel

formulations containing rapamycin and methods of treatment expire in 2038 and, for certain applications, if issued, as late as 2042.

QTORIN Pitavastatin for the Treatment of Disseminated Superficial Actinic Porokeratosis

In November 2025, we announced a new product candidate, QTORIN pitavastatin, for the treatment of DSAP. QTORIN pitavastatin was developed leveraging the QTORIN platform.

DSAP is a premalignant genetic skin disease that presents as persistent, often extensive lesions that enlarge and increase in size, number, and extent over time, causing chronic loss of skin integrity which can severely impact quality-of-life. No FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study to evaluate QTORIN pitavastatin for the treatment of DSAP. Trial initiation is anticipated in the second half of 2026.

Additional Preclinical Programs

We also have additional preclinical research programs based on our QTORIN platform for the treatment of serious, rare skin diseases and vascular malformations for which we believe there are significant unmet needs. As we plan to expand our pipeline into additional serious, rare skin diseases and vascular malformations, we plan to generate new product candidates with our QTORIN platform.

Our Vision and Approach

Our vision, supported by our mission of serving patients, is to become the leading rare disease biopharmaceutical company focused on developing and, if approved, commercializing novel therapies for serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies. We envision a future treatment paradigm in which individuals suffering from serious, rare skin diseases and vascular malformations, and the physicians treating those diseases, have significantly improved treatment options which address the underlying causes of those diseases. The core components of our approach include the following:

- *supported by our pipeline, build upon our experience in serious, rare skin diseases and vascular malformations through indication expansion and the generation of new product candidates.* We believe serious, rare skin diseases and vascular malformations represent a substantial opportunity to develop and, if approved, commercialize first-in-disease therapies. More than 98% of the reported 597 rare skin diseases do not have a therapy approved by the FDA. Our goal is to select diseases with a well-understood etiology, pathophysiology and a strong rationale for a specific pathway intervention. Many of these diseases have a debilitating, lifelong impact on individual lives. In addition to exploring other selected diseases for which QTORIN rapamycin could provide an effective therapy, we are investigating several new product candidates for additional serious, rare skin diseases and vascular malformations;

- *maximize the potential of the QTORIN platform across a wide range of molecules.* To date, we have developed QTORIN rapamycin and QTORIN pitavastatin, which we believe have broad clinical potential for several serious, rare skin diseases and vascular malformations currently without FDA-approved therapies. We intend to further leverage our scalable QTORIN platform to generate additional product candidates that target the known causes of serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies; and

- *forge meaningful patient and physician collaborations.* A key element of our approach is to take a rigorous, systematic approach to understanding the disease of the patient populations that we are addressing. A foundational pillar of this approach is to forge and maintain meaningful collaborations with physicians and disease advocacy organizations. Through this engagement, valuable learnings inform the selection and development of efficacy endpoints and what constitutes clinical meaningfulness and acceptable risk-benefit.

We believe these learnings significantly inform our product development approach, which may contribute to the regulatory acceptability of our product candidates.

Our Strategy

To achieve our vision, the key elements of our strategy include:

- *successfully develop and, if approved, commercialize QTORIN rapamycin for the treatment of microcystic LM, cutaneous VMs, clinically significant angiokeratomas and other rare skin diseases and vascular malformations and QTORIN pitavastatin for the treatment of DSAP.* QTORIN rapamycin is in clinical development for microcystic LMs and cutaneous VMs, and we plan to initiate a Phase 2 clinical study in clinically significant angiokeratomas in the second quarter of 2026. QTORIN pitavastatin is in development for DSAP. There are no FDA-approved therapies for these specific indications, and, if approved, we believe QTORIN rapamycin and QTORIN pitavastatin have the potential to become first line therapy and the standard of care. We reported topline data from our Phase 3 SELVA trial in microcystic LMs in February 2026 and plan to request FDA agreement to begin a rolling submission of a Section 505(b)(2) New Drug Application ("NDA") for QTORIN rapamycin for the treatment of microcystic LMs in the second half of 2026. In December 2025, we announced positive topline efficacy results from TOIVA, a multicenter, single-arm, open-label, baseline-controlled, Phase 2 clinical trial designed to evaluate the safety and efficacy of QTORIN rapamycin for the treatment of cutaneous VMs. Given there is a growing body of real-world evidence that rapamycin has the potential to treat a broad number of cutaneous diseases, we plan to evaluate QTORIN rapamycin in other serious, rare skin diseases and vascular malformations. We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study to evaluate QTORIN pitavastatin for the treatment of DSAP, with study initiation expected in the second half of 2026;

- *build an independent commercial organization to commercialize, if approved, our skin disease and vascular malformation therapies in the United States.* If, in the United States, QTORIN rapamycin is approved for the treatment of microcystic LM, cutaneous VMs, and/or clinically significant angiokeratomas or QTORIN pitavastatin is approved for the treatment of DSAP, we intend to independently commercialize QTORIN rapamycin and/or QTORIN pitavastatin by building a focused specialty sales force that will target vascular anomaly centers, dermatologists, or other specialists who cover a meaningful percentage of patients being treated. We expect this will provide us with a significant competitive advantage in addition to providing future operational leverage. Outside of the United States, we may consider building our own commercial infrastructure or out-licensing where appropriate. We may elect to utilize strategic collaborators, distributors or other partners to assist in the commercialization of our products candidates, if approved;

- *evaluate the potential of the QTORIN platform to treat additional serious, rare skin diseases and vascular malformations.* We have identified several serious, rare skin diseases and vascular malformations where there are no FDA-approved therapies and the genetic mutation or the cause of pathophysiology is known. The QTORIN platform provides the opportunity to target delivery of APIs with very diverse chemical structures and molecular weights to the dermis and epidermis with limited systemic absorption thereby reducing the risk of adverse effects associated with systemic delivery. The incorporation and evaluation of additional APIs into the QTORIN platform has the potential to expand the range of indications for which novel, life-changing therapies may be created. This provides the opportunity for expansion of novel QTORIN products, if approved, to address the needs of hundreds of thousands of patients with skin diseases or vascular malformations who have no FDA-approved therapies for their disease; and

- *continue to establish barriers to entry through intellectual property and regulatory exclusivities.* We have significant intellectual property rights for our current development programs, including issued patents in the U.S. directed to QTORIN rapamycin and methods of using such anhydrous gel formulations of rapamycin. We own issued patents in the US, as well as Europe, Australia, China, Israel and Japan and pending applications in the US, Europe and Japan directed to anhydrous gel formulations of rapamycin and methods of using the same to treat certain skin disorders, including microcystic LMs, venous malformations and angiokeratomas that naturally expire in 2038. We also own issued US patents and a pending US application that encompass anhydrous gel formulations of mTOR inhibitors, including rapamycin, and methods of using the same to treat skin disorders, including microcystic LMs and venous malformations that naturally expire as early as 2038. We also own pending applications in the US and other major markets directed to the use of QTORIN rapamycin for the treatment of microcystic LM that, if issued, would expire in 2042. We exclusively license an allowed US application directed to the treatment of porokeratosis by topically administering a

HMG-CoA reductase inhibitor, such as pitavastatin, that, upon issuance, will naturally expire in June 2040 and we own a pending US provisional application directed to formulations of a HMG-CoA reductase inhibitor, such as pitavastatin, that, if issued, will naturally expire in 2046. Any of our product candidates that receive regulatory approval may also potentially be protected by regulatory exclusivity, such as through the exclusive marketing period provided from Orphan Drug Designation and/or drugs approved based on new clinical investigations (other than bioavailability studies) that are conducted by the sponsor that are essential to approval. We expect to continue to expand our intellectual property portfolio as we continue to develop our product candidates.

Our QTORIN Platform

Our research team developed and designed QTORIN by testing over 80 combinations of excipients and conducted extensive manufacturing process optimization during development of the product. QTORIN is a patented and versatile platform designed to generate potential new therapies that penetrate the deep layers of the skin to locally treat a broad spectrum of serious, rare skin diseases and vascular malformations. Identification and development of novel QTORIN products begins with our team identifying serious, rare skin diseases and vascular malformations with no FDA-approved therapies that have a localized presentation and therefore could be suitable for targeted, topical drug intervention. Once target diseases are selected and key biological pathways that can be causative drivers of that specific disease have been identified, a rigorous formulation development process is undertaken with product development objectives of achieving (i) high payloads of the API in the anhydrous gel, (ii) penetration and distribution of pharmacologically active quantities of the active ingredient to the site of pathophysiology, including the dermis, while achieving minimal to no systemic absorption of the active ingredient, and (iii) optimal physicochemical, stability, and release characteristics to preserve active ingredient potency, prevent crystallization, and support efficient release of the active ingredient from the vehicle into the skin in a formulation with high bioavailability and low systemic absorption that supports consistent product performance, manufacturability, and chronic clinical use.

The QTORIN platform is composed of an anhydrous gel comprising excipients that serve as the vehicle or medium for a drug or other active substance, selected in what we believe is an optimized ratio in order to achieve therapeutic levels of drug delivered to the site of origin of the disease, often within the deepest layers of the skin. Our QTORIN product candidates are developed to accommodate the cargo at high concentrations in order to drive sufficient drug to our target deep in the epidermis and dermis. Inclusion of agents like traditional penetration enhancers are avoided in order to minimize systemic absorption. The final formulation of the drug product is designed to be less than 100% of the maximum solubility of the API to avoid physical instability due to factors such as temperature change.

The QTORIN platform is novel and has generated two program candidates for a combined four indications to date, QTORIN rapamycin and QTORIN pitavastatin.

We believe our QTORIN platform provides the following advantages:

- *reproducible platform across multiple molecules.* The QTORIN platform has demonstrated compatibility with more than 15 high potential pharmacologic agents in preclinical testing. As a result of such compatibility, we believe we will be able to generate new product candidates and reproduce the formulation results from QTORIN rapamycin while minimizing the challenges and timelines typically associated with formulation development activities;

- *versatility across a range of indications.* We believe the ability of the QTORIN platform to deliver a wide range of therapeutic cargoes enables versatility in the targets for molecular intervention, thereby potentially being able to develop novel QTORIN therapies across a diverse set of serious, rare skin diseases and vascular malformations;

- *tailored penetration and distribution of molecules to the site of where the disease originates.* In order to engage the target, a product candidate must deliver therapeutic concentrations of drug substances to the site of the pathophysiology, which is often rendered challenging due to certain agents, such as rapamycin, having high molecular weights or structures that make skin penetration challenging. By optimizing the individual

QTORIN excipient to API ratio for each therapeutic program, our platform is designed to deliver therapeutic agents to the specific site of disease origin at an effective concentration;

- *delivery of therapeutic agents designed to minimize systemic exposure.* Well-accepted mechanisms of action of rapamycin or other therapeutic agents represent potential therapies for rare skin diseases and vascular malformations. However, the adverse event profile of those agents through systemic exposure poses significant barriers to regulatory approval and patient adoption. As observed in all completed clinical trials with QTORIN rapamycin to date, our QTORIN product candidates provide targeted, localized delivery of therapeutic agents to pathogenic tissue of interest while keeping systemic exposure low and thereby reducing the risk of adverse effects associated with systemic delivery;

- *enhanced stability at ambient temperatures.* We have data to support long-term stability of QTORIN rapamycin at room temperature, which we believe is an important feature for patient acceptability, particularly for a chronic dosing regimen; and

- *scalable QTORIN manufacturing.* Under current good manufacturing practice ("cGMP") conditions, we believe we have overcome many of the challenges associated with manufacturing QTORIN rapamycin, including solubility, stability, and scalability. Based on our work to date, we believe that we can successfully scale up QTORIN rapamycin and future QTORIN product candidates to meet our future development and commercial needs.

The Role of mTOR in Cutaneous Disorders

The PI3K/mTOR family of kinases plays vital roles in cellular function by regulating proliferation, growth and survival. Dysregulation of the PI3K/mTOR pathway is associated with several cutaneous disorders, including many serious, rare skin diseases.

Over the past two decades, several studies have been published on the use of oral rapamycin in cutaneous diseases. This work has made it clear that rapamycin's well-documented anti-proliferative, anti-angiogenic, and other targeted mechanisms suggest that this drug could be an effective agent to treat skin diseases, but this promise has not been fulfilled because of the toxicity resulting from systemic dosing.

Rapamycin Challenges and Our Novel Product Candidate, QTORIN Rapamycin

Rapamycin Has Demonstrated Activity in Serious, Rare Skin Diseases and Vascular Malformations

A comprehensive review by Swarbrick and colleagues found over 200 publications demonstrating the broad potential of rapamycin in cutaneous diseases. This publication built upon an early publication by Teng and colleagues in May 2015 that highlighted the substantial promise of mTOR inhibitors, including rapamycin, in a number of difficult to treat dermatologic diseases while advocating for targeted, topical approaches suited to improve tolerability and safety. Despite the preliminary evidence of clinical benefit in many cutaneous diseases, rapamycin's use in cutaneous diseases, including rare skin diseases, remains limited, primarily due to the undesirable toxicity profile of oral rapamycin, including immunosuppression, and the limited biodistribution of oral rapamycin to the dermis.

Barriers to Oral Rapamycin's Use in Cutaneous Diseases

Rapamycin is approved by the FDA as an oral product for the prevention of organ transplant rejection and for the treatment of lymphangioleiomyomatosis. It has been well-established that inhibition of mTOR by rapamycin has the potential to have broad applications in dermatology, but there are several challenges that have limited its use:

- systemic exposure to oral rapamycin is associated with severe toxicities. In addition to its immunosuppressive nature, the most common (≥30%) adverse reactions observed in clinical studies for organ transplant rejection prophylaxis include: peripheral edema, hypertriglyceridemia, hypertension, hypercholesterolemia, increased creatinine and constipation, along with several other intolerable serious adverse reactions. Additionally, because chronic systemic use of oral rapamycin may cause immune suppression and other serious side effects

such as thrombocytopenia and hyperlipidemia, nephrotoxicity and altered insulin sensitivity, oral dosage is not well suited to treating chronic diseases of the skin;

- oral rapamycin has low biodistribution to the skin which limits the clinical utility of the systemic mode of administration against skin diseases; and
- rapamycin is a challenging molecule to formulate and deliver topically as its high molecular weight, poor solubility and chemical instability restrict penetration into the deeper layers of the skin, including the dermis, where many manifestations of rare skin diseases originate. Rapamycin has a molecular weight of 914 Daltons, almost two-fold higher than the generally accepted rule that the molecular weight of a compound should be under 500 Daltons to penetrate the skin.

Our Novel Product Candidate: QTORIN Rapamycin

We have developed QTORIN rapamycin, a novel, 3.9% anhydrous topical gel formulation containing rapamycin, for the treatment of microcystic LMs, cutaneous VMs, and other mTOR-driven skin diseases, as well as clinically significant angiokeratomas. If approved, we believe QTORIN rapamycin has the potential to become first line therapy and the standard of care in each of these diseases.

We believe we have optimized QTORIN rapamycin to deliver therapeutically active levels of rapamycin to the deep layers of the skin, including the dermis, with minimal systemic absorption below immunosuppressive levels. We estimate, based on preclinical studies that QTORIN rapamycin will deliver concentrations of rapamycin — approximately 1000-fold higher than oral rapamycin — to the cutaneous tissue with minimal systemic absorption. During the discovery and development of QTORIN rapamycin, 25 excipients were evaluated in more than 80 different combinations. QTORIN rapamycin was designed to utilize a combination of excipients that we believe maximized solubility while maintaining chemical stability. QTORIN rapamycin has completed formulation optimization and in vitro penetration assays and has demonstrated low systemic absorption in our human clinical trials to date. However, the QTORIN platform is novel and has generated two product candidates, QTORIN rapamycin for three indications and QTORIN pitavastatin for one indication to date, and clinical evidence to support these candidates is preliminary and limited at this time.

QTORIN Rapamycin for the Treatment of Microcystic LMs

Objective	● We are developing QTORIN rapamycin for the treatment of microcystic LMs ● There are no currently FDA-approved therapies ● We believe QTORIN rapamycin, if approved, would be the first approved therapy indicated for microcystic LMs
Our Targeted Approach	● Utilizing QTORIN to confer site-directed delivery of rapamycin to the dermis where microcystic LMs originate
Program Status; Anticipated Upcoming Milestones	● We reported Phase 3 topline data in the 1st quarter of 2026 ● We plan to submit an NDA to the FDA in the second half of 2026 ● We completed our Phase 2 clinical trial in the 4th quarter of 2022
Disease Burden	● Serious, rare and chronic genetic disease characterized by lymphorrhea and acute cellulitis / infections ● Usually present at birth; natural history indicates no spontaneous progression and disease progression throughout life ● Localized masses of malformed lymphatic vessels protrude through the skin barrier
Genetic Basis and Molecular Pathways	● Somatic gain of function mutation primarily in PIK3CA leads to hyperactivated P13K/mTOR signaling
Scientific Rationale	● mTOR is hyperactivated in microcystic LMs ● Rapamycin directly inhibits overactivated mTOR activity and decreases lymphangiogenesis
Market Dynamics	● Estimated prevalence: > 30,000 diagnosed patients in US ● Based on >30,000 diagnosed patients in the U.S., we believe the estimated TAM opportunity on an annualized basis is greater than $1 billion
Intellectual Property; Regulatory Designations*	● We hold U.S. patents and applications in the U.S. and major foreign markets with claims directed to anhydrous gel formulations of rapamycin and methods of use for treating microcystic LMs, expiring in 2038 and, for certain applications, if issued, as late as 2042 ● FDA Fast Track Designation* ● FDA Orphan Drug Designation* ● FDA Breakthrough Therapy Designation* ● FDA Orphan Products Clinical Trials Grant Recipient ● European Commission orphan medicinal product designation

* *Fast Track, Breakthrough Therapy or Orphan Drug Designation may not result in a faster development process, review or approval. Please see paragraphs titled "Special FDA Expedited Review and Approval Programs" and "Orphan Drugs" herein for more information.*

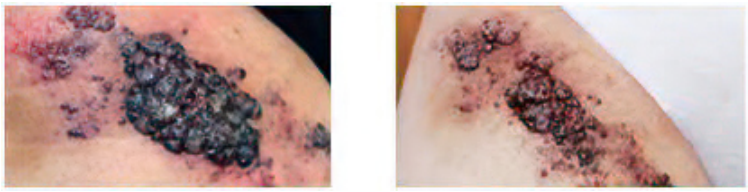
Disease Overview

Microcystic LM is a serious, rare disease of the lymphatic system characterized by lymphorrhea, which is the persistent discharge of internal lymph fluid from disrupted lymphatic vessels, and acute cellulitis, or a bacterial infection of the skin underlying tissues (Figure 1). Microcystic LMs primarily arise from somatic activating mutations in PIK3CA resulting in hyperactivation of the PI3K/mTOR signaling pathway. Microcystic LM is one of three morphologic types of LMs based on the size of the individual cysts (as opposed to the overall size of the LM): macrocystic (>2cm), microcystic (<2cm) and combined. Microcystic LMs often present at birth and are the result of congenital abnormalities of the lymphatic system thought to originate during the embryologic development of lymphatic vessels. Microcystic LM leads to malformed lymphatic vasculature, persistent infiltration of lymph fluid into soft tissues, and locally invasive masses with pathologic sequelae.

Due to the chronic lymphorrhea, cellulitis and other symptoms, microcystic LM is associated with a high degree of morbidity and has a significant impact on daily life. Microcystic LM can be located on any region of the body but is most commonly found in high areas of lymphatic vessels, including the trunk, head and neck. The malformations connect to the epidermis in the form of vesicles, papules, and plaques which can leak at the surface. Infections of malformations can occur and may lead to cellulitis of surrounding tissues or severe, life-threatening infections. The natural history of microcystic LM is progressive, with symptoms generally worsening during life, including increases in the number size of cysts that lead to complications, and morbidity.

Microcystic LMs arise due to post zygotic mutations during early embryonic development, are usually present at birth, and are persistent and progressive throughout life. Patients are usually diagnosed at a young age by pediatric dermatologists or pediatric hematologists and are often managed by multi-disciplinary teams. Due to the genetic nature of the disease, microcystic LMs are programmed to be on the skin and do not spontaneously regress. In a 2017 review of 153 patients over a 34-year period to determine if LM sub-types had spontaneous regression, spontaneous regression was observed in 0% of patients with microcystic LM (n=28; Table 1).

FIGURE 1. Example of Microcystic LM



Despite the high rate of morbidity and life-threatening cellulitis associated with microcystic LM, there are currently no FDA-approved medications for this disease. Typical treatment approaches include surgery, sclerotherapy with bleomycin or other sclerotic agents, laser, and cryotherapy, which are invasive, can induce further inflammation and result in high recurrence rates. Surgical resection remains challenging and ineffective due to the infiltrative, diffuse nature of microcystic LM. In addition, due to underlying associated somatic mutation, it is difficult to achieve accurate and clear surgical margins, resulting in high recurrence rates post resection. The high unmet need and drawbacks associated with surgical approaches have spurred the search for treatment alternatives that target the underlying pathological mechanisms of this disorder.

Microcystic LM Does Not Have Spontaneous Regression

Due to the genetic nature of the disease, microcystic LMs are persistent and progressive throughout life, without spontaneous regression. A review article, which followed subjects over a 34-year observation period, found no spontaneous regression throughout that time among 28 participants with microcystic LM (Table 1).

TABLE 1: Clinical Characteristics of Spontaneous Regression of the LM Patients

Types	Microcystic
Subtotal	28
Sex (F:M)	14:14
Age (y), mean ± SD	0.89 ± 1.4
Maximum diameter (cm), mean ± SD	—
Spontaneous regression	
Positive	0
Negative	28

Because microcystic LM does not have spontaneous regression, a baseline-controlled study, in which subjects' status on therapy is compared with the status before therapy, can be suitable for this disease because improvement does not

reflect the natural history of the disease in the absence of treatment and can therefore be attributed to be a direct therapeutic effect.

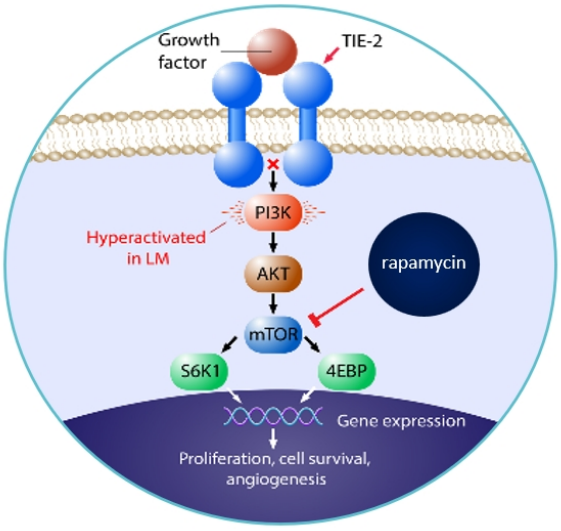
Discovery of mTOR as key driver of microcystic LMs

Important insights gained over the last decade have implicated increased activation of the PI3K/mTOR signaling pathway in microcystic LM. Enhanced mTOR signaling has been observed to increase the expression of the vascular endothelial growth factor, or VEGF, a key promoter of angiogenesis and lymphangiogenesis. This leads, in turn, to uncontrolled, disorganized, and malformed lymphatic development.

Hyperactivation of the PI3K/mTOR pathway results in lymphatic endothelial cell proliferation and migration, defective mural cell coverage and aberrant lymphatic vascular network formation. This ultimately results in the anatomic malformations in lymphatic channels seen in this disease.

Rapamycin inhibits mTOR, which is a downstream element of the over-activated PI3K/mTOR pathway (Figure 2). Rapamycin demonstrated in preclinical studies an ability to decrease mTOR signaling, thereby reducing endothelial cell proliferation and subsequently the formation of malformed lymphatic vessels. Additionally, rapamycin reduces lymph fluid formation in the affected tissue, helping to minimize clinical symptoms associated with microcystic LM.

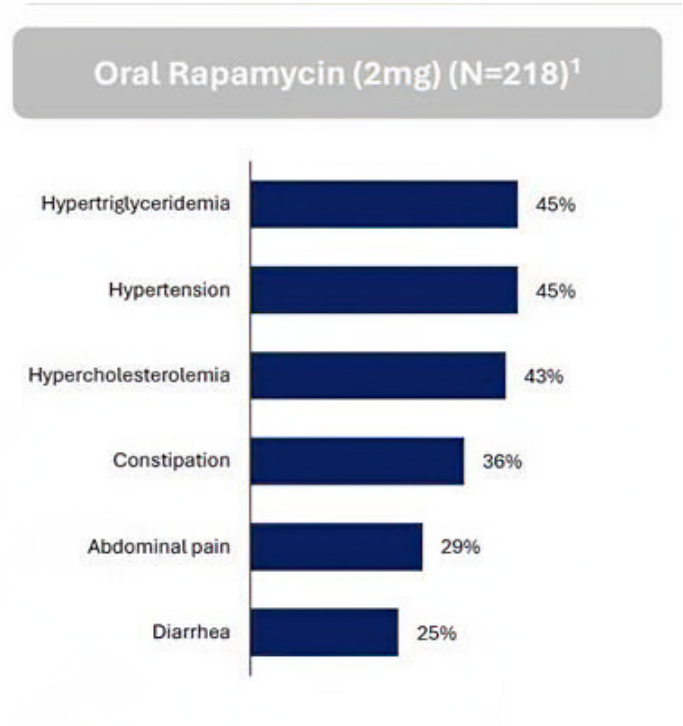
FIGURE 2. PI3K/mTOR Pathway Is Overactivated in Microcystic LM and Point of Rapamycin Pharmacologic Inhibition



A large and growing evidence base strongly supports rapamycin's activity in treating microcystic LM: since 2011, a total of 16 studies evaluating the off-label use of rapamycin in microcystic LM have been published. In a 2021 article by Kalwani *et al*, the authors stated "Sirolimus [rapamycin], a strong inhibitor of mTOR, has shown tremendous promise in the treatment of LM." Systematic reviews of rapamycin for the treatment of microcystic LMs have demonstrated that rapamycin can significantly improve the prognosis.

Oral rapamycin is sometimes used in clinical practice in leading academic vascular anomalies clinics where microcystic LM patients are often treated. Importantly, off-label use of oral rapamycin is associated with an adverse event profile that requires frequent patient monitoring and limits its use for a chronic disease such as microcystic LM (Figure 3). Particularly for pediatric and adolescent patients, these toxicities limit the use of oral rapamycin. In addition, oral rapamycin is associated with a narrow therapeutic window due to the adverse event profile described above and the poor biodistribution of oral rapamycin to the dermis, which is where microcystic LMs originate.

FIGURE 3. Adverse Events Observed with Oral Rapamycin Treatment In the Study of Prophylaxis of Organ Rejection Following Renal Transplantation



As a result, there remains a significant unmet need for a targeted rapamycin therapy for microcystic LM that limits systemic absorption and the adverse effects associated with systemic therapy.

Advancing QTORIN Rapamycin in Microcystic LM

We are evaluating QTORIN rapamycin for the treatment of microcystic LM. QTORIN rapamycin has the potential to be the first therapy and standard of care in the U.S. for microcystic lymphatic malformations, if approved.

Based on preclinical studies, we believe that QTORIN rapamycin will deliver concentrations of rapamycin approximately 1000-fold higher than systemic rapamycin to the cutaneous tissue with minimal systemic absorption. We therefore believe that QTORIN rapamycin has the potential to harness the potential therapeutic benefits of rapamycin while minimizing the well-known side effects of oral rapamycin.

We completed an open-label Phase 2 trial to evaluate QTORIN rapamycin in patients with microcystic LM in the 4th quarter of 2022. Results of that trial are detailed below. Based on those results and discussions with the FDA at a Type C Meeting in 2023 and a Type B Breakthrough Therapy Meeting in 2024, we initiated a Phase 3 trial, SELVA, to evaluate QTORIN rapamycin in patients with microcystic LM in the third quarter of 2024. In the first quarter of 2025, we expanded the trial to include patients ages three to five years old. Previously, trial participants were required to be at least six years old. This decision followed communication with the FDA in which the Agency deemed our proposed population expansion acceptable. In February 2026, we announced positive topline results from SELVA, the Phase 3, single-arm, baseline-controlled study, which evaluated the safety and efficacy of QTORIN rapamycin for the treatment of microcystic LMs in patients 3 years and older. The study met the pre-specified primary endpoint, the mLM-IGA, with a +2.13 ($p<0.001$) improvement at Week 24. The study also met its pre-specified key secondary and all four additional secondary endpoints with statistical significance (all $p<0.001$). In the first quarter of 2026, we submitted a pre-NDA meeting request to the FDA. We anticipate the meeting to occur during the second quarter of 2026. We plan to request FDA agreement to begin a rolling submission of a Section 505(b)(2) NDA in the second half of 2026. Results of the Phase 3 trial are described below.

QTORIN rapamycin has been granted FDA Fast Track Designation, Orphan Drug Designation, and Breakthrough Therapy Designation for the treatment of microcystic LM.

Clinical Development Overview

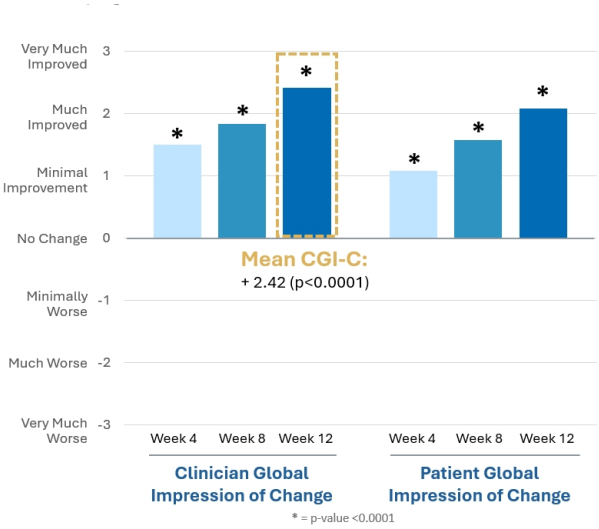
We completed an open-label, Phase 2 trial (PALV-06) with QTORIN rapamycin in patients with microcystic LM and, based on the results of that trial, Breakthrough Therapy Designation was granted to QTORIN rapamycin for the treatment of microcystic LM. A subsequent Breakthrough Therapy Designation meeting with the FDA was held and our Phase 3 trial, SELVA, was initiated in the third quarter of 2024 and we reported topline results in the first quarter of 2026. Additionally, we were awarded an FDA Orphan Products Clinical Trials Grant for up to $2.6 million supporting the SELVA Phase 3 study in the third quarter of 2024, for which we have received proceeds of $1.1 million to date.

PALV-06 Overview and Efficacy Results

The Phase 2 PALV-06 trial was a multi-center, open-label study of subjects receiving QTORIN rapamycin once-daily for 12 weeks. The Phase 2 clinical trial featured multiple pre-specified efficacy assessments, including clinician and patient global impression assessments as well as assessments of individual clinical manifestations that are important disease burdens for individuals living with microcystic LMs. As is common in Phase 2 studies, efficacy was evaluated as secondary endpoints without multiplicity adjustment or formal statistical analysis. The PALV-06 trial enrolled 12 participants, all of whom completed 12-weeks of once daily ("QD") QTORIN rapamycin treatment as well as all study related activities.

A baseline-controlled study is a clinical study in which the patient's condition during treatment is compared with their condition before treatment. In such studies, participants serve as their own control. In a placebo-controlled study, patients are randomized prior to treatment to receive either study drug or matching placebo and to determine how the efficacy of the treatment compares to placebo. Baseline-controlled studies are appropriate when the treatment effects are dramatic, occur rapidly following treatment, and are unlikely to have occurred spontaneously (e.g., general anesthesia, cardioversion, measurable tumor shrinkage).

FIGURE 4: PALV-06 Improvement in Clinician- and Patient- Reported Impression of Change



Statistically significant across key clinician-assessed individual signs of microcystic LM at week 12

- Height (p<0.0001)
- Leaking (p<0.005)
- Bleeding (p<0.05)
- Erythema (p<0.005)
- Hyperkeratosis (p<0.005)

Efficacy data from the Phase 2 open-label study demonstrated statistically significant and clinically meaningful improvements for microcystic LM participants treated with QTORIN rapamycin on several of the efficacy endpoints studied. The data demonstrated improvements as compared to pre-treatment (baseline) across several clinically relevant and important endpoints, including many of the static and impression of change global instruments (Table 2). Statistically significant improvements in the clinician global impression of severity ("CGI-S"), clinician global impression of change ("CGI-C"), and patient global impression of change ("PGI-C") were supported by visual

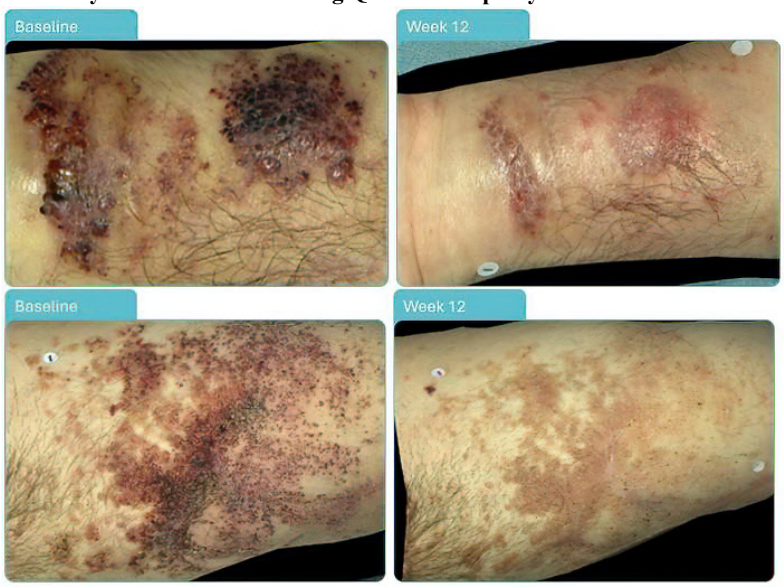
improvements of target lesions captured in photographs. Clinical meaningfulness was confirmed by participant interviews.

TABLE 2: PALV-06 Study Efficacy Results on Clinician- and Patient-Reported Impression of Change Instruments As Compared to Pre-Treatment (Baseline)

Efficacy Endpoints	**Week 12** Mean (n=12)	**Nominal, Two-sided p-value**
Clinician Global Impression of Change (CGI-C)	2.42	<0.0001
Clinician Global Impression of Severity (CGI-S) - Overall	-1.33	<0.0001
CGI-S Height	-1.67	<0.0001
CGI-S Leaking	-0.92	0.0047
CGI-S Bleeding	-0.92	0.0197
CGI-S Erythema	-1.08	0.0016
CGI-S Crusting/Hyperkeratosis	-1.17	0.0012
Patient Global Impression of Change (PGI-C)	2.08	<0.0001
CGI-C and PGI-C improvements are represented by increases; CGI-S improvements are represented by reductions CGI-C and PGI-C are 7-points scales ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3) CGI-S is a 5-point lesion severity scale p-values are nominal as there was no adjustment for multiplicity amongst efficacy endpoints All p-values from paired t-tests vs mean change of 0 as compared to baseline		

In addition to meaningful improvements in clinician- and patient-reported outcomes, visible improvement in lesions was observed following treatment with QTORIN rapamycin.

FIGURE 5: Visible Improvement in Microcystic LM Lesions During QTORIN Rapamycin Treatment in PALV-06



PALV-06 Phase 2 Pharmacokinetic and Safety/Tolerability Results

Systemic concentrations of rapamycin following administration of QTORIN rapamycin in the PALV-06 trial were <2 ng/mL for all participants at all time points tested with an average of 120.98 pg/mL across all patients and time points tested. Safety data obtained in the PALV-06 trial was similar to that observed in larger clinical studies of QTORIN rapamycin, including clinical trials in Pachyonychia Congenita (PALV-02, -03, -05) and Gorlin Syndrome (PALV-04). QTORIN rapamycin was generally well tolerated with all treatment related adverse events either mild or moderate. No study participants discontinued or withdrew from the study. No serious adverse events, clinically significant lab abnormalities or vital sign abnormalities were reported. The most common TRAEs occurring in ≥2 participants were application site pain (n=3, 25.0%), application site pruritus (n=3, 25.0%), and nausea (n=2, 16.7%).

TABLE 3: PALV-06 Treatment Related Adverse Events in Microcystic LM Participants

TREATMENT RELATED AES	RELATED ANY GRADE EVENTS (%, N=12)
Application site pain	3 (25)
Application site pruritus	3 (25)
Application site discharge	1 (8.3)
Application site erythema	1 (8.3)
Application site paraesthesia	1 (8.3)
Nodule	1 (8.3)
Eczema	1 (8.3)
Skin exfoliation	1 (8.3)
Diarrhea	1 (8.3)
Headache	1 (8.3)

SELVA Phase 3 trial and Anticipated pre-NDA Meeting

We designed our Phase 3 SELVA trial (Figure 6) based on results from the Phase 2 trial and consideration of comments from the FDA during End of Phase 2 and Breakthrough Therapy Designation Meetings. Discussions with the FDA focused on several aspects of the proposed clinical trial design, including the patient population, dosing, and endpoint selection. The FDA commented on each of these areas and advised where further clarification was requested.

Subsequent to the Breakthrough Therapy Designation Meeting and incorporation of certain FDA feedback into the Phase 3 trial design, we were notified in September 2024 that we had been awarded an FDA Orphan Products Clinical Trials Grant for up to $2.6 million to support our Phase 3 trial of QTORIN rapamycin for the treatment of microcystic LMs, for which we have received proceeds of $1.1 million to date. Since the program's inception, the FDA has awarded approximately 700 Orphan Products Clinical Trials Grants to fund clinical trials of products evaluating the efficacy and/or safety in support of a new indication or change in labeling to address unmet needs for patients with rare diseases or conditions. Grant applications are peer reviewed and evaluated for scientific and technical merit by a panel of experts in the subject field of the specific application. Consultation with the relevant FDA review division may also occur during this phase of the review to determine whether the proposed study will provide acceptable data that could contribute to product approval. A score is assigned to each application based on the scientific/technical review criteria including:

- rationale;
- study design;

- inclusion of patient input;
- investigator(s);
- infrastructure;
- financial resources; and
- ability to advance the current field.

The review panel may advise the Orphan Products Grant program staff about the appropriateness of the proposal to the goals of the grant program. Since inception, the FDA Orphan Products Grants Program has funded clinical trials that have facilitated the approval of more than 85 products. Our receipt of the grant does not guarantee FDA approval of QTORIN rapamycin for the treatment of microcystic LM or any other indication.

Our Phase 3 SELVA trial to evaluate QTORIN rapamycin in patients with microcystic LMs was originally designed to include up to 40 participants to be treated with QTORIN rapamycin QD for 24+ weeks. We enrolled 51 participants, of which 50 initiated treatment, including 49 participants aged ≥ 6 years and 1 participant in the exploratory 3- to 5-year-old cohort. The primary and key secondary endpoints are a 7-point change mLM-IGA, a dynamic assessment that uses a comparative rating scale, and a blinded evaluation using the microcystic LM multi-component static scale, respectively. Clinician-reported change in severity from the start of treatment as measured by the mLM-IGA scale is supported by Phase 2 trial results as exit interviews conducted with the clinicians who were part of the trial. More specifically, these data support that clinicians can accurately rate change in microcystic LM disease severity across each level of disease activity. The endpoints have been designed to capture clinical changes in key aspects of a patient's disease, as reported by the clinicians and patients.

We believe the following supports the use of the mLM-IGA, a dynamic assessment that uses a comparative rating scale, as the primary endpoint:

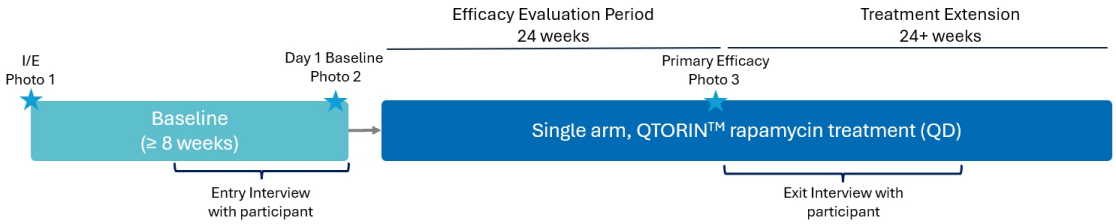
- the mLM-IGA is an endpoint that was specifically designed for this rare disease population with extensive endpoint development incorporating both physician and patient views; and
- the use of a global instrument was the strong and consistent preference of clinician investigators due to it being a multi-sign/symptom disease.

The FDA has recommended that primary efficacy in the treatment of microcystic LM be evaluated on a static multicomponent assessment scale but recommended that we provide a rationale for selecting the mLM-IGA comparative rating scale as the primary endpoint. While static scales were explored, these scores were shown to be less sensitive. Furthermore, the mLM-IGA is different from the traditional comparative rating scales in that investigators must score individual clinical signs before filling out the mLM-IGA and the mLM-IGA leverages baseline photographs to provide more objective scoring.

The mLM-IGA also leverages the well-accepted 7-point dynamic change scale that has been used in FDA labeling across many diseases/therapeutic areas.

We believe that a baseline-controlled study is an appropriate trial in patients with microcystic LM because there is evidence the effects of QTORIN rapamycin in this setting are dramatic and occur rapidly following treatment, and effects are unlikely to have occurred spontaneously. The Phase 2 study was a baseline-controlled study, and provided evidence that the treatment effect with QTORIN rapamycin was dramatic and occurred rapidly as evidenced by nominally significant results at the first timepoint measured, 4 weeks. These effects, as well as the from the Phase 3 study, are unlikely to have occurred spontaneously. Microcystic LM has a well-understood pathophysiology and a well-defined disease course such that the natural history of the disease shows that patients with microcystic LM do not have spontaneous regression. Therefore, we believe that any improvement can more confidently be attributed to study drug rather than natural fluctuations or spontaneous improvement of the disease. This aligns with the FDA's Draft Guidance on Expedited Programs for Serious Conditions – Drugs and Biologics (May 2014), which states "single-arm trials may be an important option in rare diseases with well-understood pathophysiology and a well-defined disease course."

FIGURE 6. Microcystic LM SELVA Phase 3 Trial Design



In February 2026, we announced positive topline results from SELVA, a Phase 3, single-arm, baseline-controlled clinical trial evaluating once-daily QTORIN rapamycin in individuals aged ≥ 3 years with microcystic LMs. Of the 51 participants enrolled, 50 initiated treatment, including 49 participants aged ≥ 6 years and 1 participant in the exploratory 3- to 5-year-old cohort. In accordance with the statistical analysis plan, efficacy results were reported for participants aged ≥ 6 years, which constituted the ITT population. The study was originally designed to enroll 40 participants across leading U.S. vascular anomaly centers and exceeded its target enrollment.

Topline efficacy results from SELVA are as follows:

TABLE 4: SELVA Study Efficacy Endpoints at Week 24

Efficacy Endpoints at Week 24 (ITT Population, n=49)	**Mean Change**	**Two-sided p-value**
Primary: Microcystic Lymphatic Malformation Investigator Global Assessment (mLM-IGA)*	+2.13	p<0.001
Key Secondary: Blinded mLM Multi-Component Static Scale (mLM-MCSS)**	-3.36	p<0.001
Secondary: Patient Global Impression of Change (PGI-C)*	+1.9	p<0.001
Secondary: Live mLM-MCSS**	-4.6	p<0.001
Secondary: Clinician Global Impression of Severity (CGI-S)***	-1.7	p<0.001
Secondary: Patient Global Impression of Severity (PGI-S)***	-1.0	p<0.001

• n=49 subjects aged 6 and older; data analyzed per statistical analysis plan; non-completer data handled via multiple imputation per statistical analysis plan for primary endpoint; endpoints tested according to pre-specified hierarchical testing procedure

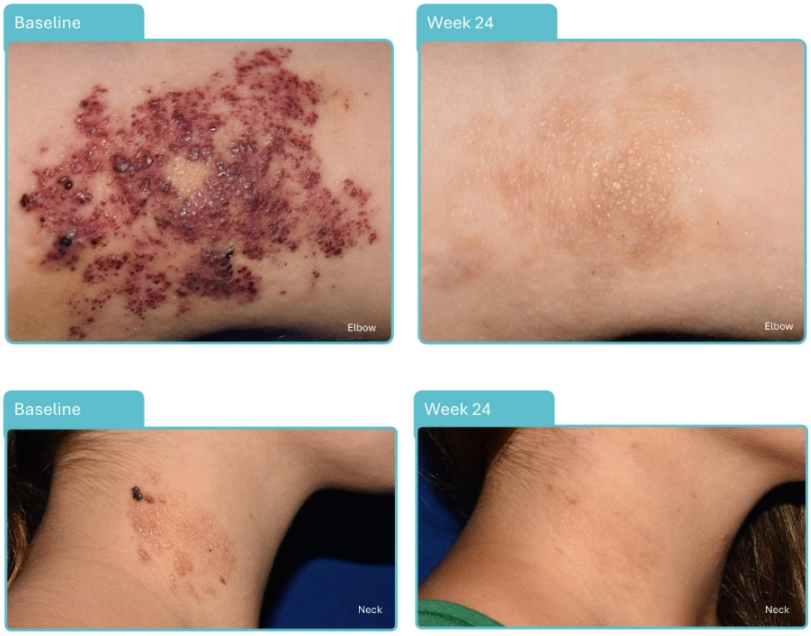
*Dynamic change scales (7-point scales ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3); positive values indicate improvements from baseline)

**mLM-MCSS (Sum of three static severity scales: Height, Leaking/Bleeding, Vesicle Appearance. Each scale rated "Clear or Almost Clear" (1) to "Very Severe" (5); total score 3-15. Test baseline to Week 24 change; negative values indicate improvements from baseline)

***Static severity scales (5-point scales ranging from 1 to 5; negative values indicate improvements from baseline)

In addition to meaningful improvements in clinician- and patient-reported outcomes, visible improvement in lesions was observed following treatment with QTORIN rapamycin.

FIGURE 7: Visible Improvement in Microcystic LM Lesions During QTORIN Rapamycin Treatment in SELVA



The primary endpoint, the mLM-IGA, is a 7-point clinician-assessed dynamic scale measuring change in disease severity from baseline ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the mLM-IGA in the ITT population (n=49), QTORIN rapamycin demonstrated a mean improvement of +2.13 points, meeting the study's primary endpoint ($p<0.001$). Of the participants aged ≥ 6 who completed the efficacy evaluation period, 95% (41/43) demonstrated at least a 1-point improvement, and 86% (37/43) were either "Much Improved" (+2) or "Very Much Improved" (+3).

The key secondary endpoint, the mLM-MCSS, a clinician-assessed static scale scored as the total of three sub-scales (minimum score: 3; maximum score: 15) capturing lesion height, leaking/bleeding, and vesicle appearance, improved by a mean of 3.36 points ($p<0.001$), based on a blinded independent review of randomized Baseline and Week 24 photographs evaluated by a committee of clinician experts.

We plan to request FDA agreement to begin a rolling submission of a Section 505(b)(2) NDA for QTORIN rapamycin for the treatment of microcystic LM in the second half of 2026. Our NDA strategy is to demonstrate the substantial evidence of effectiveness for the treatment of microcystic LM based on results from the Phase 3 study plus confirmatory evidence from the Phase 2 study, real-world evidence of rapamycin used for the treatment of microcystic LMs, and the natural history of the disease.

To support our Section 505(b)(2) NDA, we plan to bridge QTORIN rapamycin and RAPAMUNE based on a cross-study comparison between pharmacokinetic data of QTORIN rapamycin from the Phase 3 trial and the prescribing information for RAPAMUNE; the FDA recommends that bridging to support an NDA for the treatment of microcystic LM be done in a relative bioavailability study comparing the pharmacokinetics of a topical product applied under maximal use conditions and the approved oral drug. The planned cross study analysis will allow for comparison of systemic pharmacokinetic parameters, key criteria for assessing the applicability of safety findings from the listed drug, which are a result of systemic exposure from the oral formulation. We believe the proposed clinical pharmacology plan will address the requirements for bridging to support reliance on the FDA's previous findings of

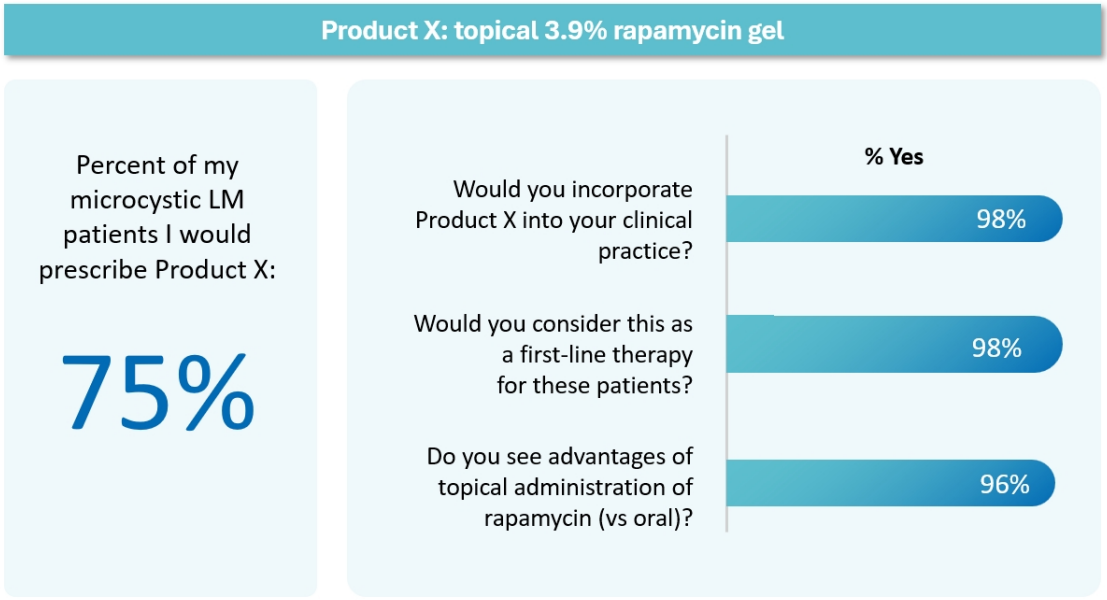
safety for RAPAMUNE tablets to support a Section 505(b)(2) NDA submission by establishing relative bioavailability to known pharmacokinetic parameters of RAPAMUNE as well as pharmacokinetics under maximal use conditions. Population pharmacokinetic analyses, including covariate analyses, will be conducted as data allows. No additional studies are planned, as a bridging approach is planned to enable labeling guidance for specific populations and drug-drug interactions. We will also rely on the listed drug for additional components of our Section 505(b)(2) NDA.

Potential Market Opportunity and Market Research

We believe that QTORIN rapamycin, if approved, has commercial potential for treating microcystic LM in the U.S. The treatment regimen in microcystic LM, we believe, would be chronic dosing due to the genetic nature of the condition. As discussed below, based on both claims analyses and a published real-world occurrence study of U.S. physicians, we estimate that there are over 30,000 diagnosed microcystic LM patients in the United States. Furthermore, the introduction of a new treatment may lead to improved awareness of the disease, better and sooner diagnosis, and more patients actively seeking therapy.

As part of better understanding the market opportunity in microcystic LM, we commissioned a primary market research study in May 2024 that surveyed 52 dermatologists and hematologists (Figure 7). As part of the market research, a target product profile describing QTORIN rapamycin, named Product X, was presented based on QTORIN rapamycin's Phase 2 results.

FIGURE 8. Market Research Report (May 2024) On Product X



Survey of 52 high-volume dermatologists and hematologists (average of 11.7 cutaneous microcystic lymphatic malformation patients)

We believe that this preliminary market research underscores both the unmet need and the significant market opportunity for QTORIN rapamycin for the treatment of microcystic LMs.

QTORIN Rapamycin for the Treatment of Cutaneous Venous Malformations

Objective	● There are currently no FDA-approved therapies indicated for the treatment of cutaneous VMs; we are developing the first targeted therapy ● We believe QTORIN rapamycin, if approved, would be the first approved therapy indicated for cutaneous VMs
Program Status; Anticipated Upcoming Milestones	● We reported topline efficacy data from our Phase 2 TOIVA study in the 4th quarter of 2025 ● We anticipate initiating a Phase 3 pivotal study in the second half of 2026
Genetic Basis and Molecular Pathways	● Somatic mutations in TEK or PIK3CA lead to aberrant PI3K/mTOR signaling
Disease Burden	● Cutaneous VMs are a serious, rare condition characterized by the overgrowth of veins that protrude through the skin and are characterized by deformities, functional impairment, and hemorrhaging ● Usually present early in life; progresses throughout life ● Localized masses of malformed veins protrude through the skin barrier
Scientific Rationale for Cutaneous Venous Malformations	● TIE2 and PI3K overactivation converge on mTOR ● Rapamycin directly inhibits overactivated mTOR activity and decreases endothelial proliferation and venous formation
Our Targeted Approach	● QTORIN is designed to confer site-directed delivery of rapamycin to the epidermis and dermis ● Estimated prevalence: >75,000 in the United States ● We believe the estimated TAM opportunity on an annualized basis is greater than $1 billion
Intellectual Property; **Regulatory Designations**	● We hold U.S. patents and patent applications in both the U.S. and major foreign markets with claims directed to anhydrous gel formulations of rapamycin and methods of use for treating cutaneous VMs, expiring in 2038 ● FDA Fast Track Designation*

**Fast Track Designation may not result in a faster development process, review or approval. Please see paragraph titled "Special FDA Expedited Review and Approval" herein for more information.*

Disease Overview

Cutaneous VMs are congenital vascular anomalies characterized by dysregulated growth of veins within the skin. They present as dilated, tortuous vessels that manifest as bluish or purplish patches or nodules on the skin. These malformations result from developmental errors in venous morphogenesis during embryogenesis, leading to abnormal connections between veins and capillaries. These anomalies are typically present at birth and can expand or become more prominent with age. They vary in size and distribution, ranging from small, localized lesions to more extensive areas of affected skin. Cutaneous VMs cause functional impairment, significantly impact quality of life and are associated with severe long-term complications.

Management of cutaneous VMs depends on factors such as symptomatology, location, and patient preferences. Treatment options, which are limited and insufficient, include conservative approaches such as observation and compression therapy, as well as interventional techniques like sclerotherapy (injection of sclerosing agents to induce vessel shrinkage), laser therapy, and surgical excision for larger or symptomatic malformations. Procedures to remove venous malformations are often not curative, with high rate of recurrence and regrowth. Complications from serial attempts to remove venous malformations including scarring, swelling, and nerve deficits are also compounded when multiple procedures are required. There are no FDA-approved treatments for cutaneous VMs and there is an urgent need for an approved pharmacologic treatment for these patients.

Cutaneous venous malformation is a chronic disease that worsens over time with no spontaneous regression. The invasiveness and limited effectiveness of current treatments, coupled with the lack of approved pharmacotherapy options, demonstrate the urgent need for an FDA-approved therapy for cutaneous VMs. A targeted topical therapy that directly addresses disease pathology is of interest to this patient population, as it could abolish the need for systemic treatments that have wider toxicity or invasive procedural interventions.

Discovery of mTOR as key driver of Venous Malformations

Cutaneous VMs are primarily caused by somatic mutations in either TEK or PIK3CA leading to overactivated PI3K/mTOR signaling. TEK encodes for the endothelial cell-specific receptor tyrosine kinase (TIE2), which in turn activates phosphatidylinositol-3-kinase (PI3K) with mutations in this gene accounting for approximately 70% of cutaneous venous malformation cases. Mutations in the PIK3CA gene, which encodes the p110α catalytic subunit of PI3K, have also been identified in cutaneous VMs accounting for approximately 30% of cases that do not have TEK mutations. The PI3K/mTOR pathway plays a crucial role in regulating cell growth, proliferation, and survival. Mutations in TEK or PIK3CA lead to increased activation of this pathway, promoting abnormal endothelial cell proliferation and result in the formation of cutaneous VMs. Rapamycin, an mTOR inhibitor, dampens PI3K/mTOR signaling, thus garnering attention as a potential therapeutic option for cutaneous VMs.

Our Solution: QTORIN Rapamycin

We are developing QTORIN rapamycin for the treatment of cutaneous VMs. Rapamycin inhibits mTOR, which is a downstream element of the PI3K/mTOR pathway. In doing so, rapamycin is thought to diminish PI3K/mTOR overactivation, thereby reducing endothelial cell proliferation and subsequently the formation of malformed vessels. Several published case studies and clinical trials have provided initial signals of activity in the use of oral rapamycin for the treatment of venous malformations.

Clinical Development Overview

In December 2025, we announced positive topline efficacy results from the Phase 2 TOIVA study, a 12-week, multicenter, single-arm, open-label, baseline-controlled Phase 2 clinical trial of QTORIN rapamycin for cutaneous VMs. The study achieved statistical significance on multiple pre-specified clinician-reported and patient-reported efficacy endpoints, including dynamic change endpoints and static severity endpoints. The study enrolled 16 participants, ages six and older, at leading vascular anomaly centers across the U.S. Multiple measures of efficacy, including change in clinician and patient global impression assessments, as well as assessments of specific individual clinical manifestations which contribute to disease burden, were evaluated. To help contextualize changes on efficacy endpoints and, specifically, better understand any patient quality of life impact resulting from QTORIN rapamycin, qualitative exit interviews were conducted by a third-party interviewer with a subset of participants from the Phase 2 study.

We completed a Preliminary Breakthrough Therapy Designation Advice meeting with the FDA in the first quarter of 2026. Based on that meeting, our intent is to submit an application to the FDA for Breakthrough Therapy Designation in the second quarter of 2026. We plan to commence a Phase 3 pivotal study in the second half of 2026.

TOIVA Overview and Efficacy Results

The Phase 2 TOIVA trial was a single-arm, open-label, baseline-controlled study of QTORIN rapamycin administered topically once daily for a 12-week efficacy evaluation period followed by a 12-week treatment extension period, for cutaneous VMs. The study enrolled 16 participants, ≥ 6 years, at leading vascular anomaly centers across the U.S. Multiple measures of efficacy, including change in clinician and patient global impression assessments, as well as assessments of specific individual clinical manifestations which contribute to disease burden, were evaluated.

A baseline-controlled study is a clinical study in which the patient's condition during treatment is compared with their condition before treatment. In such studies, participants serve as their own control. In a placebo-controlled study, patients are randomized prior to treatment to receive either study drug or matching placebo and to determine how the efficacy of the treatment compares to placebo. Baseline-controlled studies are appropriate when the treatment effects are dramatic, occur rapidly following treatment, and are unlikely to have occurred spontaneously (e.g., general anesthesia, cardioversion, measurable tumor shrinkage).

Efficacy data from the Phase 2 open-label study demonstrated nominally statistically significant ($p<0.001$) improvements at Week 12 on several of the clinically relevant and important efficacy endpoints evaluated when compared to pre-treatment (baseline), including many of the static and impression of change global instruments evaluated, including the Overall Cutaneous VM Investigator Global Assessment ("Overall cVM-IGA") (Table 5). The Overall cVM-IGA is a 7-point, clinician-assessed, single-item efficacy endpoint measuring change in severity from baseline, with the numeric rating scale ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the Overall cVM-IGA at Week 12, 73% (11/15) participants improved, with 67% (10/15) either "Much Improved" (+2) or "Very Much Improved" (+3). No trial participants (0/15) were "Minimally Worse" (-1), "Much Worse" (-2), or "Very Much Worse" (-3). Nominally statistically significant improvement in the clinician global impression of severity ("CGI-S"), clinician global impression of change ("CGI-C"), and patient global impression of change ("PGI-C") were supported by visual improvements of target lesions captured in photographs. To help contextualize changes on efficacy endpoints and, specifically, better understand any patient quality of life impact resulting from QTORIN™ rapamycin, qualitative exit interviews were conducted by a third-party interviewer with a subset of participants from the Phase 2 study.

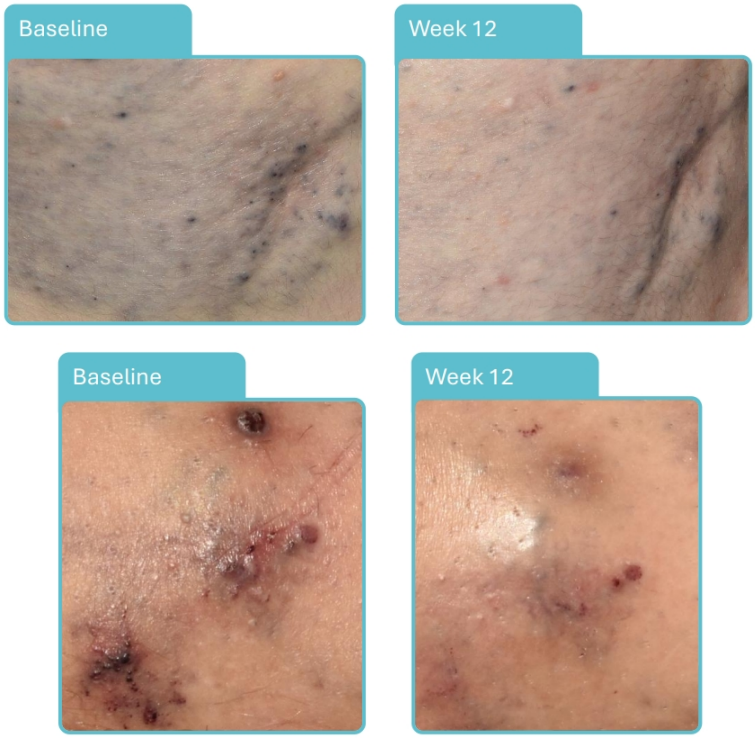
TABLE 5: TOIVA Study Efficacy Results on Clinician- and Patient-Reported Impression of Change Instruments as Compared to Pre-Treatment (Baseline)

Efficacy Endpoints at Week 12 (ITT Population)	**Mean Change from Baseline (n=15)**	**Nominal, Two-sided p-value**
Dynamic Change Scales *(7-point scales ranging from -3 to +3; positive values indicate improvements from baseline)*		
Overall Cutaneous VM Investigator Global Assessment (Overall cVM-IGA)	1.5	<0.001
cVM-IGA Height/Engorgement	1.3	<0.001
cVM-IGA Appearance (visualization/color of affected veins)	1.5	<0.001
cVM-IGA Bleeding	0.7	0.045
Overall Patient Global Impression of Change (PGI-C)	1.1	<0.001
Static Severity Scales *(5-point scales ranging from 1 to 5; negative values indicate improvements from baseline)*		
Overall Clinician Global Impression of Severity (CGI-S)	-1.0	<0.001
cVM-MCSS (Cutaneous VM Multi-Component Static Scale) Severity of Height/Engorgement	-1.3	<0.001
cVM-MCSS Severity of Appearance (visualization/color of affected veins)	-1.1	<0.001
Overall Patient Global Impression of Severity (PGI-S)	-0.5	0.027

p-values are nominal as there was no adjustment for multiplicity amongst efficacy endpoints; change scores and changes from baseline in static scores were compared vs. mean 0 using a 1-sample t-test.
n=15 subjects completed treatment period (one additional dosed participant lost to follow up); data analyzed per statistical analysis plan; analysis conducted per available data at each timepoint; ITT analyzed with no imputation of values for missing data.
Ulceration was also assessed with no disease present at baseline. CGI-S Bleeding assessed with limited disease present at baseline.

In addition to meaningful improvements in clinician- and patient-reported outcomes, visible improvement in lesions was observed following treatment with QTORIN rapamycin.

FIGURE 9: Visible Improvements in Cutaneous VM Lesions During QTORIN Rapamycin Treatment in TOIVA



TOIVA Phase 2 Pharmacokinetic and Safety/Tolerability Results

Similar to previous clinical trials of QTORIN rapamycin, in the Phase 2 TOIVA study QTORIN rapamycin was generally well-tolerated, with the most common TEAEs being application site reactions (erythema, 25%). All TRAEs were moderate or mild, with no unexpected adverse events reported. Rapamycin levels were below 2 ng/mL in systemic circulation for all participants at all timepoints in the study.

Potential Market Opportunity

We believe that QTORIN rapamycin, if approved, has significant commercial potential in cutaneous VMs in the U.S. and other markets. The treatment regimen in cutaneous VMs, we believe, would be chronic dosing due to the genetic nature of the condition. We estimate, based on published epidemiologic work, that there are >75,000 patients living with cutaneous VMs in the United States. Based on this estimated U.S. prevalence, we believe the TAM opportunity on an annualized basis for QTORIN rapamycin in cutaneous VMs is greater than $1.0 billion. Furthermore, the introduction of a new therapy may lead to improved awareness of these diseases, better and sooner diagnosis, and more patients actively seeking therapy.

QTORIN rapamycin for the treatment of Clinically Significant Angiokeratomas

Objective	● There are currently no FDA-approved therapies indicated for the treatment of clinically significant angiokeratomas; we are developing the first targeted therapy ● We believe QTORIN rapamycin, if approved, would be the first approved therapy indicated for Clinically Significant Angiokeratomas
Program Status; Anticipated Upcoming Milestones	● We plan to initiate a Phase 2 study in the second quarter of 2026
Genetic Basis and Molecular Pathways	● VEG-F activation of mTOR signaling
Disease Burden	● Angiokeratomas are associated with significant morbidity, impacts day-to-day functioning, and are associated with risks that dramatically and persistently deteriorate patients' quality of life ● Characterized by dilated vessels that swell and extend beginning in the papillary dermis
Scientific Rationale for Clinically Significant Angiokeratomas	● Angiokeratomas are associated with increased VEG-F signaling along with keratinocyte hyperproliferation driven by hyperactivation of the mTOR pathway, which in turn activates a VEG-F-driven feedback loop, leading to over proliferation, dilation, and hyperkeratosis
Our Targeted Approach	● QTORIN is designed to confer site-directed delivery of rapamycin to the epidermis and dermis ● Estimated prevalence: >50,000 in the United States
Intellectual Property; **Regulatory Designations**	● We hold U.S. patents and patent applications in both the U.S. and major foreign markets with claims directed to anhydrous gel formulations of rapamycin and methods of use for treating clinically significant angiokeratomas, expiring in 2038 ● FDA Fast Track Designation*

**Fast Track Designation may not result in a faster development process, review or approval. Please see paragraph titled "Special FDA Expedited Review and Approval Programs" herein for more information.*

In September 2025, we announced the expansion of our QTORIN rapamycin development program into clinically significant angiokeratomas.

Clinically significant angiokeratomas are superficial lymphatic malformations which can cause bleeding, pain, functional impairment, and risk of infection, with no tendency for spontaneous regression. Angiokeratomas were recently classified as an isolated lymphatic malformation in 2025 by the International Society for the Study of Vascular Anomalies ("ISSVA"). Current treatment options include potentially destructive procedural interventions that carry significant risks of pain, scarring, and recurrence. Despite the substantial disease burden, there are currently no FDA-approved treatments available for clinically significant angiokeratomas.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study of approximately 10-20 patients to evaluate QTORIN rapamycin for the treatment of clinically significant angiokeratomas. Study initiation is anticipated in the second quarter of 2026. We have received Fast Track Designation for QTORIN rapamycin for the treatment of angiokeratomas.

Additional mTOR Driven Diseases

We have identified several other serious, rare skin diseases that are driven by mTOR and available clinical data suggests that inhibition of mTOR may be a good therapeutic target for these conditions. These diseases include but are not limited to refractory vascular tumors, capillary malformations, and cutaneous sarcoidosis. We are currently evaluating several of these opportunities for clinical development.

QTORIN Rapamycin and QTORIN Platform Expansion

We plan to announce the fourth target clinical indication for QTORIN rapamycin in the second half of 2026. We believe expansion of QTORIN rapamycin into additional indications is supported by comprehensive publications which highlight the broad potential of rapamycin in several difficult-to-treat, mTOR-driven skin diseases while advocating for targeted, topical approaches suited to improve tolerability and safety.

We plan to announce the third product candidate from the QTORIN platform in the second half of 2026.

QTORIN Pitavastatin for the Treatment of Disseminated Superficial Actinic Porokeratosis

In November 2025, we announced a new product candidate, QTORIN pitavastatin, for the treatment of DSAP. QTORIN pitavastatin was developed leveraging our QTORIN platform.

DSAP is a premalignant genetic skin disease that presents as persistent, often extensive lesions that enlarge and increase in size, number, and extent over time, causing chronic loss of skin integrity which can severely impact quality-of-life; no FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study to evaluate QTORIN pitavastatin for DSAP. Trial initiation is anticipated in the second half of 2026.

Commercialization Strategy

We intend to build commercial infrastructure in the United States to support the commercialization of our product candidates, if approved. We plan to implement a phased approach to building our commercial team aligned with the progress of our clinical development and advancement towards registration. This approach allows us to grow the organization while appropriately supporting the necessary market development and launch objectives.

The initial focus of our commercial sales effort will be on multidisciplinary care teams at vascular anomaly centers and academic medical centers, many of whom we have established relationships through our clinical development initiatives. We plan to engage these physicians by building an experienced rare disease sales force which will be supported by patient and healthcare provider marketing programs tailored to the indications and communities our products treat. The potential to bring forth new differentiated treatments in rare skin diseases and vascular malformations for which no treatments currently exist will help position us to engage this population of physicians. Over time, we hope to generate operational leverage from our field organization as we expand to potential future rare skin disease and vascular malformation indications.

We expect that the patients who are prescribed our products will be serviced by a highly focused system of programs and resources to support both their access to and appropriate use of our therapies. This will include distribution through specialty pharmacy partners, reimbursement and product administration support through a patient services team trained specifically on the needs of people with rare skin diseases and vascular malformations and access programs aimed at providing copay assistance. These programs and resources will be built specifically with feedback from the individuals with these diseases and malformations and their caregivers. The patient services team will act with the highest levels of integrity and also be highly focused on ensuring that all individuals and physicians who interact with our programs, distribution partners and company have a high level of satisfaction.

To support access to and reimbursement for our therapies, we expect to deploy an experienced market access team to collaboratively engage with payors, provide education regarding the diseases our drugs treat and provide education regarding our value propositions. Value propositions based on clinical data will be key to supporting our pricing strategies. We plan on engaging with payors leading up to the potential product launch and continuing to support ongoing access creation throughout the life cycle of the product, if approved. As we seek to develop and receive regulatory approval for the treatment of new indications for existing product candidates or develop and commercialize new products, once approved, our team will seek to position itself to provide ongoing access and education.

We expect our commercial organization to be complemented by a medical affairs team tasked with appropriately educating clinical decision makers on the scientific data on our products in development, and those that are approved, if applicable. Medical affairs will do this through support of appropriate medical education initiatives, supporting the

publication of relevant data at scientific meetings, executing a publication strategy to disseminate new scientific details of our products, and responding to all incoming requests for medical information. We also plan to identify, where appropriate, the opportunity to support investigator-initiated trials that may expand the scientific body of evidence for our products, and potentially to provide grants to researchers in areas of company interest.

We anticipate that we will be required to invest significant amounts of financial and management resources to develop the appropriate infrastructure to prepare for commercialization. We intend to scale certain investments so that they align with achievement of regulatory milestones, but significant expenditures may be required prior to the receipt of any regulatory approval of our product candidates.

Outside of the United States, we may consider building our own commercial infrastructure or out-licensing, where appropriate, and may elect to utilize strategic collaborators, distributors, or other partners for making our products available to patients.

Manufacturing

While we have personnel with substantial manufacturing experience, we do not own or operate manufacturing facilities for the production of clinical or commercial supply of our product candidates and we currently have no plans to build our own clinical or commercial-scale manufacturing capabilities. We rely on third-party contract manufacturing organizations ("CMOs") to manufacture and supply our materials to be used for the development and commercialization of our current and any future product candidate and expect such reliance to continue for the foreseeable future. We also rely, and expect to continue to rely, on third parties to test, package, label, store and distribute our current and any future product candidate, as well as our commercial products if marketing approval is obtained. We believe that this strategy allows us to maintain a more efficient infrastructure by eliminating the need for us to invest in our own manufacturing facilities, equipment and personnel while also enabling us to focus our core expertise and resources on the development of our current and any future product candidates.

We are currently working with a limited number of third-party CMOs for the manufacture of our clinical supply of our product candidate for clinical trials and for the manufacture of a commercial supply of QTORIN rapamycin, if approved. We obtain supplies of drug substance for our product candidates on a purchase order basis from three sources. As we advance QTORIN rapamycin through development, we will add backup suppliers for drug product manufacture and packaging to protect against any potential supply disruptions.

Certain of our supplies, including our pumps, are obtained from sole source suppliers. For example, we have an agreement with Nemera Le Tréport SAS ("Nemera") for the supply of pumps we use to deliver QTORIN rapamycin. Nemera is a sole source supplier of these pumps, and we are required under a supply agreement to purchase from Nemera.

The use of CMOs and reliance on collaboration partners is cost-efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development. We believe available CMOs are capable of providing sufficient quantities of our product candidate, if approved, to meet anticipated full-scale commercial demands. However, there are a limited number of manufacturers capable of producing our product candidates, particularly our current product candidates which incorporate rapamycin.

Intellectual Property

Our success depends in part on our ability to obtain and maintain proprietary protection for product candidates and any of our future product candidates, core technologies, novel discoveries, product development technologies and know-how; to operate without infringing on the proprietary rights of others; and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing patents and patent applications in the United States and select foreign countries related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also seek to avoid infringing proprietary rights of others. For this reason, we routinely monitor and evaluate third-party patents and publications, and, if necessary, take appropriate action based on that evaluation. In addition, we rely on trademarks, trade secrets, know-how, continuing technological innovation and potential in-licensing opportunities to develop and maintain our proprietary position.

As of March 25, 2026, we own issued U.S. and foreign patents and pending U.S. and foreign patent applications and U.S. provisional applications relating to QTORIN rapamycin and uses thereof. Of these QTORIN rapamycin patents and patent applications:

We own issued patents in the U.S., as well as Europe, Australia, China, Israel and Japan and pending applications in the U.S., Canada, and Europe directed to anhydrous gel formulations of rapamycin and methods of using the same to treat certain skin disorders, including microcystic LM and venous malformations that naturally expire in 2038. We also own issued U.S. patents and pending U.S. applications that encompass anhydrous gel formulations of mTOR inhibitors, including rapamycin, and methods of using the same to treat skin disorders including microcystic LM and venous malformations that naturally expire as early as 2038. We own pending applications in the U.S., Europe and Japan that are directed to the use of QTORIN rapamycin for the treatment of microcystic LM, which if issued, would naturally expire in September 2042. A summary of these patent families is presented in the following table.

	Owned/Licensed	**# Patents and Countries**	**# Applications and Countries**	**Natural Expiry Date**	**Type of Patent**
Anhydrous gel formulations of rapamycin and methods of use	Owned by Palvella	11 patents in U.S., Australia, China, Israel, Europe and Japan	3 pending applications in the U.S., Canada, and Europe	January 2038	Utility
Anhydrous gel formulations of mTOR inhibitors and methods of use	Owned by Palvella	3 U.S. Patents	1 pending U.S. application	As early as January 2038	Utility
Use of QTORIN rapamycin for treating microcystic LM	Owned by Palvella	N/A	3 pending applications in U.S., Europe and Japan	September 2042	Utility
Use of QTORIN rapamycin for treating angiokeratomas	Owned by Palvella	N/A	1 pending U.S. provisional application	September 2046	Provisional

As of March 25, 2026, we exclusively license one allowed U.S. patent application, and we own one pending U.S. provisional application relating to QTORIN pitavastatin and uses thereof. Of these QTORIN pitavastatin patent applications:

We exclusively license from Yale University one allowed U.S. Application directed to the topical administration of an effective amount of a HMG CoA reductase inhibitor, including pitavastatin, to treat porokeratosis. Upon issuance, this application will naturally expire in June 2040. We also own one pending U.S. provisional application directed to formulations of pitavastatin and other HMG CoA reductase inhibitors and methods of using the same to treat certain skin disorders, including porokeratosis that will naturally expire in 2046. A summary of these patent families is presented in the following table.

	Owned/Licensed	**# Patents and Countries**	**# Applications and Countries**	**Natural Expiry Date**	**Type of Patent**
Methods of treating Porokeratosis with HMG CoA Reductase Inhibitors	Licensed from Yale University	N/A	1 pending U.S. application	June 2040	Utility
Topical Formulations of Pitavastatin and methods of use	Owned by Palvella	N/A	1 pending U.S. provisional application	November 2026	Provisional

Patent term is based on the filing or grant date of the patent, as well as the governing law of the country in which the patent is obtained. The protection provided by a patent varies from country to country, and is dependent on the type of patent granted, the scope of the patent claims, and the legal remedies available in a given country.

Obtaining patent protection is not the only method that we employ to protect our propriety rights. We also utilize other forms of intellectual property protection, including trademark and trade secrets, when those other forms are better suited to protect a particular aspect of our intellectual property. Our belief is that our propriety rights are strengthened by our comprehensive approach to intellectual property protection.

Maintaining the confidential nature of our non-publicly disclosed products and technologies is of paramount importance. For this reason, our employees, contractors, consultants, and advisors are required to enter into nondisclosure and invention assignment agreements when their employment or engagement commences. Those individuals also enter into agreements that prohibit the communication or implementation of any third-party proprietary rights during the course of their employment with us. We also require any third-party that may receive our confidential information or materials to enter into CDAs prior to receipt of that information or material. See "*Risk Factors — Risks Related to Intellectual Property*" for more information.

Ligand Development Funding Agreement

We are party to a Development Funding and Royalties Agreement with Ligand Pharmaceuticals, Inc. ("Ligand"), dated December 13, 2018, as amended May 22, 2020 and November 28, 2023 (the "Ligand Agreement"). Under the Ligand Agreement, Ligand has made payments totaling $15.0 million to fund the development of QTORIN rapamycin. As partial consideration for the funding received, we granted Ligand the right to receive up to $8.0 million in milestone payments upon the achievement of certain corporate, financing and regulatory milestones by us related to QTORIN rapamycin for the treatment of any and all indications. In addition, we are currently obligated to pay to Ligand tiered royalties ranging from 8.0% to 9.8% of any aggregate annual worldwide net product sales of any products based on QTORIN rapamycin. On a licensed product-by-licensed product and country-by-country basis, the royalty period is from the date of first commercial sale of such licensed product in a country until the latest of (i) the expiration of the last valid claim within the licensed patent rights covering such licensed product in the country in which such licensed product is made, used or sold, (ii) the expiration of the regulatory exclusivity term conferred by the applicable regulatory authority in such country with respect to such licensed product, and (iii) the fifteenth anniversary of the first commercial sale of such licensed product in such country.

The Ligand Agreement may be terminated by the earlier of a mutual written agreement of the parties or when the royalties contemplated by the agreement are paid to Ligand. Additionally, Ligand may terminate the agreement for (i) any or no reason upon a 90-day notice to us, or (ii) cause in connection with a material breach that we do not cure within a certain period of time.

The total amount of potential future milestone payments remaining under the arrangement were $5.0 million as of December 31, 2025 and 2024. The potential future milestone payments represent derivative liabilities with a fair value of $2.0 million and $1.6 million as of December 31, 2025 and 2024, respectively, which are classified as "derivative liabilities – royalty agreement" on the consolidated balance sheets appearing elsewhere in this Form 10-K.

Our obligation to pay tiered royalties under the Ligand Agreement was determined to be a debt instrument based on the likelihood of repaying the amounts provided to fund the development of QTORIN rapamycin and that we have significant continuing involvement in the generation of the cash flows potentially due to Ligand. This obligation is reflected as royalty agreement liability which is classified as a long-term liability on the consolidated balance sheets and was $17.8 million and $11.9 million as of December 31, 2025 and 2024, respectively, appearing elsewhere in this Form 10-K. Interest expense with respect to the royalty agreement liability is determined using the effective interest method based upon probability-adjusted cash flow estimates of our potential future royalty payments under the Ligand Agreement, yielding an effective interest rate of 44.9% and 39.9% as of December 31, 2025 and 2024, respectively. Changes in these estimates impact the amount of interest expense recognized through the accompanying consolidated statements of operations. During the second quarter of 2024, we received data from certain of our clinical trials that reduced the projected net product sales related to QTORIN rapamycin and the corresponding probabilities of successful commercialization, resulting in a significant reduction in the future royalty agreement liability. In addition, in the fourth quarter of 2024,we began conducting a Phase 3 clinical trial in microcystic lymphatic malformations. In the fourth quarter of 2025, we reported positive topline data for our Phase 2 clinical trial for cutaneous venous

malformations and increased the corresponding probability of successful commercialization. We incurred non-cash interest expense of $5.8 million and $3.9 million for the years ended December 31, 2025 and 2024, respectively, all of which is a component of the royalty agreement liability on the consolidated balance sheets appearing elsewhere in this Form 10-K.

The Ligand Agreement requires us to make certain estimates and assumptions about future development, FDA approval, commercialization, and net sales of any product containing QTORIN rapamycin. These estimates and assumptions are subject to significant variability and are thus subject to significant uncertainty. Therefore, these estimates and assumptions are likely to change as we develop and commercialize products containing QTORIN rapamycin that may result in significant future adjustments to the royalty agreement liability, the derivative liabilities, and the accretion of interest expense.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition, and a strong emphasis on proprietary products. To the best of our knowledge, potential competitors with product candidates in development for serious, rare skin diseases and vascular malformations include Kaken Pharmaceutical Co., Ltd., Nobelpharma Co., Ltd., Novartis Pharmaceuticals, Protara Therapeutics, Inc., Relay Therapeutics, Inc., Vaderis Therapeutics AG, and Quoin Pharmaceuticals. While we believe that our technology, expertise, scientific knowledge and intellectual property provide us with competitive advantages, we face and will continue to face competition from these companies and other sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, governmental agencies and public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization. Any product candidates that we successfully develop and, if approved, commercialize may compete with existing therapies or procedures and new therapies that may become available in the future. Moreover, our industry is characterized by the existence of large numbers of patents and frequent allegations of patent infringement.

There are no FDA-approved pharmacotherapies currently available for the treatment of microcystic LMs, cutaneous VMs, clinically significant angiokeratomas or DSAP. The current treatment options for microcystic LMs include a high-risk surgical procedure and off-label use of sclerosants, including doxycycline, bleomycin, ethanol and sodium tetradecyl sulfate. The current treatment options for cutaneous VMs include conservative approaches such as observation and compression therapy, as well as interventional techniques like sclerotherapy, laser therapy and surgical excision for larger or symptomatic malformations. The current treatment options for clinically significant angiokeratomas are limited to potentially destructive procedural interventions that carry meaningful risks of pain, scarring, and recurrence. There are currently no FDA-approved therapies for DSAP. There are a number of drug development companies and academic researchers exploring oral and topical formulations of various agents for the treatment of LMs and VMs including macrolides, phosphodiesterase inhibitors, PI3K inhibitors, AKT inhibitors, and mTOR inhibitors. A majority of these are in early development.

The key competitive factors affecting the commercial success of our current or future product candidates, if approved, are likely to be their efficacy, safety, convenience and price, the level of competition and the availability of coverage and adequate reimbursement from third-party payors. If any of our product candidates are approved and successfully commercialized, it is likely that we will face increased competition as a result of other companies pursuing development of products to address the same or similar diseases.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We may pursue the in-license or acquisition of rights to complementary technologies and product candidates on an opportunistic basis. The acquisition and licensing of technologies and product candidates is a competitive area, and a number of more established companies also have similar strategies to in-license or acquire technologies and product candidates that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to in-license or acquire the relevant technology or product candidate on terms that would allow us to make an appropriate return on our investment.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any product candidates that we may develop, and, if approved, successfully commercialize. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for our own, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected by insurers or other third-party payors seeking to encourage the use of generic products. Because of our primary focus on rare diseases, if our product candidates achieve marketing approval, we expect to seek specialty pricing which reflects the relatively small patient populations for our drugs.

In addition, some of the market demand for topical rapamycin and pitavastatin may be satisfied by compounding pharmacies. Although such pharmacies will be unable to compound any drug that is essentially a copy of QTORIN rapamycin or QTORIN pitavastatin, if approved, a compounded product would not be considered a copy of QTORIN rapamycin or QTORIN pitavastatin if there were a difference between our product and the compounded product that was made for an individual patient and which the prescribing practitioner determines produces a significant difference for that patient. Physicians may determine that such differences exist for some or all of their patients and may choose to prescribe compounded rapamycin or pitavastatin provided rapamycin or pitavastatin appears on a list established by the FDA of bulk drug substances for which there is a clinical need or satisfies other limited conditions. In the event compounders are authorized to compound rapamycin or pitavastatin products following approval of QTORIN rapamycin or QTORIN pitavastatin, if approved, we could be subject to significant competition from those formulations.

Government Regulation

Government authorities in the United States, at the federal, state, and local level, and in other countries and jurisdictions, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, pricing, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in other countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

FDA Review and Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA under the U.S. Federal Food, Drug, and Cosmetic Act ("FDCA") and other federal and state statutes and regulations. The failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as a clinical hold, the FDA's refusal to approve pending NDAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Pharmaceutical product development for a new product or certain changes to an approved product in the U.S. typically involves laboratory and animal tests (referred to as preclinical or nonclinical studies), the submission to the FDA of an Investigational New Drug Application, or "IND", which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation, and toxicity, as well as laboratory and animal studies to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including Good Laboratory Practices information about product chemistry, manufacturing and controls ("CMC"), and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted. A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans.

If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. Clinical trials involve the administration of the product candidate to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in compliance with good clinical practices ("GCPs"), an international standard meant to protect the rights, safety and welfare of research participants and to define the roles of clinical trial sponsors, administrators, and monitors; as well as (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety - including the capture and reporting of all serious adverse events occurring during the trial regardless of whether such events are assessed as related to the investigational product - and the effectiveness criteria to be evaluated. In addition, clinical trial protocols require the capture and monitoring of all adverse events, including serious adverse events, that occur during the clinical trial regardless of their suspected relationship to the investigational product. Each protocol involving testing on U.S. participants and subsequent protocol amendments must be submitted to the FDA as part of the IND. While the IND is active, progress reports summarizing the results, if known, of the clinical trials and preclinical studies performed since the last progress report, among other information, must be submitted at least annually to the FDA, and written expedited IND safety reports must be submitted to the FDA and clinical investigators in certain applicable circumstances.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial participants. The imposition of a clinical hold may be full or partial. The study protocol and informed consent information for patients in clinical trials must also be submitted to an Institutional Review Board ("IRB") for review and approval before each trial begins. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap or be combined. In Phase 1, the initial introduction of the drug into healthy volunteers or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses, and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to evaluate the effectiveness of the drug for a particular indication, determine optional dose and regimen, and to identify common adverse effects and safety risks. If a drug demonstrates initial evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, one or more Phase 3 trials are undertaken to obtain additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In many cases, particularly for prevalent diseases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy and safety of the drug. In many other conditions, particularly for rare diseases, a single Phase 3 or Phase 2 trial may be sufficient in conjunction with confirmatory evidence. A single adequate and well-controlled Phase 3 or Phase 2 trial may also be sufficient, though it is less common when the trial is a large, multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible, or the trial is supported by other confirmatory evidence. In February 2026, FDA leadership announced a plan to adopt the default position that one adequate and well-controlled trial, combined with confirmatory evidence, can serve as the basis of marketing authorization for novel products. Approval on the basis of a single trial may be subject to a requirement for additional post-approval studies.

The sponsor of an investigational drug in a Phase 2 or Phase 3 clinical trial for a serious or life-threatening disease is required to disclose, such as by posting on its website, its policy on evaluating and responding to requests for expanded access to such investigational drug. Sponsors are also required to make their expanded access policies publicly

available, within 15 days, if their product candidate receives Fast Track Designation or Breakthrough Therapy Designation by the FDA.

Additionally, clinical trials may utilize an "open-label" trial design. An "open-label" clinical trial is one where both the patient and the investigator know whether the patient is receiving the investigational drug or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational drug and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with product candidates when studied in a controlled environment with a placebo or active control.

Concurrent with clinical trials, sponsors usually complete additional nonclinical studies, and also must develop additional information about the chemistry and physical characteristics of the product candidate, and as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product and, among other things, sponsors must develop methods for testing the identity, strength, quality, potency, and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the investigational drugs do not undergo unacceptable deterioration over their shelf life.

Assuming successful completion of the required nonclinical and clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting an NDA is substantial. Furthermore, under the Prescription Drug User Fee Act ("PDUFA"), the submission of most NDAs is additionally subject to a substantial application user fee, and the applicant under an approved NDA is also subject to an annual program fee for each prescription product. These fees are typically increased annually. An NDA for a drug that has received Orphan Drug Designation is not subject to an application fee, unless the NDA includes an indication for other than a rare disease or condition.

The FDA has 60 days from its receipt of an NDA to conduct a preliminary review and determine whether the application will be filed based on the agency's threshold determination that it is sufficiently complete to permit substantive review. If the FDA determines the application is incomplete because it does not on its face contain required information, the FDA may refuse to file the application and request additional information rather than file an NDA. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to preliminary review before the FDA files it. Once the application is filed, the FDA begins an in-depth review. Under PDUFA, the FDA has agreed to certain performance goals in the review of NDAs to encourage timeliness. For applications subject to standard review, the FDA's current performance goals under PDUFA are ten months from receipt for most NDAs and twelve months for certain applications, including some new molecular entities ("NMEs"). For applications granted Priority Review, the FDA's performance goal is six months from the receipt date. Priority Review can be applied to drugs that the FDA determines offer major advances in treatment or provide a treatment where no adequate therapy exists. NDAs for most Priority Review drug products are reviewed within eight months from submission of NDAs for NMEs and six months from submission of NDAs for non-NMEs. The review process for both standard and Priority Review may be extended by the FDA for three months to consider information the FDA considers to be a major amendment to the NDA.

In the past, the FDA has referred applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an outside advisory committee — typically a panel that includes clinicians, statisticians and other experts — for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with cGMPs is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the claimed indication.

After the FDA evaluates the NDA and completes clinical and manufacturing site inspections, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the NDA and may require substantial additional testing, or information, in order for the FDA to reconsider the application. The applicant may either resubmit the NDA, addressing all the deficiencies identified in the letter, or withdraw the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with such additional data and information, the FDA may decide that the resubmitted NDA does not satisfy the criteria for approval. An approval letter authorizes commercial marketing of the drug with specific prescribing information for one or more specific indications. The FDA may require that certain contraindications, warnings, precautions or interactions be included in the product labeling, and as a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy ("REMS") to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use ("ETASU"). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, the FDA may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained, or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and the FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing original NDAs.

Special FDA Expedited Review and Approval Programs

The FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment, and which demonstrate the potential to address unmet medical needs for the disease. The FDA has various programs, including Fast Track Designation, priority review, accelerated approval, and Breakthrough Therapy Designation, the purpose of which is to provide important new drugs or biologics to patients earlier than under standard FDA review procedures.

Fast Track Designation

The Fast Track Designation program is intended to expedite the process for developing and reviewing product candidates that are intended to treat a serious or life-threatening disease or condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the disease or condition. The sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a Fast Track drug concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for Fast Track Designation within 60 days of receipt of the sponsor's request. Fast Track Designation may not result in a faster development process, review or approval compared to conventional FDA procedures. The FDA

may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from the clinical development program.

If a development product is granted Fast Track Designation, the sponsor may engage in more frequent interactions with the FDA may be eligible for Priority Review. In addition, the FDA may review sections of the NDA on a rolling basis before the application is complete. This rolling review is available if the applicant provides, and the FDA approves, a schedule for the submission of the sections of the NDA, and the applicant pays applicable user fees upon submission of the first section of the NDA. However, the FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted.

Breakthrough Therapy Designation

The Breakthrough Therapy Designation is a program by the FDA that aims to expedite the development and review of drugs and biologics for serious or life-threatening conditions.

To qualify for Breakthrough Therapy Designation, a drug must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

A Breakthrough Therapy Designation includes all of the features of Fast Track Designation, as well as intensive guidance on an efficient drug development program, organizational commitment involving senior FDA managers, and eligibility for rolling review and Priority Review, if relevant criteria are met. The FDA may rescind the designation if subsequent data no longer support the designation.

Priority Review

The FDA may grant Priority Review to drugs that are intended to treat a serious condition, and if approved, would provide a significant improvement in safety or efficacy compared to available therapies. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. These six- and ten-month review periods are measured from the "filing" date rather than the receipt date for NDAs for NMEs, which typically adds approximately two months to the timeline for review and decision from the date of submission. Most products that are eligible for Fast Track Designation and Breakthrough Therapy Designation are also likely to be considered appropriate to receive a Priority Review.

Accelerated Approval

In addition, products intended for treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality ("IMM"), that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the disease and the availability or lack of alternative treatments. As a condition of approval, the FDA generally requires a sponsor of a drug receiving accelerated approval to perform post-marketing studies, which must be conducted with due diligence, to verify and describe the predicted effect on IMM or other clinical endpoint. The FDA is permitted to require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. The FDA also has the authority to withdraw approval of a drug granted accelerated approval on an expedited basis if the sponsor fails to conduct such trials in a timely manner, send the necessary updates to the FDA, or if such post-approval confirmatory trials fail to verify the drug's predicted clinical benefit. The FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory study or submit timely reports to the agency on their progress.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition. This generally means a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan Drug Designation must be requested before submitting an NDA. After the FDA grants Orphan Drug Designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan Drug Designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with the FDA Orphan Drug Designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that orphan indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same orphan indication, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. A product can be considered clinically superior if it is safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other potential benefits of Orphan Drug Designation are tax credits for certain qualified clinical testing expenses and, in some circumstances, exemption from the NDA application user fee; however, these benefits are subject to statutory and regulatory limitations and may be modified by future legislation

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA regulated products, including drugs, are required to register and disclose certain clinical trial information within specific timeframes for publication in the ClinicalTrials.gov database. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration of the clinical trial. Sponsors are also obligated to disclose certain results of their clinical trials after completion, although such disclosure can be delayed in certain circumstances for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs as well as clinical trial design.

Pediatric Information

Under the Pediatric Research Equity Act ("PREA"), certain NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant full or partial waivers, or deferrals, for submission of such data. With certain exceptions, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The Best Pharmaceuticals for Children Act ("BPCA"), provides NDA holders a six-month extension of any exclusivity — patent or nonpatent regulatory exclusivity— for a drug if certain conditions are met. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric studies, and the applicant agreeing to perform, and timely submit study responding to the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

Post-Approval Requirements

Once an NDA is approved, a product will be subject to numerous post-approval requirements, including, among other things, record-keeping requirements, providing the FDA with updated safety information, product sampling and distribution requirements, and promotion and advertising requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. Drugs may be marketed or promoted only for the approved indication(s) and in accordance with the provisions of the approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including investigation by federal and state authorities.

Adverse event reporting and submission of periodic reports are required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, and surveillance to monitor the effects

of an approved product, or the FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and notify the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, manufacturers must continue to expend time, money, and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals, request product recalls, or take other administrative or judicial enforcement actions if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

The Hatch-Waxman Amendments

Orange Book Listing

NDA applicants are required to list with the FDA each patent whose claims cover the applicant's drug or approved method of using the drug. Upon approval of a drug, each of the patents listed for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be referenced by potential generic competitors in support of approval of an Abbreviated New Drug Application ("ANDA"). An ANDA provides for marketing of a drug product that has the same active ingredients, strengths, and routes of administration in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic" or "therapeutic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug pursuant to each state's laws on drug substitution.

An ANDA applicant is required to provide a certification to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify to each patent in one of the following ways: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid, unenforceable or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain any language regarding the patented method-of-use (such use is "carved-out") rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired. A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to any listed patent, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been received for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity

Upon NDA approval of a drug that contains an active moiety that has been previously approved by the FDA in any other NDA, when the application contains reports of a new clinical investigations (other than bioavailability studies) conducted by the applicant that were essential to approval, that drug product receives three years of exclusivity. During this three-year period of exclusivity, the FDA may not approve any Section 505(b)(2) NDA or ANDA seeking approval of a version of that drug that includes the same conditions of use approved in the relevant NDA.

Section 505(b)(2) NDAs

A special type of NDA, commonly referred to as a Section 505(b)(2) NDA, enables the applicant in certain circumstances to rely, in part, on the FDA's prior findings in approving a similar product or published literature in support of its application. A Section 505(b)(2) NDA may provide an alternate path to FDA approval for a new or improved formulation, a new route of administration, or a new use of a previously approved product.

Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the Section 505(b)(2) applicant can establish that reliance on the FDA's prior findings of safety and/or effectiveness is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all, or some, of the indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) NDA applicant is relying on the FDA's prior findings of safety or effectiveness for an already approved product, the applicant is required to certify any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus, approval of a Section 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as five-year exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Combination Products

A combination product is a product comprised of two or more regulated components, e.g., drug and medical device, that are physically combined and produced as a single entity, packaged together in a single package, or packaged separately but intended to be labeled for use together.

The FDA is divided into various branches, or Centers, by product type. Different Centers typically review drug, biologic, or device applications. In order to review an application for a combination product, the FDA must decide which Center should be responsible for the review. FDA regulations require that the FDA determine the combination product's primary mode of action, or PMOA, which is the single mode of a combination product that provides the most important therapeutic action of the combination product. The Center that regulates that portion of the product that generates the PMOA becomes the lead evaluator. If there are two independent modes of action, neither of which is subordinate to the other, the FDA makes a determination as to which Center to assign the product based on consistency with other combination products raising similar types of safety and effectiveness questions or to the Center with the most expertise in evaluating the most significant safety and effectiveness questions raised by the combination product. When evaluating an application, a lead Center may consult other Centers but still retain complete reviewing authority, or it may collaborate with another Center, by which the Center assigns review of a specific section of the application to another Center, delegating its review authority for that section. Typically, the FDA requires a single marketing application submitted to the Center selected to be the lead evaluator, although the agency has the discretion to require separate applications to more than one Center.

Medical Device Products

The FDCA classifies medical devices into one of three categories – Class I, Class II, or Class III - depending on the risks associated with the device and the level of control necessary to provide reasonable assurance of safety and effectiveness.

Most medical devices can be legally sold within the United States only if the FDA has: (i) approved a premarket approval application ("PMA"), prior to marketing, generally applicable to Class III devices; or (ii) cleared the device in response to a premarket notification ("510(k)") submission, generally applicable to Class I and II devices. However, most Class I and some Class II devices can be marketed without prior FDA authorization. If a device falls into a generic category of Class I or Class II devices that the FDA has exempted by regulation, a 510(k) submission is not required before marketing the device in the United States. Some 510(k)-exempt devices are also exempt from Quality Management System Regulation ("QMSR") requirements.

After a device is placed on the market, numerous regulatory requirements apply. These include: the QSR, labeling regulations, the FDA's general prohibition against promoting products for unapproved or "off-label" uses, reporting to the FDA by a manufacturer if their device may have caused or contributed to a death or serious injury or malfunctioned in a way that would likely cause or contribute to a death or serious injury if it were to recur, and reporting to the FDA by a manufacturer any recalls and field actions to reduce a risk to health posed by the device or to remedy a violation of the FDCA, among other things.

The FDA enforces these requirements by inspection and market surveillance. If the FDA finds a violation, it can institute a wide variety of enforcement actions, ranging from a public warning letter to more severe sanctions such as: fines, injunctions, and civil penalties; recall or seizure of products; operating restrictions, partial suspension or total shutdown of production; refusing requests for 510(k) clearance or PMA approval of new products; withdrawing 510(k) clearance or PMA approvals already granted; and criminal prosecution.

U.S. Anti-Kickback, False Claims and Other Healthcare Fraud and Abuse Laws

In the United States, there are federal and state anti-kickback laws that prohibit offering, the payment, solicitation, or receipt of kickbacks, bribes or other remuneration intended to induce the purchase or recommendation of healthcare products and services. Violations of these laws can lead to civil and criminal penalties, including exclusion from participation in federal healthcare programs. These laws apply to manufacturers of products, such as us, with respect to our financial relationship with hospitals, physicians and other potential purchasers or acquirers of our products. The U.S. government has published regulations that identify "safe harbors" or exemptions for certain practices from enforcement actions under the federal anti-kickback statute, and we will seek to comply with the safe harbors where possible. To qualify for a safe harbor, the activity must fit squarely within the safe harbor. Arrangements that do not meet a safe harbor are not necessarily illegal but must be evaluated on a case-by-case basis. A person or entity may be found to violate the anti-kickback statute even absent actual knowledge of this statute or specific intent to violate it. In addition, the government may assert that a claim that includes items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act ("FCA").

The civil FCA prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment to, or approval by, the federal government, knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or avoiding, decreasing, or concealing an obligation to pay money to the federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. The civil FCA has been used to assert liability on the basis of kickbacks and other improper referrals, improper use of Medicare provider or supplier numbers when detailing a provider of services, improper promotion of off-label uses not covered by a device's clearance or approval, and allegations as to misrepresentations with respect to products, contract requirements, and services rendered. In addition, private payors have been filing follow-on lawsuits alleging fraudulent misrepresentation, although establishing liability and damages in these cases is more difficult than under the FCA. Intent to deceive is not required to establish liability under the civil FCA. Civil FCA actions may be brought by the government or may be brought by private individuals on behalf of the government, called "qui tam" actions. If the government decides to intervene in a qui tam action and prevails in the lawsuit, the individual will share in the proceeds from any fines or settlement funds. If the government declines to intervene, the individual may pursue the case alone. The civil FCA provides for treble damages and a civil penalty for each false claim, such as an invoice or pharmacy claim for reimbursement, which can aggregate into millions of dollars. For these reasons, FCA lawsuits against biopharmaceutical and device companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements, as much as $3.0 billion, regarding certain sales practices and promoting off label uses. Civil FCA liability may further be imposed for known Medicare or Medicaid overpayments that are not refunded within 60 days of discovering the overpayment, even if the overpayment was not caused by a false or fraudulent act. In addition, conviction or civil judgment for violating the FCA may result in exclusion from federal health care programs, and suspension and debarment from government contracts, and refusal of orders under existing government contracts.

The government may further prosecute conduct constituting a false claim under the criminal FCA. The criminal FCA prohibits the making or presenting of a claim to the government knowing such claim to be false, fictitious, or fraudulent and, unlike the civil FCA, requires proof of intent to submit a false claim.

The civil monetary penalties statute is another statute under which medical device companies may potentially be subject to enforcement. Among other things, the civil monetary penalties statue imposes fines against any person who offers to provide remuneration to any individual eligible for benefits under Medicare or Medicaid that the offeror knows or should know is likely to influence the individual to order or receive from a particular provider or supplier of any item or service reimbursable under those programs.

The federal Health Insurance Portability and Accountability Act ("HIPAA") statute also created federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, a healthcare benefit program, regardless of whether the payor is public or private, in connection with the delivery or payment for health care benefits, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items, or services relating to healthcare matters. Additionally, the Affordable Care Act amended the intent requirement of certain of these criminal statutes under HIPAA so that a person or entity no longer needs to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation.

Further, federal laws and many states require that pharmaceutical manufacturers report on a periodic basis pricing information related to their products.

The Sunshine Act requires applicable device and drug manufacturers of covered products to report annually to the Centers for Medicare and Medicaid Services ("CMS") any payments or other transfers of value to certain health care providers, as well as ownership and investment interests held by physicians and their immediate family members.

Further, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH") and its respective implementing regulations imposes certain requirements on covered entities relating to the privacy, security, and transmission of certain individually identifiable health information, known as protected health information. Among other things, HITECH, through its implementing regulations, makes HIPAA's security standards and certain privacy standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains, or transmits protected health information on behalf of a covered entity for a function or activity regulated by HIPAA. HITECH also strengthened the civil and criminal penalties that may be imposed against covered entities, business associates, and individuals, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, other federal and state laws may govern the privacy and security of health and other information in certain circumstances, many of which differ from each other in significant ways and may not be preempted by HIPAA, thus complicating compliance efforts.

Many states have also adopted laws similar to each of the above federal laws, which may be broader in scope and apply to items or services reimbursed by any third-party payor, including commercial insurers. Certain states also require implementation of commercial compliance programs and compliance with the medical device industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government, or otherwise restrict payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; impose restrictions on marketing practices; or require companies to track and report information related to payments, and other items of value to physicians and other healthcare providers.

If our operations are found to be in violation of any of the laws or regulations described above or any other applicable laws, we may be subject to penalties or other enforcement actions, including criminal and significant civil monetary penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, corporate integrity agreements, suspension and debarment from government contracts, and refusal of orders under existing government contracts, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Enforcement actions can be brought by federal or state governments, or as "qui tam" actions brought by individual whistleblowers in the name of the government under the civil FCA if the violations are alleged to have caused the government to pay a false or fraudulent claim.

To the extent that any of our products are sold outside the U.S., we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act ("FCPA") generally prohibits offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a government official employed by a non-U.S. country in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our industry is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. Additionally, in many other countries, the health care providers who prescribe pharmaceuticals and medical devices are employed by their government, and the purchasers are government entities; therefore, our dealings with these prescribers and purchasers are subject to regulation under the FCPA. The SEC and Department of Justice ("DOJ") have increased their FCPA enforcement activities with respect to pharmaceutical and medical device companies. Violations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of our facilities, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Enforcement actions may be brought by the DOJ and SEC, and legislation has expanded the SEC's power to seek disgorgement in all FCPA cases filed in federal court and extended the statute of limitations in SEC enforcement actions in intent-based claims such as those under the FCPA from five years to ten years.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product that receives regulatory approval. In the United States, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities and health programs in the United States such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all FDA-approved drugs for a particular indication. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. While we cannot predict whether any proposed cost-containment measures will be adopted or otherwise implemented in the future, the impact of changes in agency leadership, or whether the Trump Administration or future presidential administrations, may propose additional regulatory reforms, these requirements or any announcement or adoption of such proposals may prevent us from obtaining adequate prices for our product candidates, if approved.

A payor's decision to provide coverage for a drug product does not imply that patients will be able to easily access the medication, as step edits, prior authorizations, and other utilization management approaches can be used that have the effect of limiting patient access to certain medications. Further, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to support physicians by providing scientific evidence and support for the use of any Palvella product which receives marketing authorization to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. New metrics frequently are used as the basis

for reimbursement rates, such as average sales price, average manufacturer price and actual acquisition cost. In order to obtain coverage and reimbursement for any product that might be approved for sale, it may be necessary to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the products, in addition to the costs required to obtain regulatory approvals. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us or our partners to sell our products at a profit.

The marketability of any of our current or any future product candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. European Union member states may approve a specific price for a product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to set their own prices for products but monitor and control company profits. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations may not allow favorable reimbursement and pricing arrangements.

Healthcare Reform

The United States and many other jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system, including implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs.

There have been, and continue to be, significant judicial, administrative, executive and legislative efforts by the federal government, state governments, regulators and third-party payors to control or manage the increased costs of health care and, more generally, to reform the U.S. healthcare system. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in March 2010, the Affordable Care Act was enacted, which was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry.

Several healthcare reform proposals culminated in the enactment of the Inflation Reduction Act of 2022 (“IRA”) for example, contains substantial drug pricing and other reforms to Medicare’s coverage of pharmaceuticals. Among other things, the IRA eliminated, beginning in 2025, the coverage gap under Medicare Part D by significantly lowering the enrollee maximum out-of-pocket cost and requiring manufacturers to subsidize, through a newly established manufacturer discount program (replacing the former Coverage Gap Discount Program), 10% of Part D enrollees’ prescription costs for brand drugs below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. The IRA also requires HHS to negotiate the selling price of a statutorily specified number of drugs and biologics each year that CMS reimburses under Medicare Part B and Part D. The negotiated price may not exceed a statutory ceiling price. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) are eligible to be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. In addition, CMS selected and announced the negotiated maximum fair

price for 15 additional Medicare Part D drugs, which will become effective in 2027. For 2028, an additional 15 drugs, comprised of drugs covered under Medicare Part D and, for the first time, drugs payable under Medicare Part B. For 2029 and subsequent years, 20 Part B or Part D drugs will be selected. Due to a statutory amendment in July 2025, a drug or biological product that has one or more Orphan Drug Designations will be excluded from the IRA's price negotiation requirements, but will lose that exclusion if it is approved for an indication that is not one of the designated rare diseases or conditions, unless such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation.

The IRA also imposes rebates on Medicare Part B and Part D drugs whose prices have increased at a rate greater than the rate of inflation and in November 2024, CMS finalized regulations for these inflation rebates. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. These provisions may be subject to legal challenges. For example, the provisions related to the negotiation of selling prices of high-expenditure single-source drugs and biologics have been challenged in multiple lawsuits brought by pharmaceutical manufacturers. The outcome of these lawsuits is uncertain. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry and the pricing of prescription drug products.

In addition, the One Big Beautiful Bill Act of 2025 ("OBBBA") imposed significant reductions in Medicaid funding, additional work requirements for Medicaid recipients, and more frequent reenrollment requirements. These changes are expected to place substantial pressure on state Medicaid budgets, reduce enrollment, and limit covered services, which could decrease utilization of, and reimbursement for, our products, if approved.

Furthermore, the Trump Administration has issued executive orders and supported proposed regulatory initiatives in 2025 that could have a significant impact on the prices that we, or any collaborators, may receive for any approved products.

For example, on May 12, 2025, President Trump signed an executive order directing the Secretary of HHS to set and communicate most-favored-nation ("MFN") price targets to manufacturers and propose a rulemaking plan to impose MFN pricing if "significant progress" is not made, and also directing the federal government to support regulatory paths to allow direct-to-patient sales for companies that meet these targets. The executive order further states that the Administration will take additional action (for example, examining whether marketing approvals should be modified or rescinded or considering individual drug importation waiver authorities) should manufacturers fail to offer American consumers the MFN lowest price. In July 2025, President Trump sent letters to certain pharmaceutical companies demanding that these companies extend MFN pricing to Medicaid and newly launched drugs as well as move to direct-to-consumer models priced at MFN pricing, and soliciting binding commitments by September 29, 2025. Since this time, multiple drug manufacturers have announced plans to, for certain of their drugs, lower prices to reflect similar pricing around the world, and to sell these reduced-price drugs on a direct-to-consumer purchasing platform developed by the federal government; however, it is not known what results will occur to the extent the recipients of these letters do not reduce their U.S. prices.

On December 19, 2025, CMS released two proposed rules that would incorporate MFN pricing principles into federal reimbursement for prescription drugs. The first proposal, the Global Benchmark for Efficient Drug Pricing Model ("GLOBE") for Medicare Part B, would require manufacturers of specified single source drugs and sole source biologics to pay incremental rebates based on international benchmark prices, with participation triggered for products meeting CMS's spending and eligibility criteria. The second proposal, the Guarding U.S. Medicare Against Rising Drug Costs ("GUARD") model for Medicare Part D, would similarly mandate manufacturer rebates for qualifying sole source drugs where the Medicare net price exceeds an MFN benchmark derived from international reference pricing methodologies. As proposed, GLOBE would begin a five year performance period on October 1, 2026 and GUARD would begin its performance period in 2027. These proposals will likely be subject to legal challenges that could delay their implementation or modify their impact on manufacturer pricing and revenue. Additionally, in November 2025, CMS introduced the GENErating cost Reductions fOr U.S. Medicaid ("GENEROUS") Model, a voluntary MFN framework for manufacturers participating in the Medicaid Drug Rebate Program. Although it is voluntary, the GENEROUS Model could also impact the drug pricing landscape for manufacturers.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain

product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Additional federal and state healthcare reform measures may be adopted in the future.

The effect of these healthcare reform initiatives on our business and the pharmaceutical industry in general is not yet known, but could be substantial and materially adverse to our ability to successfully commercialize our product candidates at profitable price points.

Foreign Regulatory Requirements

We may be subject to widely varying foreign regulations, which may be quite different from those of the FDA, governing clinical trials, manufacturing, product registration and approval, pharmaceutical sales and data protection. Whether or not FDA approval has been obtained, we must obtain a separate approval for a product by the comparable regulatory authorities of non-U.S. countries prior to the commencement of product marketing in these countries. In certain countries, regulatory authorities also establish pricing and reimbursement criteria. The approval process varies from country to country, and the time may be longer or shorter than that required for FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

European Union Drug Development and Approval

The process governing approval of medicinal products in the EU generally follows the same lines as in the US. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires submission to the relevant competent authorities of a marketing authorization application and granting of a marketing authorization by these authorities before the product can be marketed and sold in the EU.

Clinical Trial Approval

In April 2014, the EU adopted the new Clinical Trials Regulation (EU) No 536/2014, or Clinical Trials Regulation, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022. All clinical trials in the EU must now be conducted in accordance with the Clinical Trials Regulation. The Clinical Trials Regulation aims at simplifying and streamlining the approval of clinical trials in the EU, for example, it provides for a streamlined application procedure via a single-entry point, rules on the protection of subjects and informed consent, transparency requirements, and strictly defined deadlines for the assessment of clinical trial applications.

Marketing Authorization

To obtain a marketing authorization for a product in the European Economic Area (i.e., the EU as well as Iceland, Liechtenstein and Norway), or EEA, an applicant must submit a marketing authorization application either under a centralized procedure administered by the EMA, or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the EEA.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid throughout EEA. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (*i.e.* gene therapy, somatic-cell therapy and tissue-engineered medicines) and products with a new active substance indicated for the treatment of certain diseases, including HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases, products that are a significant therapeutic, scientific or technical innovation, or products for which authorization would be the interest of public health at EU level, the centralized procedure is optional.

Under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use, or CHMP, is responsible for conducting the initial assessment of a product and for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the maximum timeframe for the EMA's scientific evaluation of a marketing authorization application is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Clock stops may extend the timeframe of evaluation of a marketing authorization application considerably beyond 210 days. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days (excluding clock stops) but it is possible that the CHMP can revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. At the end of this period, the CHMP provides a scientific opinion on whether or not a marketing authorization should be granted in relation to a medicinal product. Where the CHMP gives a positive opinion, the EMA provides the opinion together with supporting documentation to the European Commission, who makes the final decision to grant a marketing authorization, which is issued within 67 days of receipt of the EMA's recommendation.

The decentralized marketing authorization procedure allows an applicant to apply for simultaneous authorization in more than one EU Member State of medicinal products that have not yet been authorized in any EU Member State and that do not fall within the mandatory scope of the centralized procedure.

The mutual recognition procedure is based on the acceptance by the competent authorities of the EU Member States of the marketing authorization of a medicinal product by the competent authorities of another EU Member State. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

Regulatory Data Protection in the EU

In the EU, innovative medicinal products approved on the basis of a complete and independent data package (i.e. reference products) qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. Data exclusivity prevents applicants for authorization of generics or biosimilars of these innovative products from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization, for a period of eight years from the date on which the reference product was first authorized in the EU. During an additional two-year period of market exclusivity, a generic or biosimilar marketing authorization application can be submitted and authorized, and the innovator's data may be referenced, but no generic or biosimilar medicinal product can be placed on the EU market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be an innovative medical product so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing authorization based on a marketing authorization application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Orphan Drug Designation and Exclusivity

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000, provides that a medicinal product can be designated as an orphan medicinal product by the European Commission (following an assessment at the EMA) if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (i) such condition affects no more than five in ten thousand persons in the EU when the application is made, or (ii) without incentives it is unlikely that the marketing of the product in the EU would generate sufficient return to justify the necessary investment in its development; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the product will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to ten years of market exclusivity in all EU Member States and a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure and a reduction or elimination of registration and marketing authorization fees. During the period of market exclusivity, a marketing authorization may only be granted for a "similar medicinal product" with the same orphan indication as an authorized orphan medicinal product only if: (i) the marketing authorization holder for the original orphan medicinal product consents to the authorization of the second medicinal product; (ii) the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities of the product; or (iii) it is established that the second product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The period of market exclusivity may, in addition, be reduced to six years if at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation because, for example, the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity. This 10-year period of market exclusivity can be extended to 12 years upon completion of an agreed paediatric investigation plan.

All of the aforementioned EU rules are generally applicable in the EEA.

Reform of the Regulatory Framework in the European Union

The European Commission introduced legislative proposals in April 2023 that, if implemented, will replace the current regulatory framework in the EU for all medicines (including those for rare diseases and for children). In April 2024, the European Parliament adopted its position on the legislative proposals, and in June 2025, the Council of the European Union adopted its position. A common position on the text has been agreed upon on December 11, 2025, in the context of subsequent inter-institutional trilogue negotiations. The proposed revisions remain to be adopted, and are not expected to become applicable before 2028.

Brexit and the Regulatory Framework in the United Kingdom

Following the end of the Brexit transition period on January 1, 2021 and the implementation of the Windsor Framework on January 1, 2025, the UK is not generally subject to EU laws in respect of medicines. The EU laws that have been transposed into UK law through secondary legislation remain applicable in the UK, however, new legislation such as the EU Clinical Trials Regulation is not applicable in the UK.

As of January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or MHRA, is the UK's standalone medicines and medical devices regulator. On January 1, 2025 a new arrangement called the "Windsor Framework" came into effect and made the MHRA the only authority approving medicines for the UK market, including Northern Ireland. The Windsor Framework removes EU licensing processes in relation to Northern Ireland for medicines placed on the UK market, disapplies EU Falsified Medicines Directive (FMD) safety-feature requirements there, and introduces a UK-wide licensing process; medicines placed on the UK market must be labeled 'UK Only' (and cannot be placed on the EU/EEA market in that packaging).

However, although a separate authorization is now required to market medicinal products in the UK, under an international recognition procedure which was put in place by the MHRA on January 1, 2024, the MHRA may take into account prior approvals from specified reference regulators when considering a marketing authorization application, but the MHRA retains ultimate authority to accept or reject the application. There is now no separate pre-marketing authorization orphan designation process in the UK. Instead, status is assessed as part of (and alongside) the evaluation of the corresponding marketing authorization application. The criteria are essentially the same, but have been tailored for the UK market, i.e., the prevalence of the condition in the UK (rather than the EU) must not be more than five in 10,000. On grant of a marketing authorization with orphan status, the medicinal product may benefit from up to 10 years of market exclusivity from similar products in the approved orphan indication (which may be reduced to 6 years in certain circumstances), starting from the date of first approval of the product in the UK; where the results of studies completed in accordance with a compliant paediatric investigation plan are reflected in the Summary of Product Characteristics, an additional 2 years of market exclusivity may be available.

Employees and Human Capital Resources

As of March 25, 2026, we had 29 full-time employees, of which two have Ph.Ds. None of our employees are represented by labor unions or covered by collective bargaining agreements, and we believe our relationship with our employees is good. We also utilize the services of several independent consultants to support our research and development and general and administrative operations.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing, and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

Corporate Information

We were incorporated in the State of Nevada in May 2013 under the name "Marika Inc." and began operating the business of Pieris Pharmaceuticals GmbH (formerly Pieris AG, a German company which was founded in 2001), through a reverse acquisition on December 17, 2014.

On December 13, 2024, we completed a reverse merger transaction (the "Merger") with Legacy Palvella, and, upon completion of the Merger, we changed our name to "Palvella Therapeutics, Inc." Shares of our common stock commenced trading on the Nasdaq Capital Market under the ticker symbol "PVLA" as of market open on December 16, 2024.

Legacy Palvella was formed under the laws of the State of Delaware on September 11, 2015 as Palvella Therapeutics LLC, a limited liability company. On May 30, 2018, Legacy Palvella converted into a Delaware corporation and changed its name to Palvella Therapeutics, Inc. Since Legacy Palvella's inception, it has devoted substantially all of its time to identifying, researching and conducting preclinical and clinical activities for its product candidates, acquiring and developing its platform technology, organizing and staffing its company, business planning, raising capital and establishing its intellectual property portfolio.

Our principal executive office is located at 353 W. Lancaster Avenue, Suite 200, Wayne, Pennsylvania 19087, and our telephone number is (484) 253-1461.

Information Available on the Internet

We use our website (www.palvellatx.com), LinkedIn (https://www.linkedin.com/company/palvella-therapeutics/) and Twitter's "X" (https://x.com/PalvellaTX) as distribution channels for Company information. The information contained on, or that can be accessed through our website, LinkedIn or Twitter, which may be deemed material, is not part of this Annual Report on Form 10-K and such internet addresses are included in this document solely as inactive textual references. We make available free of charge through our website, our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and exhibits and amendments to these reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after we electronically file or furnish such materials to the SEC. You may obtain any of the documents filed by us with the SEC at no cost from the SEC's website at *http://www.sec.gov*.

Item 1A. Risk Factors.

Investing in our securities involves risks. Before you make a decision to buy our securities, in addition to the risks and uncertainties discussed above under "Cautionary Note Regarding Forward-Looking Statements," you should carefully consider the specific risks set forth herein. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. If any of these risks actually occur, it may materially harm our business, financial condition, liquidity and results of operations. As a result, the market price of our securities could decline, and you could lose all or part of your investment.

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We have historically incurred significant operating losses and anticipate that we will continue to incur significant operating losses for at least the next several years. We may never achieve or maintain profitability.

We have historically incurred significant operating losses and have never generated any revenue. Our operating loss for the years ended December 31, 2025 and 2024 was $38.6 million and $14.1 million, respectively. As of December 31, 2025, we had an accumulated deficit of $135.5 million. We expect to continue to incur significant operating losses for at least the next several years, and we may never achieve or sustain profitability. We have historically devoted substantially all of our efforts to identifying, researching and conducting preclinical and clinical activities for our product candidates, developing our QTORIN platform, organizing and staffing our company, business planning, raising capital and establishing our intellectual property portfolio. We have never obtained regulatory approval for, or commercialized, any products. We anticipate that our expenses will increase substantially for the foreseeable future if, and as, we:

- seek regulatory approval for QTORIN rapamycin for the treatment of microcystic LM, cutaneous VMs, and/or clinically significant angiokeratomas, QTORIN pitavastatin for the treatment DSAP and any other product candidates that successfully complete clinical trials;
- continue clinical development of our product candidates, including our planned Phase 3 pivotal study for QTORIN rapamycin in patients with cutaneous VMs, planned Phase 2 study for QTORIN rapamycin for clinically significant angiokeratomas, and planned Phase 2 study for QTORIN pitavastatin for DSAP which we plan to begin in the second half of 2026;
- continue IND-enabling development for QTORIN pitavastatin for the treatment of DSAP;
- establish a specialized commercial organization in the United States to commercialize any product candidate for which we obtain marketing approval;
- initiate and continue relationships with suppliers and manufacturers and have clinical and commercial quantities of our product candidates manufactured at acceptable cost and quality levels and in compliance with the FDA and other regulatory requirements;
- initiate additional clinical trials and preclinical studies for our other product candidates;
- seek to identify and develop or in-license additional product candidates;
- incur additional costs associated with operating as a public company, which will require us to add operational, financial, and management information systems and personnel, including personnel to support product development, any future commercialization efforts, and our transition to a public company;
- expand our infrastructure and facilities to accommodate our growing employee base, including adding equipment and physical infrastructure to support our research and development; and
- maintain, expand and protect our intellectual property portfolio.

We may never succeed in any or all of these activities and, even if we do, we may never generate revenue.

We have never generated revenue from product sales and may never achieve or maintain profitability.

To become and remain profitable, we must succeed in developing and eventually commercializing products that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials, obtaining regulatory approval, procuring commercial-scale manufacturing, and marketing and selling any products for which we obtain regulatory approval. We have never obtained regulatory approval, procured commercial-scale manufacturing or marketed any product, and we may never succeed in these activities. Even if we do obtain regulatory approval for and begin commercializing QTORIN rapamycin for microcystic LM, cutaneous VMs, clinically significant angiokeratomas or any other indication, QTORIN pitavastatin for DSAP or any other indication or any future product candidates, our ability to become profitable will be dependent upon, in part and among other things, the size of the markets in the territories for which we gain regulatory approval, the number of competitors in such markets, the accepted price for any such product candidate and the degree of market acceptance we achieve.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of our investment.

Our limited operating history may make it difficult to evaluate our business to date and our future viability.

We are a late clinical-stage biopharmaceutical company with a limited operating history. We have no products approved for commercial sale and have not generated any revenue from product sales. As an organization, we have limited experience successfully completing pivotal clinical trials, and have not yet demonstrated an ability to prepare and submit an NDA, obtain marketing approval, manufacture a commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, we have little or no meaningful operations upon which to evaluate our business, and predictions about our future success or viability may not be as accurate as it could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

Our ability to generate product revenue and become profitable depends upon our ability to successfully complete the development of, and obtain the necessary regulatory approvals for, our product candidates. Even if we receive regulatory approval for any product candidate, we do not know when or if such product candidate will generate product revenue. We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives, including with respect to our technology and product candidates. In the future, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may also need to secure strategic collaborations with partners in order to commercialize any approved product candidates outside of the U.S. market. We may not be successful in making such a transition or in securing such strategic collaborations.

We will likely require substantial additional funding to finance our operations, which may cause dilution to our stockholders. Failure to obtain this necessary funding when needed on acceptable terms, or at all, could force it to delay, limit, reduce or terminate our product development, commercialization efforts or other operations.

As of December 31, 2025, we had cash and cash equivalents of $58.0 million, which, together with the $215.8 million in net proceeds from our recently completed public offering of common stock, and based upon our current operating plan, we believe that our cash and cash equivalents will be sufficient to fund our planned operations for at least the next twelve months from the date of this Annual Report on Form 10-K. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. To finance our operations beyond that point we may need to raise additional capital, which cannot be assured. Our operating plans may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other sources, such as strategic collaborations. To the extent that we raise additional capital by issuing equity securities, our existing stockholders may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that could harm the rights of a common stockholder. Any agreements for future debt or preferred equity financings, if available, may

involve covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. We may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future capital requirements depend on many factors, including, but not limited to:

- the scope, progress, timing, results and costs of researching and developing our lead product candidates or any future product candidates, and conducting preclinical studies and clinical trials;
- the number and scope of clinical programs we decide to pursue;
- the cost, timing and outcome of seeking regulatory approvals of our product candidates;
- the cost of manufacturing our product candidates and any products we commercialize, including costs associated with building out our supply chain;
- the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such agreements that we may enter into;
- the timing, receipt and volume of sales of future approved products, if any;
- the timing and amount of milestone or royalty payments due to Ligand, under Ligand Agreements (as defined below), or under similar arrangements with any future collaboration or licensing partners;
- the expenses needed to attract and retain skilled personnel;
- our need and ability to implement additional internal systems and infrastructure, including financial and reporting systems; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property portfolio.

Adequate additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis or on attractive terms, we may be required to reduce our workforce, delay, limit, reduce or terminate our research and development activities, preclinical studies, clinical trials or other development activities and future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise develop and market ourselves. In addition, attempting to secure additional financing may divert the time and attention of our management from daily activities and distract from our research and development efforts.

Our development funding agreement with Ligand obligates us to make certain milestone payments, some of which will be triggered prior to our commercialization of any of our product candidates.

Certain of the milestone payments payable by us in connection with the Amended Ligand Agreement are due upon events that will occur prior to our planned commercialization of our lead product candidate, QTORIN rapamycin. Accordingly, we may be required to make payments in an aggregate amount of up to $5.0 million prior to the time at which we are able to generate revenue, if any, from sales of QTORIN rapamycin for any indication, if approved. There can be no assurance that we will have the funds necessary to make such payments, or be able to raise such funds when needed, on terms acceptable to us, or at all. In order to make the required payments when due, we may be required to divert our capital resources by delaying, limiting, reducing or terminating our product development or future commercialization efforts, or we may have to grant rights to develop and market product candidates that we would

otherwise develop and market ourselves. If we are required to raise funds but are unable to do so, or if we are unable to otherwise maintain sufficient liquidity to make our payment obligations if and when they become due, we may be in material breach of the Amended Ligand Agreement, and Ligand may seek legal action or remedies against us (including by seeking to terminate the Amended Ligand Agreement), which would harm our business, financial condition, results of operations and prospects. If we are able to raise funds, we may not be able to do so on terms that are favorable to us, and our existing stockholders may experience substantial dilution, we may agree to certain covenants limiting or restricting our ability to take specific actions, or we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates.

Our ability to utilize our net operating loss ("NOL") carryforwards and certain other tax attributes may be limited.

Our federal NOL carryforwards may be unavailable to offset future taxable income because of restrictions under U.S. tax law. Our federal NOL carryforwards generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but for taxable years beginning after December 31, 2020, the deductibility of such federal NOL carryforwards is limited to 80% of our current year taxable income. It is uncertain if and to what extent limitations under state law may differ.

As of December 31, 2025, we had net operating loss carryforwards for U.S. federal income tax purposes of $129.4 million and net operating loss carryforwards for state income tax purposes of $113.3 million. Federal tax loss carryforwards that were created prior to December 31, 2017 expire through 2037 and federal losses created after that date do not expire. State loss carryforwards expire starting in 2035.

In addition, as noted above, under Sections 382 and 383 of the Code, if a corporation undergoes an "ownership change" (generally defined as a cumulative change in the corporation's ownership by "5-percent shareholders" that exceeds 50 percentage points over a rolling three-year period), the corporation's ability to use our pre-change NOL carryforwards and certain other pre-change tax attributes to offset our post-change taxable income may be limited. Similar rules may apply under state tax laws. We believe that as a result of the Merger, our ability to utilize NOLs acquired in this transaction is expected to be severely limited by Section 382 of the Code. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. There is also a risk that due to regulatory changes, such as suspensions on the use of NOL carryforwards, or other unforeseen reasons, our existing NOL carryforwards could expire or otherwise be unavailable to offset future income tax liabilities. Because our ability to utilize our NOL carryforwards and certain other tax attributes could be limited as described above, we may not be able to utilize a material portion of our NOL carryforwards and certain other tax attributes, which could have a material adverse effect on our cash flows and results of operations.

As of December 31, 2025, we had German corporate income tax and trade tax net operating loss carryforwards of approximately $219.4 million and $215.1 million, respectively. Under current German laws, tax loss carryforwards may only be used to offset any relevant later assessment period (calendar year) of $1.2 million plus 60% of the exceeding taxable income and trade profit of such period and do not expire. In addition, certain transactions, including transfers of shares or interest in the loss holding entity, may result in the partial or total forfeiture of tax losses existing at that date. Partial or total forfeiture of tax losses may further occur in corporate reorganizations of the loss holding entity.

Risks Related to the Discovery, Development, Regulatory Approval and Commercialization of Our Product Candidates

Clinical drug development is a lengthy, complex and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development of our product candidates.

Our lead product candidate, QTORIN rapamycin, is in clinical development and the risk of failure is high. It is impossible to predict when or if any of our product candidates will prove effective and safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. Clinical trial failure may result from a multitude of factors including, but not limited to, flaws in trial design, carryover effect, dose selection, placebo

effect, patient enrollment criteria and failure to demonstrate favorable safety or efficacy, and failure in clinical trials can occur at any stage. For example, our Phase 2b clinical trial of QTORIN rapamycin in patients with Gorlin Syndrome and Phase 3 clinical trials of QTORIN rapamycin in patients with pachyonychia congenita failed to meet their respective primary endpoints.

In the first quarter of 2026, we reported topline data from our Phase 3 clinical trial of QTORIN rapamycin for the treatment of microcystic LM, and are planning for a potential NDA submission to the FDA in the second half of 2026. We intend to commence a Phase 3 pivotal trial of QTORIN rapamycin for the treatment of cutaneous VMs in the second half of 2026 and a Phase 2 trial of QTORIN rapamycin for the treatment of clinically significant angiokeratomas in the second quarter of 2026. Additionally, we received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study to evaluate QTORIN pitavastatin in subjects with DSAP, with trial initiation anticipated in the second half of 2026. Our other programs under evaluation for the treatment of other serious, rare skin diseases, vascular malformations and other genetic diseases are in early-stage preclinical development, or in the case of QTORIN pitavastatin, are in IND-enabling development.

We may experience numerous unforeseen events that could delay or prevent our ability to receive marketing approval for our product candidates, including:

- regulators or IRBs may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site, or may halt or suspend an ongoing trial;
- clinical trials of our product candidates may fail to show safety, efficacy or an acceptable benefit-risk profile, produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional nonclinical studies or clinical trials or abandon drug development programs;
- the design of any of our clinical trials may be flawed, and those flaws may not become apparent until such clinical trial is well advanced or completed;
- regulators may not agree with our selection of novel endpoints or other key clinical trial design features, such as choice of control, used in our clinical evaluation of our product candidates; for example, the FDA has commented that a placebo-controlled trial or additional trials assessing different clinical endpoints may be required to assess the efficacy of QTORIN rapamycin for the treatment of microcystic LMs;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- the number of subjects required for clinical trials of our product candidate may be larger than anticipated, enrollment in the clinical trials for our product candidates may be slower than we anticipate, we may have difficulty identifying and enrolling suitable participants given the small patient populations of the diseases we are targeting, or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective contract research organizations (“CROs”) the terms of which can be subject to extensive negotiation and may vary significantly among different trial sites and CROs; the cost of clinical trials of our product candidates may be greater than we anticipate, particularly if the FDA or other equivalent foreign regulatory authorities require post-marketing studies and/or a patient registry; and
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that are currently contemplated, if we are unable to successfully complete clinical trials of our product candidates or other testing in a timely manner, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may incur unplanned costs, be delayed in seeking and obtaining regulatory approval, if we receive regulatory approval with labeling that includes warnings or precautions or limitations of use, or be subject to additional post-marketing testing requirements. If we experience any delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates may not be successful. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development process and jeopardize our ability to receive regulatory approval and commence product sales and generate revenues. Any of these occurrences could materially adversely affect our business, financial condition, results of operations and prospects.

Our future success is substantially dependent on the successful clinical development, regulatory approval and commercialization of QTORIN rapamycin, which is in later stages of development than our other product candidates.

We currently have no products that are approved for commercial sale. We are developing our lead product candidate, QTORIN rapamycin, for the treatment of three serious, rare skin diseases. We completed our Phase 3 clinical trial evaluating QTORIN rapamycin in patients with microcystic LM and are planning for a potential NDA submission in the second half of 2026. We intend to commence a Phase 3 pivotal trial evaluating QTORIN rapamycin in patients with cutaneous VMs in the second half of 2026. We are working on preclinical development of QTORIN rapamycin for the treatment of clinically significant angiokeratomas and plan to initiate a clinical study in the second quarter of 2026. We expect that a substantial portion of our efforts and expenditures over the next few years will be devoted to the continued clinical evaluation of QTORIN rapamycin and the commercialization of this product candidate for the treatment of microcystic LM, if approved. Accordingly, the success of our business, including our ability to finance our company and generate any revenue in the future, will primarily depend on the successful development, regulatory approval and commercialization of QTORIN rapamycin.

The clinical and commercial success of QTORIN rapamycin will depend on many factors, including the following:

- the ability to raise any additional required capital on acceptable terms, or at all;
- timely completion of our preclinical studies and clinical trials, which may be significantly slower or cost more than we currently anticipate and may depend substantially upon the performance of certain third-party contractors;
- the ability to demonstrate the safety, efficacy and acceptable benefit-risk profile of QTORIN rapamycin to the satisfaction of the FDA and equivalent foreign regulatory authorities;
- delays in developing and testing, or inability to develop and test, any clinical outcome assessments to the extent necessary for the FDA and equivalent foreign regulatory authorities to agree to their use as endpoints utilized in a clinical trial to support labeling claims;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with QTORIN rapamycin, if any, or experienced by competitors who are developing topical rapamycin (also known as sirolimus) products or who are targeting the same indications in the rare skin diseases space;
- the timely receipt of necessary marketing approvals from the FDA and equivalent foreign regulatory authorities and, if granted, completion of any required post-marketing studies or trials and available funding to perform any such studies or trials;
- the ability of any CMO, upon which we rely to manufacture clinical and commercial supplies of our product candidates or any future product candidates to remain in good standing with relevant regulatory authorities

and to develop, validate and maintain commercially viable manufacturing processes that are compliant with cGMPs;

- our ability to successfully develop a targeted rare disease commercial strategy and thereafter establish sales, marketing and distribution capabilities to launch and commercialize QTORIN rapamycin in the United States and internationally, if approved for marketing, reimbursement, sale and distribution in such countries and territories;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of QTORIN rapamycin, if approved;
- obtainment and maintenance of coverage, adequate pricing and adequate reimbursement from third-party payors, including government payors;
- our ability to retain subjects who have enrolled in a clinical study but may be prone to withdraw due to the rigors of the clinical trial, lack of efficacy, side effects, personal issues or loss of interest;
- the size of the potential markets for QTORIN rapamycin, if approved; and
- our ability to establish and enforce intellectual property rights to QTORIN rapamycin, if any.

Even if we complete clinical testing and receive approval from the FDA or applicable equivalent foreign regulatory authorities for QTORIN rapamycin, the FDA or equivalent foreign regulatory authorities may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post- market clinical trials, or impose restrictions on the product's distribution in the form of a REMS. The FDA or equivalent foreign regulatory authority may also approve QTORIN rapamycin, for a more limited indication or a narrower patient population than we originally requested. In addition, the FDA or equivalent foreign regulatory authorities may not approve QTORIN rapamycin with the labeling that we believe is necessary or desirable, or may approve it with labeling that includes warnings or precautions or limitations of use that may not be desirable for the successful commercialization of QTORIN rapamycin.

The factors outlined above, many of which are beyond our control, could cause us to experience significant delays or affect our ability to obtain regulatory approvals or commercialize QTORIN rapamycin. If we are unable to obtain regulatory approval and successfully commercialize QTORIN rapamycin, our financial position will be materially adversely affected, and we may not be able to generate sufficient revenue to continue our business.

We plan to submit an NDA to the FDA for the marketing approval of QTORIN rapamycin for microcystic LMs, based largely on data from our completed Phase 3 SELVA study; however, there can be no assurance that the data from our clinical trials will ultimately support filing of an NDA by the FDA or that the FDA will grant marketing approval of QTORIN rapamycin for microcystic LMs without additional clinical or nonclinical studies, or at all.

We plan to submit an NDA to the FDA for the marketing approval of QTORIN rapamycin for microcystic LMs, based largely on data from our completed Phase 3 SELVA study. However, the FDA may not agree microcystic LM is an appropriate setting for a baseline-controlled Phase 3 study and has commented that a placebo-controlled trial or additional trials assessing different clinical endpoints may be required to assess the efficacy of QTORIN rapamycin for the treatment of microcystic LM. Further, the FDA may refuse to file our planned NDA for substantive review or, if the application is filed by the FDA, the FDA may ultimately conclude after a more in-depth review of our data that our application is insufficient to support regulatory approval. If the FDA does not approve our planned NDA for QTORIN rapamycin for microcystic LMs, it may require that we conduct additional clinical or nonclinical studies or provide additional manufacturing information before it will reconsider our application for filing and subsequent review. Depending on the extent of these or any other studies or information required by the FDA to support approval, the FDA's filing decision or subsequent approval of an NDA may be significantly delayed or we may be unable to obtain approval of an NDA because such studies or information may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA. If any of these outcomes occur, we may be forced to abandon our planned NDA, which would materially adversely affect our business and could potentially cause us to cease operations. We face similar regulatory risks in a foreign jurisdiction.

We may be unable to obtain regulatory approval for our product candidates under applicable regulatory requirements. The denial or delay of any such approval would adversely impact our potential to generate revenue, our business and our results of operations.

The research, testing, manufacturing, labeling, sale, marketing and distribution of biopharmaceutical products are subject to extensive regulation by the FDA and comparable regulatory authorities in the United States and other countries, and such regulations differ from country to country. We are not permitted to market our product candidates in any jurisdiction until we receive the requisite marketing approval from the applicable regulatory authorities of such jurisdictions. To gain approval to market our product candidates, we must provide the FDA and foreign regulatory authorities with preclinical, manufacturing and clinical data that adequately demonstrate the safety and efficacy of the product for the intended indication applied for in the applicable regulatory filing. The approval process is typically lengthy and expensive, and approval is never certain.

We reported topline data from our Phase 3 trial of QTORIN rapamycin for the treatment of microcystic LM in the first quarter of 2026 and are planning for a potential NDA submission in the second half of 2026. We reported topline data from our Phase 2 trial of QTORIN rapamycin for the treatment of cutaneous VMs in the fourth quarter of 2025 and intend to commence a Phase 3 pivotal trial evaluating QTORIN rapamycin in patients with cutaneous VMs in the second half of 2026. Data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in clinical trials have nonetheless failed to obtain marketing approval of their drugs.

The FDA or any foreign regulatory authorities can delay, limit or deny approval of QTORIN rapamycin for the treatment of microcystic LM, cutaneous VMs, or clinically significant angiokeratomas, QTORIN pitavastatin for the treatment of DSAP or any future product candidates for many additional reasons, including:

- the FDA or other equivalent foreign regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or equivalent foreign regulatory authorities that any of our product candidates are safe and effective for the requested indication(s);
- the results of our clinical trials may not meet the level of statistical significance or clinical meaningfulness or establish an acceptable benefit-risk profile required by the FDA or other equivalent foreign regulatory authorities for marketing approval;
- the FDA or other equivalent foreign regulatory authorities may not accept data generated from our clinical trial sites;
- the FDA or other equivalent foreign regulatory authorities may find the CMC data insufficient to support the quality of our product candidates;
- the FDA or other equivalent foreign regulatory authorities may identify deficiencies in the manufacturing processes or facilities of our CMOs;
- the FDA or other equivalent foreign regulatory authorities may disagree with our assessment that the delivery device component associated with our QTORIN platform is a Class I device exempt from premarket notification requirements as well as Quality System Regulation;
- the FDA or equivalent foreign regulatory authorities may not approve the formulation, dosing, labeling or specifications; or
- the potential for approval policies or regulations of the FDA or other equivalent foreign regulatory authorities to significantly change in a manner rendering our data insufficient for approval or invalidated.

Any of these factors, many of which are beyond our control, may result in our failing to obtain regulatory approval to market any of our product candidates, which could materially adversely affect our business, financial condition, results of operations and prospects.

Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials.

Results from preclinical studies or early clinical trials are not necessarily predictive of future clinical trial results and are not necessarily indicative of final results. Our product candidates may fail to show the desired characteristics in clinical development sufficient to obtain regulatory approval, despite positive results in preclinical studies or having successfully advanced through earlier clinical trials. The results of past and future nonclinical studies and early clinical trials of QTORIN rapamycin, QTORIN pitavastatin or any future product candidates may not be predictive of the results of later-stage clinical trials. Differences in trial design between early-stage clinical trials and later-stage clinical trials may make it difficult to extrapolate the results of earlier clinical trials to later clinical trials. For example, in our Phase 2 study of QTORIN rapamycin in patients with microcystic LM, efficacy was evaluated as secondary endpoints without multiplicity adjustment or statistical analyses, but in our Phase 3 study in microcystic LM, a single efficacy hypothesis was tested as the primary endpoint. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Moreover, even though we are using and plan to use the same formulation of QTORIN rapamycin to support multiple investigational development programs in multiple product candidates, we cannot be certain that any success we have or may in the future have with respect to the development of QTORIN rapamycin for the treatment of microcystic LMs, cutaneous VMs, or clinically significant angiokeratomas or QTORIN pitavastatin for the treatment of DSAP will lead to the successful development of QTORIN rapamycin or QTORIN pitavastatin for additional indications or additional product candidates.

In addition, the design of a pivotal clinical trial can determine whether our results will support approval of a product candidate and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced or completed. We have limited experience in designing and conducting pivotal clinical trials and we may be unable to successfully design and execute pivotal clinical trials of our product candidates to support regulatory approval.

We reported topline data from our Phase 2 clinical trial of QTORIN rapamycin for the treatment of cutaneous venous malformations in the fourth quarter of 2025. However, we may be unable to duplicate these results in other clinical trials we may conduct. Additionally, even if the FDA or other regulatory authorities accept the novel clinical endpoints we establish in connection with our Phase 2 trial in cutaneous venous malformations, there are no assurances that the FDA or other regulatory authorities will find the efficacy endpoints we propose in our future pivotal clinical trials to be sufficiently developed and tested and clinically meaningful, or that our product candidates will achieve the pre-specified endpoints in future pivotal clinical trials to a degree of statistical significance. For example, the FDA has commented that a placebo-controlled trial or additional trials assessing different clinical endpoints may be required to assess the efficacy of QTORIN rapamycin for the treatment of microcystic LM.

The rare skin diseases and vascular malformations we are currently targeting have no FDA-approved therapies, which subjects the design and execution of our clinical development programs to complexities and known as well as unknown risks, including those related to novel and/or subjective clinical endpoints and varying patient population characteristics.

There are currently no FDA-approved therapies indicated for the treatment of microcystic LMs, cutaneous VMs, clinically significant angiokeratomas or DSAP. We have concentrated our current research and development efforts on developing effective therapies for these indications, in addition to other rare skin diseases, vascular malformations and rare genetic conditions in other disease areas, and our future success depends on the success of this approach. The clinical trial requirements of the FDA and other comparable regulatory agencies and the criteria these regulators use to determine the safety and efficacy of any product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential product. Given the nature of the skin diseases we are targeting, the design and execution of our clinical development program is subject to both known and unknown risks.

As with QTORIN rapamycin for the treatment microcystic LMs or cutaneous VMs or clinically significant angiokeratomas, QTORIN pitavastatin for the treatment of DSAP, and any other indications or future product candidates that may require us to use new or novel endpoints or methodologies, the FDA or other regulatory authorities may not consider the endpoints of our clinical trials or the magnitude of treatment effect observed in our clinical trials to provide clinically meaningful results. Even if applicable regulatory authorities do not object to our proposed endpoints in an earlier-stage clinical trial, such regulatory authorities may require evaluation of additional or different clinical endpoints in later-stage clinical trials or may not accept the clinical endpoints evaluated in later-stage clinical trials. For example, while the primary endpoint in the Phase 3 clinical trial of QTORIN rapamycin for the treatment of microcystic LM employed a dynamic assessment that used a comparative rating scale, which was also assessed as

one of several efficacy endpoints in the Phase 2 study in microcystic LM, the FDA has recommended that primary efficacy in the treatment of microcystic LM to be evaluated on a static multicomponent assessment scale but also recommended that we provide a rationale for selecting the comparative rating scale should we proceed with a comparative rating scale. If the FDA does not agree with our primary endpoint, the FDA may instead consider the Phase 3 clinical trial's key secondary endpoint, which is a static multicomponent assessment scale, as pivotal to assessing efficacy, if alpha-protected. Alternatively, the FDA may consider the study to not be adequate and well-controlled and could request additional clinical trials to assess a static multicomponent assessment scale as the primary endpoint. As a result, the design and conduct of our ongoing and future clinical trials of our product candidates may take longer, be more costly or be less definitive.

Further, different results may be achieved depending upon the characteristics of the population enrolled in our studies and which analysis population is used to analyze results. As a result, there is no guarantee that our clinical trials will produce statistically significant results with respect to subject-reported outcomes, and there can be no guarantee that the characteristics of the population enrolled in our clinical trials does not adversely impact the results reported for such trials.

Any delays in, or the denial of, approval of any of our product candidates resulting from our inability to establish effective trial designs for serious, rare skin diseases or vascular malformations could materially adversely affect our business, financial condition, results of operations and prospects.

Our lead product candidates are based on our QTORIN platform, which is highly dependent on the successful development of this novel and unproven technology.

Our proprietary QTORIN platform is novel and was developed over several years of research to overcome inherent challenges, including chemical stability, skin penetration and skin distribution, with topical delivery of mTOR inhibitors, such as rapamycin and other therapeutic agents. QTORIN is an anhydrous gel comprising excipients intentionally selected in a ratio designed to achieve drug stability at room temperature and enable cutaneous distribution of therapeutics levels of cargoes into the target cells in the basal layer of the epidermis and to the dermis. Our product candidate for the treatment of microcystic LMs, cutaneous VMs, and clinically significant angiokeratomas leverages QTORIN as a mechanism of delivery of a 3.9% concentration of rapamycin to treat the applicable disease. Our product candidate for the treatment of DSAP leverages QTORIN as a mechanism of delivery of pitavastatin. The QTORIN platform has generated two program candidates, QTORIN rapamycin for three indications to date and QTORIN pitavastatin for one indication to date, and clinical evidence to support these candidates is preliminary and limited at this time by their respective stages of development.

QTORIN is the platform for our current clinical-stage product candidates and for other research-stage product candidates in our pipeline, and accordingly, our future success depends in significant part on the successful development of this novel technology. Negative results in the development of QTORIN rapamycin for the treatment of microcystic LMs, cutaneous VMs, or clinically significant angiokeratomas, or of QTORIN pitavastatin for the treatment of DSAP, may affect our ability to become the standard of care. In addition, negative results may impact our ability to obtain regulatory approval for other product candidates which we expect to develop based on our QTORIN platform, either at all or within anticipated timeframes because, although we may be targeting different indications, the underlying technology platform is the same for each product candidate and there may be commonalities in the manufacturing and development processes. Accordingly, a failure in any one QTORIN-based program may decrease confidence in our technology and affect our ability to conduct clinical programs for, and ultimately obtain regulatory approval for, other QTORIN-based product candidates.

We have not yet succeeded and may not succeed in completing clinical development of or obtaining regulatory approval for any of our product candidates using our QTORIN platform. As a result, it is more difficult for us to predict whether the application of our QTORIN platform, or any similar or competitive platforms, will result in the development and marketing approval of any products. Any developmental problems we experience in the future related to our QTORIN platform or any of our research programs may cause significant delays or unanticipated costs or may prevent the development of a commercially viable product. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all, which could materially adversely affect our business, financial condition, results of operations and prospects.

We may find it difficult to enroll subjects in our clinical trials, which could delay or prevent us from proceeding with clinical trials of our product candidates.

Identifying and qualifying a sufficient number of eligible subjects to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on our ability to recruit subjects to participate, as well as the completion of required follow-up periods. Patients may be unwilling to participate in our clinical trials because of negative publicity from adverse events related to the biotechnology or pharmaceutical fields, competitive clinical trials for similar patient populations, the existence of current treatments or for other reasons outside of our control. The timeline for recruiting subjects, conducting studies and obtaining regulatory approval of our product candidates may be delayed, which could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

Importantly, the indications that we are currently targeting and may in the future target are serious, rare diseases and vascular malformations, which may limit the pool of subjects that may be enrolled in our ongoing or planned clinical trials. To the extent our clinical trials are limited to specific genotypes, the population of eligible trial participants is even further limited. Microcystic LMs affect an estimated greater than 30,000 diagnosed patients in the United States. Cutaneous VMs affect an estimated greater than 75,000 people in the United States. Clinically significant angiokeratomas and DSAP affect an estimated greater than 50,000 patients in the United States. Some of the other diseases we intend to target have similarly limited patient populations. We expect to rely in part on our relationships with patient advocacy groups to assist in identifying eligible patients, and any deterioration of those relationships could impede our ability to successfully enroll patients in our clinical trials. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible subjects to participate in these trials as required by the FDA or similar regulatory authorities outside of the United States.

Subject enrollment and trial completion are affected by numerous factors, including the:

- size and nature of the target population and the process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria for the trial;
- safety profile, to date, of the product candidate under study;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials for similar product candidates or targeting subjects meeting our trial eligibility criteria;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor subjects adequately during and after treatment.

If we have difficulty enrolling a sufficient number of subjects to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

Orphan Drug Designation has been granted for QTORIN rapamycin for the treatment of LM, but we may be unable to obtain such designation for other diseases or conditions or product candidates or to realize the benefits of Orphan Drug Designation, including potential marketing exclusivity, even if such designation is obtained.

Regulatory authorities in some jurisdictions, including the United States and other major markets, may designate drugs intended to treat conditions or diseases affecting relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the European Commission, following recommendation from the EMA's Committee for Orphan Medicinal Products, may grant orphan designation in respect of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating disease affecting not more than five in 10,000 persons in the European Union when the application is made. Additionally, orphan designation may be granted by the European Commission for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic disease when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product. In each case, the sponsor must show that no satisfactory method of diagnosis, prevention or treatment of the condition in question has been authorized in the European Union or, if such method exists, the product will be of significant benefit to those affected by that condition.

We have received Orphan Drug Designation for QTORIN rapamycin for the treatment of microcystic LM from the FDA and European Commission. If we request Orphan Drug Designation or the foreign equivalent for any of our other or future product candidates, there can be no assurances that the FDA or non-U.S. regulatory authorities will grant any of our product candidates such designation. Additionally, the designation of any of our product candidates as an orphan product does not mean that any regulatory agency will expedite regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant Orphan Drug Designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Generally, if a product candidate with an Orphan Drug Designation receives the first marketing approval for the disease or condition for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or foreign regulatory authorities (as applicable) from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our Orphan Drug Designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period unless FDA concludes that our drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. The applicable exclusivity period is 7 years in the United States and 10 years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition in the United States and the same drug may be approved for different conditions. Even after an orphan drug is approved, the FDA may subsequently approve another drug for the same disease if the FDA concludes that the latter drug is not the same drug or is clinically superior. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication as an authorized orphan product if the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior; if the holder of the marketing authorization for the original medicinal product consents to a second orphan medicinal product application; or if the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

Our inability to obtain Orphan Drug Designation or the foreign equivalent for our product candidates, or to realize the benefits of such designation, could have an adverse effect on our business, financial condition, results of operations and prospects.

We are targeting serious, rare skin diseases and vascular malformations, and the small patient populations associated with such diseases and malformations present additional risks with respect to clinical development, regulatory approvals and commercialization of product candidates.

Our approach of targeting rare skin diseases and vascular malformations presents risks related to the clinical development, regulatory approval and commercialization of our product candidates, including the following:

- we may have difficulty establishing safety and efficacy in these types of patient populations given there is less known about the natural history of the disease;
- we expect to face challenges with respect to patient enrollment in our clinical trials, as described above;
- small sample sizes in our clinical trials suggest that we face the risk of substantial variability in the results of our trials, and so the outcome of nonclinical testing and early clinical trials is less likely to be predictive of the success of later-stage clinical trials;
- following approval of our product candidates, if any, pricing and level of reimbursement may not be sufficient to offset costs of development, manufacturing, marketing, and commercialization; and
- market size is a significant variable in disease indications classified as rare. Our projections of both the number of people who have these diseases and malformations, as well as the subset of people with these diseases or malformations who have the potential to benefit from treatment with product candidates we may develop, are based on estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient advocacy groups or market research. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, and patients may not be amenable to treatment with our product candidates we may develop or may become increasingly difficult to identify or gain access to. Accordingly, even if we obtain significant market share for our product candidates, because the potential target populations are small, we may never achieve profitability without obtaining regulatory approval for additional indications.

Adverse developments with respect to any of the foregoing could result in significant changes in our business plan and have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our development and commercialization strategy for our product candidates depends, in part, on published scientific literature and the FDA's prior findings regarding the safety and efficacy of rapamycin, pitavastatin and other planned or future product candidates. If we are not able to pursue this strategy, we may be delayed in receiving regulatory authority approval.

The Hatch-Waxman Amendments added Section 505(b)(2) to the U.S. Federal Food, Drug, and Cosmetic Act, or FDCA. Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from investigations that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted. The FDA interprets Section 505(b)(2) of the FDCA, for purposes of approving an NDA, to permit the applicant to rely, in part, upon published literature and/or the FDA's previous findings of safety and efficacy for an approved product. The FDA also requires companies to perform additional clinical trials or measurements to support any deviation from the previously approved product and to justify that it is scientifically appropriate to rely on the applicable published literature or referenced product, referred to as bridging. The FDA may then approve the new product candidate for all or some of the indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant, if such approval is supported by study data. The labeling, however, may be required to include all or some of the limitations, contraindications, warnings or precautions or restrictions on use included in the reference product's labeling, including a boxed warning, or may require additional limitations, contraindications, warnings or precautions or restrictions on use.

We currently plan to pursue marketing approval for QTORIN rapamycin for several indications and QTORIN pitavastatin for one indication in the United States through Section 505(b)(2) NDAs and will be completing bridging analyses comparing our QTORIN programs to the approved oral rapamycin product, a previously approved organ rejection prophylactic, prior to NDA submission. If the FDA disagrees with our conclusions regarding the appropriateness of our reliance on the FDA's prior findings of safety and efficacy for the approved oral rapamycin product or on published literature, or if we are not otherwise able to bridge to the listed drug or published literature to demonstrate that our reliance is scientifically appropriate, we could be required to conduct additional nonclinical toxicology, clinical safety or efficacy trials, or other studies to support our NDA, which could lead to unanticipated costs and delays or to the termination of our development programs. For example, while we plan to bridge QTORIN rapamycin and the approved oral rapamycin product based on cross-study comparison between pharmacokinetic data from the prescribing information for the approved product, the FDA recommends that bridging to support an NDA for the treatment of microcystic LM be done in a relative bioavailability study comparing the pharmacokinetics of a topical product applied under maximal use conditions and the approved oral drug. The planned cross-study analysis allows for comparison of systemic pharmacokinetic parameters, key criteria for assessing the applicability of safety findings from the listed drug, which are a result of systemic exposure from the oral formulation. If the FDA does not agree with our pharmacokinetic approach, we may need to conduct a relative bioavailability study, which compares direct assessment of pharmacokinetics of both products administered under similar conditions. For example, FDA may request different specific criteria for comparisons that cannot be evaluated based on limitations in the pharmacokinetic data available in the prescribing information of the approved drug. If we are unable to obtain approval for our product candidates through the Section 505(b)(2) NDA process, we may be required to pursue the more expensive and time consuming Section 505(b)(1) approval process, which consists of full reports of investigations of safety and effectiveness conducted by or for the applicant. Even if we are allowed to pursue the Section 505(b)(2) regulatory pathway for FDA approval, we cannot assure you that our product candidates will receive the requisite approvals for commercialization.

The validity, scope and enforceability of any patents that we may list in the Orange Book that cover QTORIN rapamycin or QTORIN pitavastatin, if approved by the FDA for any indication, can be challenged by competitors.

If QTORIN rapamycin or QTORIN pitavastatin is approved by the FDA for any indication, one or more third parties may challenge the patents covering QTORIN rapamycin or QTORIN pitavastatin with respect to such indication, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or could result in a finding of non-infringement. For example, if a third party files an ANDA, for a generic drug bioequivalent to our QTORIN rapamycin or QTORIN pitavastatin, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify that either: (1) there is no patent information listed in the FDA's Orange Book with respect to our NDA for the applicable approved drug candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of the third party's generic drug. Alternatively, a third party that files an ANDA for a generic drug bioequivalent to QTORIN rapamycin or QTORIN pitavastatin may elect to submit a "section viii" statement stating that our proposed label does not contain any language regarding the patented method of use or carves out the patented method of use rather than certify to a listed method of use patent. This section viii statement does not require notice to the patent holder or NDA owner. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved drug candidate, or that such patents are invalid, is called a Paragraph IV certification. If the third party submits a Paragraph IV certification to the FDA, a notice of the Paragraph IV certification must also be sent to us once the third party's ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party's ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party's ANDA will not be subject to the 30-month stay of FDA approval. Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management's attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with our product candidates.

Other companies could receive FDA approval for a topical rapamycin or pitavastatin product before we receive FDA approval for our product candidates for microcystic LMs, cutaneous VMs, clinically significant angiokeratomas or DSAP and thus could be granted regulatory exclusivity that could significantly delay our ability to receive approval for and commercialize our product candidates and therefore dramatically reduce our market potential.

Other companies may submit a Section 505(b)(2) NDA and receive approval for a topical rapamycin or pitavastatin product prior to the approval of any future NDAs we submit for these candidates or may pursue in the future. The first approved Section 505(b)(2) product for a particular condition of use or change to a marketed product, such as a new formulation for a previously approved product, may be granted three-year exclusivity if one or more clinical studies, other than bioavailability or bioequivalence studies, was essential to the approval of the application and was conducted/sponsored by the applicant. The grant of three-year exclusivity can delay the FDA's approval of other Section 505(b)(2) applications for the same condition of use or change to the drug product, such as the first approval of a topical formulation of rapamycin, that was granted exclusivity, regardless of the date of submission of each NDA.

We believe that other companies are developing topical rapamycin products. In order to obtain regulatory approval with a Section 505(b)(2) NDA, other companies would have to sponsor or conduct new clinical investigations (other than bioavailability studies) that are essential to approval of the application, as well as conduct the required bridging studies. For example, if the FDA approves another company's Section 505(b)(2) NDA for a topical rapamycin product, even for another indication, and grants the other company three-year exclusivity before we receive approval for QTORIN rapamycin for the treatment of microcystic LM, the FDA may be precluded from approving any Section 505(b)(2) NDA we may submit with respect to QTORIN rapamycin until after that three-year exclusivity period has expired unless we pursue the more expensive and time consuming Section 505(b)(1) approval process, which would likely require that we sponsor or conduct additional nonclinical and/or clinical studies. For example, upon approval in March 2022 of a Section 505(b)(2) NDA for the treatment of facial angiofibroma associated with tuberous sclerosis, Hyftor, a topical gel product containing sirolimus (also known as rapamycin), received three years of new product exclusivity. If another rapamycin topical product were to receive three-year exclusivity for a condition of use that overlaps with QTORIN rapamycin, approval of QTORIN rapamycin would be delayed until the expiration of such exclusivity.

It is also not uncommon for a sponsor of an approved product to file a citizen petition with the FDA seeking to delay approval of, or impose additional approval requirements for, pending competing products. If successful, such petitions can significantly delay, or even prevent, the approval of the new product. However, even if the FDA ultimately denies such a petition, the FDA may substantially delay approval while it considers and responds to the petition.

Any such delay could dramatically reduce our expected market potential for our QTORIN rapamycin for any disease indication and could materially adversely affect our business, financial condition, results of operations and prospects.

Serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.

As we continue our development of our product candidates and initiate additional preclinical studies or clinical trials of these or future product candidates, if any, serious adverse events, unacceptable levels of toxicity, undesirable side effects or unexpected characteristics may emerge. Although we have designed QTORIN rapamycin for topical application and in a manner that we believe will not result in systemic absorption, systemic exposure to rapamycin, the active ingredient in our lead product candidate, at levels consistent with the approved oral dosage form, is known to result in significant adverse reactions, including peripheral edema, hypertriglyceridemia, hypertension, hypercholesterolemia, creatinine increases, abdominal pain, diarrhea, headache, fever, urinary tract infection, anemia, nausea, arthralgia, pain and thrombocytopenia. Investigators may attribute infectious diseases occurring during clinical trials of QTORIN rapamycin to suspected or possible immunosuppression, based on the systematic mechanism of action of rapamycin. Further, we have conducted and continue to conduct open-label studies of QTORIN rapamycin and, without a concurrent control arm, adverse events may be attributed to QTORIN rapamycin that may be a result of background disease or other external factors. Other APIs we select for our product candidates may have similar adverse event profiles. The emergence of any such serious adverse events, unacceptable levels of toxicity, undesirable side effects or unexpected characteristics could cause difficulty recruiting and retaining participants for our trials or we may abandon these product candidates, institute burdensome monitoring programs or limit their development to

more narrow uses, less frequent dosing, lower potency levels or subpopulations in which the serious adverse events, unacceptable levels of toxicity, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk/benefit perspective. The FDA, an IRB, or equivalent foreign regulatory authorities, may also require that we suspend, discontinue, or limit our clinical trials based on safety information or that there is inadequate prospect of treatment benefit. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the product candidate.

Additionally, if one or more of our product candidates receive marketing approval, and we or others identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may suspend, withdraw or limit their approvals of such products;
- regulatory authorities may require additional warnings, precautions, modification or limitations of use in the labeling;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to implement a REMS;
- we may decide to recall or remove such products from the marketplace;
- we may be required to conduct additional clinical trials as post-marketing requirements;
- we could be sued and held liable for harm caused to patients; and
- our reputation and physician or patient acceptance of our products may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, which could materially adversely affect our business, financial condition, results of operations and prospects.

Fast Track Designation has been granted for QTORIN rapamycin for the treatment of microcystic LM, the treatment of cutaneous VMs and the treatment of angiokeratomas, but we may never be able to realize the benefits of such designation for QTORIN rapamycin or any other indications or future product candidates, if granted, and such designation may not lead to a faster development, regulatory review, approval process or increase the likelihood that our product candidates will receive marketing approval.

We were granted Fast Track Designation by the FDA for QTORIN rapamycin for the treatment of microcystic LM, the treatment of cutaneous VMs and the treatment of angiokeratomas and may seek such designation for QTORIN rapamycin for other indications, and for certain other product candidates. If a drug is intended for the treatment of a serious or life-threatening disease and preclinical or clinical data demonstrates the potential of the new drug to address unmet medical needs for such disease, the drug's sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot be assured that the FDA would decide to grant it. Even if we do receive Fast Track Designation, as we have for QTORIN rapamycin for the treatment of microcystic LM, the treatment of cutaneous VMs and the treatment of angiokeratomas, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development programs. Many drugs that have received Fast Track Designation have failed to obtain approval.

Breakthrough Therapy Designation has been granted for QTORIN rapamycin for the treatment of microcystic LM but we may never be able to realize the benefits of such designation for QTORIN rapamycin or any other indications

or future product candidates, if granted, and such designation may not lead to a faster development, regulatory review or approval process or increase the likelihood that our product candidates will receive marketing approval.

We were granted Breakthrough Therapy Designation by the FDA for QTORIN rapamycin for the treatment of microcystic LM and may seek such designation for QTORIN rapamycin for other indications, and for certain other product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the NDA.

Designation as a breakthrough therapy is at the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a drug may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and it would not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates is granted a breakthrough therapy designation, the FDA may later decide that the product candidate no longer meets the conditions for designation.

Interim, topline or preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim, topline or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary or topline data we previously published. For example, we reported topline data from our Phase 3 clinical trial of QTORIN rapamycin for the treatment of microcystic LM in the first quarter of 2026 and we reported topline data from our Phase 2 clinical trial of QTORIN rapamycin for the treatment of cutaneous VMs in the fourth quarter of 2025, but any such topline or preliminary data may change following further auditing. As a result, preliminary and topline data should be viewed with caution until the final data are available. If the interim, topline, or preliminary data that we report differs from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for and commercialize our product candidates, our business, operating results, prospects or financial condition may be harmed. In addition, the information we choose to publicly disclose regarding a particular clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay the pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Failure to allocate resources or capitalize on strategies in a successful manner will have an adverse impact on our business.

We may choose not to continue developing or commercializing any of our product candidates at any time during development or after approval, if granted, which would reduce or eliminate our potential return on investment for those product candidates.

At any time, we may decide to discontinue the development or commercialization of any of our product candidates during or after approval, if granted, for a variety of reasons, including, but not limited to, the appearance of new technologies that render our product obsolete, competition from a competing product or changes in or inability to comply with applicable regulatory requirements. If we terminate a program in which we have invested significant resources, we will not receive any return on our investment and we will have missed the opportunity to allocate those resources to potentially more productive uses.

If we seek to market any product candidates in our pipeline in countries other than the United States, we will need to comply with the regulations of each country in which we seek to market our products. Additionally, although our trials are currently being conducted in the U.S., we may conduct clinical trials for our product candidates at clinical trial sites outside the U.S. and the FDA and equivalent non-U.S. regulatory authorities may not accept data from such sites.

None of our product candidates are currently approved for sale by any government authority. If we fail to comply with regulatory requirements in any market we decide to enter, or to obtain and maintain required approvals, or if regulatory approvals in the relevant markets are delayed, our target market will be reduced and our ability to realize the full market potential of our product candidates, if approved, will be harmed. Marketing approval in one jurisdiction, including the United States, does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one jurisdiction may have a negative effect on the regulatory process in others. Failure to obtain a marketing approval in countries in which we seek to market our products or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for any of our products. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional nonclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries.

Additionally, although our trials are currently being conducted in the U.S., we may in the future choose to conduct one or more of our clinical trials at clinical trial sites outside the United States, including in Canada and Europe. Although the FDA or equivalent foreign regulatory authority may accept data from clinical trial sites conducted outside the United States or the applicable jurisdiction, acceptance of such study data by the FDA or equivalent foreign regulatory authority may be subject to certain conditions. Where data from foreign clinical trial sites are intended to serve as the basis for marketing approval in the United States, the FDA will not approve the application on the basis of foreign data alone unless those data are applicable to the U.S. population and U.S. medical practice; the site study conduct was performed by clinical investigators of recognized competence; and the data are considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. Many foreign regulatory authorities have similar requirements. In addition, such foreign studies would be subject to the applicable local laws of the foreign jurisdictions where the studies are conducted. Additionally, recent policy proposals in the United States may make acceptance by the FDA or inclusion of foreign clinical trial data in a marketing application more difficult. There can be no assurance the FDA or equivalent foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or equivalent foreign regulatory authority does not accept such data, it would likely result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan.

Governments outside of the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations. To obtain

coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available procedures. If reimbursement of our product candidates is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, it could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Risks Related to Commercialization of Our Product Candidates, if Approved

Even if QTORIN rapamycin, QTORIN pitavastatin or any future product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

Even if QTORIN rapamycin for the treatment of microcystic LMs, for the treatment of cutaneous VMs or the treatment of clinically significant angiokeratomas or QTORIN pitavastatin for the treatment of DSAP or any future product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, patient advocacy groups, third-party payors and others in the medical community. If our product candidates do not achieve an adequate level of acceptance, we may never be able to generate adequate product revenue or become profitable. The degree of market acceptance of a product candidate, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the safety, efficacy, risk-benefit profile and potential advantages compared to alternative or existing treatments, which include, with respect to microcystic LM,
- surgery, sclerotherapy, laser, and cryotherapy, any alternative treatment options of which physicians may perceive to be adequately effective or to present less risk for some or all patients;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies over existing treatment alternatives;
- support from patient advocacy groups;
- side effects that may be attributable to our product candidates and the difficulty of or costs associated with resolving such side effects;
- the timing of market introduction of our product candidates as well as competitive products;
- the clinical indications for which a product candidate is approved;
- restrictions on the use of our product candidates in the labeling approved by regulatory authorities, including boxed warnings, contraindications, or a REMS, which may not be required of alternative treatments and competitors' products;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the effectiveness of our sales, marketing and market access efforts;
- the cost of treatment in relation to alternative treatments or methods of symptom management;
- our ability to offer our products, if approved, for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- publicity relating to our product candidates or those of our competitors;
- the availability of third-party coverage and adequate reimbursement at any given price level of each of our product candidates and patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement; and

- utilization controls imposed by third-party payors, such as prior authorizations and step edits.

We cannot assure you that our current or future product candidates, if approved, will achieve market acceptance among physicians, patients, patient advocacy groups, third-party payors or others in the dermatological community necessary for commercial success. Any failure by our product candidates that obtain regulatory approval to achieve market acceptance or commercial success could materially adversely affect our business, financial condition, results of operations and prospects.

We currently have no sales organization and have no experience as a company in commercializing products, and we may have to invest significant resources to develop these capabilities. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may not be able to generate product revenue.

We have never commercialized a product. To achieve commercial success for any product for which we obtain marketing approval, we will need a sales and marketing organization. We do not currently have any infrastructure for the sales, marketing, or distribution of any product, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market QTORIN rapamycin, QTORIN pitavastatin or any other planned or future product candidate, if approved, we must build our sales, distribution, marketing, managerial and other nontechnical capabilities or make arrangements with third parties to perform these services.

We believe that we will be able to commercialize QTORIN rapamycin for the treatment of microcystic LM and our QTORIN platform for each of our other targeted disease programs, if approved, with a specialized sales force that targets a focused subset of medical dermatologists, and is supported by sales management, medical liaisons, market access, an internal marketing group, and distribution support. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, provide adequate training to sales and marketing personnel, and effectively manage geographically dispersed sales and marketing teams to generate sufficient demand. Other factors that may inhibit our efforts to commercialize our product candidates, once approved, include:

- the ability to recruit and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs, and other support personnel;
- the ability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing our product candidates;
- the ability to maintain our collaborative relationships with patient advocacy groups and leverage those relationships to increase patient identification and outreach and the rate of new patient acceptance of our product candidates;
- the ability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and other healthcare providers regarding rare diseases and our future products;
- the costs associated with training sales and marketing personnel on legal and regulatory compliance matters and monitoring their actions;
- the implementation of effective and robust promotional compliance controls. If any of our product candidates are approved, our promotional and medical affair activities are subject to scrutiny by the FDA's Office of Prescription Drug Promotion ("OPDP");
- the ability to negotiate and enter into commercial, supply, and distribution contracts to support commercialization efforts, and to hire and manage additional qualified personnel;
- the clinical indications for which the products are approved and the claims that we may make for the products;
- the ability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;

- limitations of use, contraindications, or warnings, including boxed warnings, contained in the products' approved labeling;
- any distribution and use restrictions imposed by the FDA or to which we agree as part of a mandatory REMS or voluntary risk management plan;
- liability for sales or marketing personnel who fail to comply with the applicable legal and regulatory requirements;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization or engaging a contract sales organization.

If we are unable to establish adequate sales, marketing, and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our product candidates, if approved, and will not become profitable. We may be competing with companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If we enter into arrangements with third parties to perform sales, marketing and distribution services in the United States or in any markets outside of the United States, our revenues from product sales and our profitability, if any, may be lower than if we were to market, sell and distribute any products that we develop ourselves in all such territories. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates, if approved, or may be unable to do so on terms that are acceptable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates, if approved.

Our estimated market opportunities for our product candidates are subject to numerous uncertainties and may prove to be inaccurate. If we have overestimated the size of our market opportunities, our future growth may be limited.

Our estimated addressable markets and market opportunities for our product candidates are based on a variety of inputs, including data published by third parties, including patient advocacy groups, our own market insights and internal market intelligence, and internally generated data and assumptions. We have not independently verified any third-party information and cannot assure you of our accuracy or completeness. Market opportunity estimates, whether obtained or derived from third-party sources or developed internally, are subject to significant uncertainty and are based on assumptions and estimates that may not prove to be accurate. While we believe our market opportunity estimates are reasonable, such information is inherently imprecise. In addition, our assumptions and estimates of market opportunities are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including but not limited to those described in this "*Risk Factors*" section. If these third-party or internally generated data prove to be inaccurate or we make errors in our assumptions based on that data, our actual market may be more limited than our estimates. In addition, these inaccuracies or errors may cause us to misallocate capital and other critical business resources, which could harm our business.

The size of the markets for our product candidates has not been established and may be smaller than we estimate.

Our estimates of the annual total addressable markets for our product candidates are based on internal and third-party estimates, including, without limitation, estimated incidence and prevalence of these diseases, and estimated annual price per patient for our product candidates. In addition, because there are currently no FDA-approved therapies indicated for the treatment of microcystic LMs, cutaneous VMs, clinically significant angiokeratomas or DSAP those patients may not regularly seek care from a treating physician and consequently may not be prescribed our product candidates, if approved. While we believe our assumptions and the data underlying our estimates are reasonable, these assumptions and estimates may not be correct and the conditions supporting our assumptions or estimates may change

at any time, thereby reducing the predictive accuracy of these underlying factors. As a result, our estimates of the annual total addressable market for our product candidates may prove to be incorrect. If the annual total addressable markets for our product candidates are smaller than we have estimated, this may have an adverse effect on our business, financial condition, results of operations, and prospects.

If we are unable to achieve and maintain coverage and adequate levels of reimbursement for any of our product candidates for which we receive regulatory approval, or any future products we may seek to commercialize, our commercial success may be severely hindered.

As to any of our product candidates that become available by prescription only, our success will depend on the availability of coverage and adequate reimbursement for our product from third-party payors. Because there are currently no products approved for the treatment of microcystic LM, cutaneous VMs, clinically significant angiokeratomas or DSAP, the pricing and reimbursement of our product candidates, if approved, is uncertain. Novel medical products, if covered at all, may be subject to enhanced utilization management controls designed to ensure that the products are used only when medically necessary. Such utilization management controls may discourage the prescription or use of a medical product by increasing the administrative burden associated with our prescription or creating coverage uncertainties for prescribers and patients. Moreover, our target patient populations are small, as a result of which the pricing and third-party payor reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. Eligibility for reimbursement does not imply that a medical product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected.

Patients who are prescribed medicine for the treatment of their diseases generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. The availability of coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and private third-party payors is critical to new product acceptance. If any of our product candidates fail to demonstrate attractive efficacy and safety profiles, they may not qualify for coverage and reimbursement. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate, particularly given the small patient populations for our targeted indications, or may require co-payments that patients find unacceptably high. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products.

In addition, third-party payors, whether foreign or domestic, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In addition, in the United States, although private third-party payors tend to follow Medicare, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

We believe that future coverage and reimbursement will likely be subject to increased restrictions in both the United States and in international markets. Third-party coverage and reimbursement for any of our product candidates for which we may receive regulatory approval may not be available or adequate in either the United States or international markets, which could materially adversely affect our business, financial condition, results of operations and prospects.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do, thus rendering our products non-competitive, obsolete or reducing the size of our market.

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face and will continue to face competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, government agencies and public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

In addition, some of the market demand for topical rapamycin or pitavastatin may be satisfied by state-licensed compounding pharmacies lawfully operating under Section 503A of the FDCA. Although such pharmacies will be unable to compound any drug that is essentially a copy of QTORIN rapamycin or QTORIN pitavastatin, if approved, a compounded product would not be considered a copy of QTORIN rapamycin or QTORIN pitavastatin if there were a difference between the FDA-approved and commercially available product and the compounded product that was made for an individual patient and which the prescribing practitioner determines produces a significant difference for that patient. Physicians may determine that such a significant difference exists for some or all of their patients and may choose to prescribe compounded rapamycin or pitavastatin because, if QTORIN rapamycin or QTORIN pitavastatin is approved, it would be a component of an FDA-approved drug product. In addition, if the FDA-approved drug product is not commercially available and thus added to the FDA's published drug shortage list, compounders also would be able to copy it without the necessity of noting a significant difference between the compounded formulation and the FDA-approved drug. In the event compounders engage in the compounding of rapamycin or pitavastatin products if FDA approval of QTORIN rapamycin or QTORIN pitavastatin is obtained, we could be subject to significant competition from compounding pharmacies that prepare those compounded formulations.

The companies against which we may compete may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ourselves, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. Because of our primary focus on rare diseases, if our product candidates achieve marketing approval, we expect to seek premium pricing.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to conducting marketing and sales activities in international jurisdictions and entering into international business relationships, including:

- different regulatory requirements for approval of drugs in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- the need to seek additional patent approvals, licenses to patents held by third parties and/or face claims of infringing third-party patent rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;

- economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- differing reimbursement regimes and price controls in certain non-U.S. markets;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the FCPA, the U.K. Bribery Act 2010 or other comparable foreign regulations;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including pandemics, or other outbreaks of infectious disease, earthquakes, typhoons, floods and fires.

These and other risks associated with international operations may materially adversely affect our ability to attain or maintain profitable operations.

Risks Related to Our Business and Operations

We will need to increase the size of our organization, and we may experience difficulties in executing our growth strategy and managing any growth, including with respect to any acquired businesses, therapeutic candidates or technologies.

As of March 25, 2026, we had 29 full-time employees. Our management and personnel, systems and facilities currently in place are not adequate to support our future growth. We will need to expand our managerial, operational, finance and other resources in order to manage our operations and clinical trials, continue our development activities, commercialize our lead product candidates or any future product candidates and operate as a public company. In order to effectively execute our growth strategy, we will need to identify, recruit, retain, incentivize and integrate additional employees in order to expand our ability to:

- manage our clinical trials effectively;
- manage our internal development and operational efforts effectively while carrying out our contractual obligations to third parties;
- continue to improve our operational, financial, management and regulatory compliance controls and reporting systems and procedures;
- develop a marketing, sales and distribution capability;
- manage our commercialization activities for our product candidates effectively and in a cost-effective manner;
- establish and maintain relationships with development and commercialization partners; and
- manage our third-party supply and manufacturing operations effectively and in a cost-effective manner, while increasing production capabilities for our current product candidates to commercial levels.

Should we in the future acquire any complementary business, therapeutic candidates or technologies, our ability to integrate and manage acquired businesses, therapeutic candidates or technologies will depend upon a number of factors, including the size of the acquired business, the complexity of any therapeutic candidate or technology and the resulting difficulty of integrating the acquired business's operations, if any. Our relationship with current employees

or employees of any acquired business may become impaired. We may also be subject to unexpected claims and liabilities arising from such acquisitions. These claims and liabilities could be costly to defend, could be material to our financial position and might exceed either the limitations of any applicable indemnification provisions or the financial resources of the indemnifying parties. There can also be no assurance that we will be able to assess ongoing profitability and identify all actual or potential liabilities of a business, therapeutic candidate or technology prior to our acquisition. If we acquire businesses, therapeutic candidates or technologies that result in assuming unforeseen liabilities in respect of which it has not obtained contractual protections or for which protection is not available, this could materially adversely affect our business, financial condition, results of operations and prospects.

Our future financial performance and our ability to commercialize our product candidates, if approved, will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of our attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities.

If we are unable to successfully identify, recruit, retain, incentivize and integrate additional employees and otherwise expand our managerial, operational, finance and other resources, our business and operational performance will be materially and adversely affected.

If we fail to attract and retain management and other key personnel, we may be unable to continue to successfully develop our current and any future product candidates, commercialize our product candidates or otherwise implement our business plan.

Our ability to compete in the highly competitive pharmaceuticals industry depends upon our ability to attract and retain highly qualified managerial, scientific, medical, sales and marketing and other personnel. We are highly dependent on our management and scientific personnel, including Wesley H. Kaupinen, our Chief Executive Officer, Matthew Korenberg, our Chief Financial Officer, Kathleen Goin, our Chief Operating Officer, Jeffrey Martini Ph.D., our Chief Scientific Officer, and Ashley Kline, our Chief Commercial Officer. The loss of the services of any of these individuals could impede, delay or prevent the successful development of our product candidates and otherwise negatively impact our ability to successfully implement our business plan. If we lose the services of any of these individuals, we might not be able to find suitable replacements on a timely basis or at all, and our business could be harmed as a result. We maintain “key person” insurance only for our Chief Executive Officer.

Our employment agreement with Mr. Kaupinen may be terminated immediately by us for cause or by Mr. Kaupinen with good reason, or upon thirty days’ notice if terminated for any other reason. In order to retain valuable employees at our company, in addition to salary and cash incentives, we provide stock options that vest over time. The value to employees and key consultants of stock options that vest over time will be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract offers from other companies.

We might not be able to attract or retain qualified management and other key personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses, particularly in the eastern Pennsylvania area where we are headquartered. We could have difficulty attracting experienced personnel to our company and may be required to expend significant financial resources in our employee recruitment and retention efforts. Many of the other pharmaceutical companies with whom we compete for qualified personnel have greater financial and other resources, different risk profiles and longer histories in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. If we are not able to attract and retain the necessary personnel to accomplish our business objectives, we may experience constraints that will harm our ability to implement our business strategy and achieve our business objectives.

In addition, we have scientific and clinical advisors who assist us in formulating our development and clinical strategies. These advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, our advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with us.

We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability for our product candidates and may have to limit our commercialization if we obtain regulatory approval.

The use of our product candidates in clinical trials, and the sale of any of our product candidates for which we obtain regulatory approval, exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. For example, we may be sued if any product candidate we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against these claims, we will incur substantial liabilities or be required to limit development or commercialization of our product candidates for which we obtain regulatory approval. Even successful defense would require significant financial and management resources. Regardless of merit or eventual outcome, product liability claims may result in:

- loss of revenue from decreased demand for our product candidates, if approved;
- impairment of our business reputation or financial stability;
- costs of related litigation;
- substantial monetary awards to patients or other claimants;
- exhaustion of any available insurance and our capital resources;
- diversion of management attention;
- withdrawal of clinical trial participants and potential termination of clinical trial sites or entire clinical programs;
- the inability to commercialize our product candidates, if approved;
- significant negative media attention;
- decrease in our stock price; or
- initiation of investigations, and enforcement actions by regulators; and product recalls, withdrawals, revocation of approvals, or labeling, marketing or promotional restrictions.

We currently hold $5.0 million in product liability insurance coverage in the aggregate, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we initiate additional clinical trials. We will need to further increase our insurance coverage if we commence commercialization of any of our product candidates for which we obtain marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. On occasion, large judgments have been awarded in class action lawsuits based on therapeutics that had unanticipated side effects. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. A successful product liability claim or series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business and our prospects.

Unfavorable global economic or political conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A global financial crisis or a global or regional political disruption could cause extreme volatility in the capital and credit markets. A severe or prolonged economic downturn or political disruption could result in a variety of risks to our business, including weakened demand for our lead product candidates or any future product

candidates, if approved, and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy or political disruption could also strain our manufacturers or suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and it cannot anticipate all the ways in which the political or economic climate and financial market conditions could materially adversely affect our business, financial condition, results of operations and prospects.

We are subject to stringent and changing laws, regulations and standards, and contractual obligations relating to privacy, data protection, and data security. Our business and operations would suffer in the event of cybersecurity incidents, data breaches, or system failures, and we face risks related to our collection and use of data, which could result in investigations, inquiries, litigation, fines, legislative and regulatory action and negative press about our privacy and data protection practices.

Our information security systems and internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war, and telecommunication and electrical failures. Like other companies in our industry, we, and our third party vendors, have experienced threats and cybersecurity incidents relating to our information technology systems and infrastructure. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product candidate development and, if such product candidates are approved, commercialization programs.

Additionally, our business processes personal data, including some data related to health. When conducting clinical trials, we face risks associated with collecting trial participants' data, especially health data, in a manner consistent with applicable laws and regulations. Attempts to disrupt or gain unauthorized access to our and our third-party vendors' information systems from malicious third parties or insider threats may incorporate widely varying and frequently changing tactics, which may be enhanced or facilitated by artificial intelligence. We also face risks inherent in handling large volumes of data and in protecting the security of such data. We could be subject to attacks on our systems by outside parties or fraudulent, inadvertent, or other inappropriate behavior by our service providers and/or insider employees. Third parties may also gain access to the company's systems using stolen or inferred credentials, computer malware, denial-of-service attacks, ransomware viruses, spamming, social engineering fraud (including phishing attacks) or other means to threaten or compromise the security, confidentiality, integrity and availability of systems and information, and unauthorized third-parties may use such access to obtain personal data. A successful cyberattack could result in theft, destruction or misuse of intellectual property, data, or other misappropriation of assets, or otherwise compromise our confidential or proprietary information and disrupt our operations.

We rely on our third-party service providers to implement effective security measures and identify and correct for any such failures, deficiencies, vulnerabilities, compromises, cybersecurity incidents or data breaches. We also rely on our employees and consultants to safeguard their security credentials and follow our policies and procedures regarding use and access of computers and other devices that may contain protected information and our sensitive information. If we, our third-party providers, or our employees and consultants fail to maintain or protect our information technology systems, infrastructure and data integrity effectively or fail to anticipate, plan for or manage significant disruptions to our information technology systems and infrastructure, we or our third-party providers could have difficulty preventing, detecting and controlling cyberattacks. Cyberattacks generally are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Although we devote resources designed to protect our information systems and infrastructure, we realize that cyberattacks are a threat, and there can be no assurance that our efforts will prevent or adequately address information security compromises, cybersecurity incidents or data breaches that would result in business, legal, financial or reputational harm to us, or would have a material adverse effect on our results of operations and financial condition.

Any failure to prevent or mitigate cybersecurity incidents, data breaches, or other security compromises could require us to notify relevant stakeholders (including affected individuals, investors and regulators), and subject us to individual or consumer class action litigation and governmental investigations and proceedings by federal, state and local regulatory entities in the United States and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities, including various domestic and international privacy and security regulations. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations.

Further, although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or data breach.

The legislative and regulatory landscape for privacy and data protection continues to evolve. In the United States, numerous states have adopted privacy and security laws and regulations that may be more stringent than applicable federal law. These state laws allow for statutory fines for noncompliance. For example, California enacted the California Consumer Privacy Act of 2018 ("CCPA"), which creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal data. The CCPA requires covered companies to provide certain disclosures to consumers about its data collection, use and sharing practices, and to provide affected California residents with ways to opt-out of certain sales or transfers of personal information. While there is currently an exception for protected health information that is subject to HIPAA, as currently written, the CCPA may impact our business activities. In addition, the California Privacy Rights Act of 2020 ("CPRA"), which went into effect on January 1, 2023, imposes additional obligations on companies covered by the legislation and significantly modifies the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that is vested with authority to implement and enforce the CCPA and CPRA. Following California's lead, similar laws have been passed in numerous other states, including Virginia, Colorado, Connecticut, New Jersey, New Hampshire and other states have proposed such laws. While these laws incorporate many similar concepts to those in the CCPA and CPRA, there are also several key differences in the scope, application, and enforcement of the law that will change the operational practices of regulated businesses. The new laws will, among other things, impact how regulated businesses collect and process personal sensitive data, conduct data protection assessments, transfer personal data to affiliates, and respond to consumer rights requests. There are also states that are specifically regulating health information. For example, Washington's My Health My Data Act, which became effective on March 31, 2024, regulates the collection and sharing of health information and has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain specific types of information. For example, a small number of states have passed laws that regulate biometric data specifically. The existence of a patchwork of privacy laws in different states will make our compliance obligations more complex and costly and may increase the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. We may also in the future be subject to data protection laws and regulations of other jurisdictions, such as the EU's GDPR, which provides data subjects with certain rights and requires organizations to adopt technical and organizational safeguards to protect personal data.

At the federal level, failing to take appropriate steps to keep consumers' personal information secure may constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act, 15 U.S.C § 45(a). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business and the cost of available tools to improve security and reduce vulnerabilities. Regulators and legislators in the U.S. are also increasingly scrutinizing and restricting certain personal data transfers and transactions involving foreign countries. For example, the Department of Justice's January 8, 2025, rule on "Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons," prohibits data brokerage transactions involving certain sensitive personal data categories, including health data, genetic data, and biospecimens, to countries of concern, including China. The regulations also restrict certain investment agreements, employment agreements and vendor agreements involving such data and countries of concern, absent specified cybersecurity controls. Actual or alleged violations of these regulations may be punishable by criminal and/or civil sanctions, and may result in exclusion from participation in federal and state programs.

All of these evolving compliance and operational requirements impose significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. In addition, such requirements may require us to modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects. Any failure or perceived failure by us to comply with any applicable federal, state or foreign laws and regulations relating to data privacy and security could result in damage to our reputation, as well as proceedings or litigation by governmental agencies or other third parties, including class action privacy litigation in certain jurisdictions, which would subject us to significant fines, sanctions, awards, injunctions, penalties or judgments. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our use of new and evolving technologies, such as artificial intelligence, may present risks and challenges that can impact our business, including by posing cybersecurity and other risks to our confidential and/or proprietary information, including personal information, and as a result we may be exposed to reputational harm and liability.

We may use and integrate artificial intelligence (AI) into our business processes both in our own development and implementation of AI and through the adoption of commercially available tools. Use of this technology could pose cybersecurity, data privacy, IT, intellectual property, regulatory, legal, operational, competitive, reputational and other risks and challenges that could affect our business. Specifically, risks related to accuracy, bias, AI hallucinations, discrimination, harmful content, misinformation, fraud, scams, targeted attacks (including model poisoning or data poisoning), surveillance, data leakage, inequality, environmental harms, and other harms may flow from our development, use, or deployment of AI technologies. If we enable or use solutions that draw controversy due to perceived or actual negative societal impact, we may experience brand or reputational harm, competitive harm or legal liability.

A growing number of legislators and regulators are adopting laws and regulations and have focused enforcement efforts on the adoption of AI, and use of such technologies in compliance with ethical standards and societal expectations. These developments may increase our compliance burden and costs in connection with use of AI and lead to legal liability if we fail to meet evolving legal standards or if use of such technologies results in harms or other causes of action we did not predict. For example, the EU's Artificial Intelligence Act ("AI Act") is now in effect and is expected to undergo amendments, as introduced in the EU's November 2025 Digital Omnibus. As enacted, the AI Act imposes significant obligations on providers and deployers of AI systems, and encourages providers and deployers of AI systems to account for EU ethical principles in their development and use of these systems. The scope of requirements depends on legal and risk determinations that rely on novel legal provisions that have not yet been interpreted by courts or regulators, and non-compliance can lead to significant fines.

In the U.S., the AI regulatory environment is complex and uncertain. Over the past year, states have advanced, and in some cases passed, dozens of laws focusing on AI governance and regulation, including on deployment of AI in healthcare settings. At the federal level, the Trump Administration has endorsed a federal moratorium on the enforcement of state AI laws, including through a December 11, 2025, executive order on "Ensuring a National Policy Framework for Artificial Intelligence." So far, these efforts have not been successful at curtailing state action on AI regulation, contributing to a complicated legislative patchwork, which may be litigated in state and federal courts. In addition, various federal regulators have issued guidance and focused enforcement efforts on the use of AI in regulated sectors. The FDA, for example, issued draft guidance on the use of AI in regulatory decision-making for drug and biological products that centers on the context of use while establishing a credibility assessment framework for establishing and evaluating AI model outputs intended to support regulatory decision-making. If we develop or use AI systems governed by these laws or regulations, including as informed by regulatory guidance, we will need to meet various standards of data quality, transparency, monitoring and human oversight, and we would need to adhere to specific and potentially burdensome and costly ethical, accountability, and administrative requirements, with the potential for significant enforcement or litigation in the event of any perceived non-compliance.

The rapid evolution of AI will require the application of significant resources to design, develop, test and maintain such systems to help ensure that AI is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. The use of certain AI technologies can also give rise to intellectual property risks, including by disclosing or otherwise compromising our confidential or proprietary intellectual property, or by undermining our ability to assert or defend ownership rights in intellectual property created with the assistance of artificial intelligence tools.

Our vendors may in turn incorporate AI tools into their offerings, and the providers of these AI tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of artificial intelligence, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. In addition, the use of generative AI models in our internal or third-party systems may create new attack surfaces or methods for adversaries, which could impact us and our vendors. The integration of AI systems, by us or by our vendors, may increase cybersecurity risk. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

If we or our third-party contractors fail to comply with environmental, health and safety laws and regulations, it could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and our third-party contractors are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. Although we maintain workers' compensation insurance to cover costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

Even if we receive regulatory and marketing approval of our product candidates, we will be subject to extensive and ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any regulatory approvals or other marketing authorizations we obtain for our product candidates may be subject to limitations on the indicated uses for which the product may be marketed or the conditions of approval or marketing authorization or contain requirements for potentially costly post-market testing and surveillance to monitor the safety and efficacy of the product candidate. The FDA may also require a REMS as a condition of approval of any of our existing or future drug product candidates, such as QTORIN rapamycin for the treatment of microcystic LM, which could include requirements for a medication guide, physician communication plans or additional elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or an equivalent foreign regulatory authority authorizes our product candidates for marketing, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and GCP requirements for any clinical trials that we conduct post-approval. For certain commercial prescription drug products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Accordingly, we and others with whom we work must expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control. Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or with our CMOs or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of our product candidates, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- warning or untitled letters, Form 483s, or holds on clinical trials;
- refusal by the FDA to accept new marketing applications or supplements, approve or otherwise authorize for marketing pending applications or supplements to applications filed by us or suspension or revocation of approvals or other marketing authorizations;

- product seizure or detention, or refusal to permit the import or export of our product candidates; and
- fines, injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, and we may not achieve or sustain profitability, which could materially adversely affect our business, financial condition, results of operations and prospects.

Our product candidates, if approved for marketing, may cause or contribute to adverse medical events that we are required to report to the FDA, and if we fail to do so, we would be subject to sanctions that could harm our reputation, business, financial condition and results of operations. The discovery of serious safety issues with our product candidates, or a recall of our products either voluntarily or at the direction of the FDA or another governmental authority, if such products are marketed, could have a negative impact on us.

With respect to any of our product candidates in clinical testing or approved by the FDA, we will be subject to the FDA's safety reporting requirements. The timing of our obligation to report is triggered by the date we become aware of the adverse event as well as the nature of the event. We may fail to report adverse events of which we become aware within the prescribed timeframe. We may also fail to recognize that we have become aware of a reportable adverse event, especially if it is not reported to us as an adverse event or if it is an adverse event that is unexpected or removed in time from the use of the product. If we fail to comply with our reporting obligations, the FDA could take actions, including among other things, issuing FDA Form 483s, warning letters or untitled letters, administrative actions, criminal prosecution, imposition of civil monetary penalties, revocation of our approval or delay in approval of future products.

We may choose to voluntarily recall a product if any material deficiency is found. A recall could occur as a result of an unacceptable risk to health, component failures, malfunctions, manufacturing defects, labeling or design deficiencies, packaging defects or other deficiencies or failures to comply with applicable regulations. Product defects or other errors may occur in the future. Recalls involving our product candidates, if and when they are approved or otherwise authorized for marketing, could materially adversely affect our business, financial condition, results of operations and prospects.

We will be subject to healthcare laws and regulations relating to our business and could face substantial penalties if we are determined not to have fully complied with such laws, which would have an adverse impact on our business.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, customers and patients, will expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any products for which we obtain marketing approval. For a detailed discussion of these laws, see the section of this Form 10-K titled, "Business—Government Regulation—U.S. Anti-Kickback, False Claims and Other Healthcare Fraud and Abuse Laws."

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities may conclude that our business practices, including our consulting arrangements with and/or ownership interests by physicians and other healthcare providers, do not comply with current or future statutes, regulations, agency guidance or case law involving applicable healthcare laws. If our operations are found to be in violation of any of these or any other health regulatory laws that may apply to us, we may be subject to significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, individual imprisonment, possible exclusion from participation in Medicare, Medicaid and other U.S. healthcare programs, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Defending against any such actions can be costly, time-consuming and may

require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the above occur, it could materially adversely affect our business, financial condition, results of operations and prospects.

Disruptions at the FDA, the SEC and other government agencies or comparable regulatory authorities may extend the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. In addition, there is substantial uncertainty regarding new regulatory initiatives and how these might impact the FDA, its implementation of laws, regulations, policies and guidance, and its personnel. Similar initiatives may also be directed toward other government agencies which could prevent, limit or delay development and regulatory approval of our product candidates and negatively impact our business.

The ability of the FDA or comparable foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, leadership and policy changes that may otherwise affect the FDA's or comparable foreign regulatory authorities' ability to perform routine functions. In addition, government funding of the SEC and other government agencies or comparable foreign regulatory authorities on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies, including substantial leadership, personnel, and policy changes, have and may continue to slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would harm our business. Changes in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. Similar consequences would also result in the event of another significant shutdown of the federal government. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, or if geopolitical or global health concerns prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could materially adversely affect our business, financial condition, results of operations and prospects. Such changes could significantly impact the ability of the FDA to timely review and take action on our regulatory submissions, which could have a material adverse effect on our business. Further, in our operations as a public company, future government shutdowns or substantial leadership, personnel, and policy changes could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations. If the FDA is constrained in its ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

There is continued substantial uncertainty as to the extent and how the Trump Administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates. This uncertainty could present new challenges and/or opportunities as we navigate development of our product candidates. Some of these efforts have manifested to date in the form of personnel measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development and obtain the requisite regulatory approvals in the future. Moreover, the Trump Administration has proposed action to freeze or reduce the budget of the National Institutes of Health ("NIH") as related to its funding for medical research, which could decrease the ability of facilities that rely on NIH funding to enroll and conduct clinical trials or increase the costs to us of conducting clinical trials. Additionally, it is unclear whether the up to $2.6 million in grant funding we were awarded from the Orphan Products Grants Program to support our SELVA program will be adversely impacted or whether grant funding could be disrupted or terminated. There remains general uncertainty regarding future activities involving the current Administration. The Trump Administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic products. Alternatively, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by future governmental orders, regulations, policies or guidance as a result of the Trump Administration, there could be a material adverse effect on us and our business.

Healthcare reform measures may increase the difficulty and cost for us to successfully commercialize our product and product candidates, if approved, and affect the prices we may obtain.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could restrict or regulate post-approval activities relating to our product and product candidates, if approved, including implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For a detailed discussion of healthcare reform initiatives, see the section of this Form 10-K titled, "Business—Government Regulation—Healthcare Reform." We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing authorizations of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing authorization, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

The Inflation Reduction Act's ("IRA's") drug price negotiation provisions, inflation rebates, and Medicare Part D discount requirements, along with proposed most-favored-nation pricing models under the recently issued GLOBE, GUARD, and GENEROUS frameworks by the Centers for Medicare and Medicaid Services, could significantly impact our ability to obtain adequate pricing for our product candidates. Although a drug or biological product that has one or more orphan drug designations will be excluded from the IRA's price negotiation requirements, we do not know if additional drug pricing reforms could modify this exemption in the future. These provisions are subject to ongoing legal challenges, and their ultimate implementation and impact remain uncertain. Additionally, the One Big Beautiful Bill Act of 2025 included reductions in Medicaid funding, work requirements, and reenrollment requirements that could have the effect of decreasing utilization of, and reimbursement for, our products, if approved.

We expect that the IRA, as well as other healthcare reform measures that have and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for our product and product candidates, if approved, and could seriously harm our future revenues. Any reduction in reimbursement from Medicare, Medicaid, or other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or successfully commercialize our product and product candidates, if approved.

Any product candidates for which we are able to obtain regulatory approval in the future may become subject to unfavorable pricing regulations, third-party payor coverage and reimbursement policies, or healthcare reform initiatives.

Our ability to commercialize any of our other product candidates, if approved, successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government payor programs at the federal and state level, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. For a detailed discussion of coverage and reimbursement, see the section of this Form 10-K titled, "Business—Government Regulation—Coverage and Reimbursement."

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications or procedures. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Coverage and reimbursement may not be available for any drug that we or our collaborators commercialize and, even if these are available, the level of reimbursement for a product or procedure may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our product candidates, if approved, may be difficult. We may be required to conduct expensive pharmacoeconomic studies to seek to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize any of our other product candidates for which marketing approval is obtained.

As discussed above, the IRA contains substantial drug pricing reforms, including the establishment of a drug price negotiation program within the HHS, the establishment of rebate payment requirements on manufacturers of certain drugs payable under Medicare Parts B and D whose prices have increased at a rate greater than the rate of inflation, and the requirement for manufacturers to provide discounts on Part D drugs. Substantial penalties can be assessed for noncompliance with the drug pricing provisions. Although a drug or biological product that has one or more orphan drug designations are excluded from the IRA's price negotiation requirements, we do not know if additional drug pricing reforms could eliminate this exemption and therefore affect the prices we can charge and reimbursement we receive for our product candidates, if approved, thereby reducing our profitability. The full extent of the IRA on our business and the pharmaceutical industry in general is not yet known.

Future efforts to reform healthcare or reduce costs under government insurance programs may result in lower reimbursement for our products and product candidates or exclusion of our products and product candidates from coverage. The cost containment measures that healthcare payors and providers are instituting and any healthcare reform could significantly reduce our revenue from the sale of any approved product candidates. We cannot provide any assurances that we will be able to obtain and maintain third-party coverage or adequate reimbursement for our product candidates in whole or in part.

If any of our product candidates are approved for marketing and we are found to have improperly promoted off-label uses, we may become subject to prohibitions on the sale or marketing of our products, product liability claims and significant fines, penalties and sanctions, and our brand and reputation could be harmed.

The FDA and other equivalent foreign regulatory authorities strictly regulate the marketing of and promotional claims that are made about drug products. In particular, a product may not be promoted for uses or indications that are not approved by the FDA or such other equivalent foreign regulatory authorities as reflected in the product's approved labeling. In addition, although we believe our product candidates may exhibit a lower risk of side effects or more favorable tolerability profile or better symptomatic improvement than other products for the indications we are studying, without head-to-head data, we will be unable to make comparative claims for our product candidates, if approved. If we receive regulatory approval for any of our product candidates and are found to have promoted any of our products for off-label uses, we may become subject to significant liability, which would materially harm our business. Both federal and state governments have levied large civil and criminal fines against companies for alleged improper promotion and have enjoined several companies from engaging in off-label promotion. If we become the target of such an investigation or prosecution based on our marketing and promotional practices, we could face similar sanctions, which would materially harm our business. In addition, management's attention could be diverted from our business operations, significant legal expenses could be incurred, and our brand and reputation could be damaged. The FDA has also previously requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we are deemed by the FDA to have engaged in the promotion of our products for off-label use, we could be subject to FDA regulatory or enforcement actions, including the issuance of an untitled letter, a warning letter, injunction, seizure, civil fine or criminal penalties. Further, FDA's OPDP actively scrutinizes promotional communications, including digital and social media; any materials that are false, misleading, or promote unapproved uses can lead to enforcement actions and could necessitate corrective communications. OPDP has increased efforts to monitor promotional activities, particularly those directed to consumer and patient audiences. It is also possible that other federal, state or foreign enforcement authorities might take action if they determine our business activities constitute promotion of an off-label use, which could result in significant penalties, including criminal, civil or administrative penalties, damages, fines, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations.

We cannot, however, prevent a physician from using our product candidates, once approved, as he or she may deem appropriate in his or her medical judgment even if such use falls outside of the scope of the approved indications. Physicians may also misuse our product candidates or use improper techniques, which may lead to adverse results, side effects or injury and, potentially, subsequent product liability claims. Furthermore, the use of our product candidates for indications other than those approved by the FDA and/or other regulatory authorities may not effectively treat such diseases which could harm our brand and reputation among both physicians and patients.

Our business activities may be subject to the FCPA and similar anti-bribery and anti-corruption laws of other countries in which we operate, as well as U.S. and certain foreign export controls, trade sanctions, and import laws

and regulations. Compliance with these legal requirements could limit our ability to compete outside of the U.S. market and subject us to liability if we violate them.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. Our business activities may be subject to the FCPA and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we operate. The FCPA generally prohibits companies and their employees and third-party intermediaries from offering, promising, giving or authorizing the provision of anything of value, either directly or indirectly, to a non-U.S. government official in order to influence official action or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. Additionally, in many other countries, hospitals owned and operated by the government, and doctors and other hospital employees would be considered foreign officials under the FCPA. Recently the SEC and Department of Justice have increased their FCPA enforcement activities with respect to biotechnology and pharmaceutical companies. There is no certainty that all our employees, agents or contractors, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, disgorgement, and other sanctions and remedial measures, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our brand, our international activities, our ability to attract and retain employees and our business, financial condition, results of operations and prospects.

In addition, our products and activities may be subject to U.S. and foreign export controls, trade sanctions and import laws and regulations. Governmental regulation of the import or export of our products, or our failure to obtain any required import or export authorization for our products, when applicable, could harm our international sales and adversely affect our revenue. Compliance with applicable regulatory requirements regarding the export of our products may create delays in the introduction of our products in international markets or, in some cases, prevent the export of our products to some countries altogether. Furthermore, U.S. export control laws and economic sanctions prohibit the shipment of certain products and services to countries, governments, and persons targeted by U.S. sanctions. If we fail to comply with export and import regulations and such economic sanctions, penalties could be imposed, including fines and/or denial of certain export privileges. Moreover, any new export or import restrictions, new legislation or shifting approaches in the enforcement or scope of existing regulations, or in the countries, persons, or products targeted by such regulations, could result in decreased use of our products by, or in our decreased ability to export our products to existing or potential customers with international operations. Any decreased use of our products or limitation on our ability to export or sell access to our products would likely adversely affect our business, financial condition, results of operations and prospects.

Risks Related to Our Reliance on Third Parties

We currently rely on CMOs to manufacture preclinical and clinical supplies of our product candidates and expect to rely on CMOs for the commercial supplies of any approved product candidates. The loss of these manufacturers, or their failure to provide us with sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.

We do not currently have nor do we plan to build or acquire the infrastructure or capability internally to manufacture supplies of our product candidates or the materials necessary to produce our product candidates for use in the conduct of our preclinical studies or clinical trials, and we lack the internal resources and the capability to manufacture any of our product candidates on a preclinical, clinical or commercial scale. Instead, we currently rely on a limited number of CMOs to manufacture preclinical and clinical supplies of our product candidates and intend to rely on such CMOs for the commercial supplies of any approved product candidates, and may partner with other third party manufacturers for our clinical and commercial supply of QTORIN rapamycin and QTORIN pitavastatin. Please see "*Business—Manufacturing*" for a discussion of our current manufacturing and supply agreements.

Reliance on CMOs entails risks to which we may not be subject if we manufactured product candidates ourselves, including:

- the possible failure of the third party to manufacture our product candidates according to our schedule, or at all, including if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- reliance on the third party for regulatory compliance and quality control and assurance and failure of the third party to comply with regulatory requirements;
- the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control (including a failure to manufacture our product candidates in accordance with our product specifications);
- a disruption to one or more of our CMOs' relevant operations;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or vehicle not being properly identified;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales;
- the risk that our CMOs face financial difficulties or declare bankruptcy; and
- the possibility of our failure to enter into agreements for manufacturing services, on commercially reasonable terms or at all, or the possibility of termination or nonrenewal of the agreement by the third party at a time that is costly or damaging to us

Moreover, there are a limited number of manufacturers capable of producing our product candidates, which exposes us to the risk of disruption in the supply of product candidates for our preclinical studies and clinical trials, and if approved, ultimately for commercial sale. In the case of QTORIN rapamycin which we are currently developing for the treatment of microcystic LMs, cutaneous VMs, and clinically significant angiokeratomas, there is a limited number of manufacturers that will work with an API, such as rapamycin, that has immunosuppressant properties. If our third-party manufacturing agreements were to be terminated for any reason, we may be unable to procure alternative manufacturers for clinical or commercial manufacture of QTORIN rapamycin, as applicable, on a timely basis or at all.

Additionally, while we have entered into agreements with a limited number of CMOs for the commercial manufacture of our existing product candidates, these organizations must complete a scale-up process that includes the completion of various technical and regulatory steps before they will be able to produce commercial supply of our QTORIN rapamycin. If our current CMOs fail for any reason to carry out our contractual duties or otherwise fails to meet our manufacturing requirements prior to our completion of the process of qualifying each as a commercial manufacturer, and we are unable to replace them with a new collaboration partner, our business, financial condition, results of operations and prospects could be materially and adversely affected.

We may be unable to enter into additional agreements with third-party manufacturers or suppliers or do so on favorable terms. Our anticipated reliance on a limited number of third party-manufacturers or suppliers exposes us to the following risks:

- reliance on the third party for regulatory, compliance and quality assurance;
- reliance on the third party for product development, analytical testing and data generation to support regulatory applications;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier, the issuance of an FDA Form 483 or warning letter or other enforcement action by the FDA or other regulatory authority;

- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of proprietary information, including trade secrets and know-how;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our drugs under specified storage conditions and in a timely manner.

If any of these risks materialize and impact our CMOs' ability to produce our product candidates, our CMOs will have no other means of producing our product candidates until the adverse impact is mitigated or us or they procure alternative manufacturing facilities or sources of supply. Though we carefully manage our relationships with our CMOs, there can be no assurance that we will not encounter challenges or delays in the future. The loss of any of our CMOs, or their failure to provide us with sufficient quantities at acceptable quality levels or prices, or at all, would materially and adversely affect our business.

If the manufacturers upon whom we rely fail to produce our product candidates or components pursuant to the terms of contractual arrangements with us or fail to comply with stringent regulations applicable to pharmaceutical manufacturers, we may face delays in the development and commercialization of, or be unable to meet demand for, our product candidates, if approved, and may lose potential revenues.

We have agreements governing the activities of the CMOs that manufacture our preclinical, clinical and commercial supply of our product candidates, and we expect to enter into agreements with additional CMOs in the future, but we have or will have limited influence and control over their actual performance and activities. If our CMOs do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with strictly enforced federal, state, and foreign regulations, or if there are disagreements between us and such parties, clinical development or marketing approval of our product candidates could be delayed.

All manufacturers of our product candidates and therapeutic substances must comply with cGMP requirements enforced by the FDA that are applicable to both finished products and their active components used both for clinical and commercial supply. The cGMP requirements include quality control, quality assurance, and the maintenance of records and documentation. The FDA enforces these requirements through our facilities inspection program. If the FDA determines that our manufacturers are not in compliance with FDA laws and regulations, including those governing cGMPs, the FDA may not approve a marketing application until the deficiencies are corrected or until we replace the manufacturer in our application with a manufacturer that is in compliance. Our manufacturers will also be subject to continuing FDA and other regulatory authority inspections should we receive marketing approval for any of our product candidates. Any such deviations from the regulatory requirements of the FDA or other regulatory authorities may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility.

Our active pharmaceutical ingredient, rapamycin, is currently sourced from suppliers located in India. Any disruption in the supply of these ingredients or components or any problems in their quality could materially delay our development programs and affect our ability to manufacture our product candidates and could result in legal liabilities that could materially harm our business, financial, and operating results, or, if marketing approval is received, our ability to commercialize our product candidates.

While we are ultimately responsible for the manufacture of our product candidates, other than through our contractual arrangements, we expect to have limited ability to control our manufacturers' compliance with these regulations and standards. A failure to comply with the applicable regulatory requirements may result in regulatory enforcement actions against our manufacturers or us, including fines and civil and criminal penalties. Depending on the severity of any potential regulatory action, our clinical or commercial supply could be interrupted or limited. Any problems or delays we experience in preparing for commercial-scale manufacturing of a product candidate or component, including manufacturing validation, may result in a delay in FDA approval or commercial launch of the product candidate or may impair our ability to manufacture commercial quantities or such quantities at an acceptable cost, which could

result in the delay, prevention, or impairment of commercialization of our product candidates and could adversely affect our business.

Certain of our supplies, including the pumps we intend to use to dispense our QTORIN rapamycin and QTORIN pitavastatin, if approved, are obtained from a sole source supplier. If we experience an interruption in supply from these suppliers, our business may be harmed.

Certain of our suppliers, including the pumps we intend to use to dispense QTORIN rapamycin or QTORIN pitavastatin, if approved, are obtained from a sole source supplier. For example, we have an agreement with Nemera Le Tréport SAS, or Nemera, for such pumps. If there is an interruption in the supply of these pumps from Nemera or any other supplies due to pricing, timing, availability or other issues, if Nemera or any other sole-source supplier does not successfully carry out our contractual duties, meet expected deadlines or supply these pumps or other supplies in accordance with the terms of our agreement and with applicable federal, state, and foreign regulations, or if there are disagreements between us and Nemera or such other sole source supplier, clinical development, marketing approval or commercial manufacturing of our product candidates, if approved, could be delayed.

If our agreement with Nemera is terminated or if Nemera otherwise ceases to supply the pumps we intend to use to dispense QTORIN rapamycin or QTORIN pitavastatin, if approved, or if any other sole source supplier ceases to supply critical materials, there is no guarantee that we will find an alternative supplier for the necessary packaging materials on terms acceptable, or at all. As a result, we would have to redesign our commercial packaging which would be subject to FDA review. This may cause delays in the commercialization of our product candidates and cause us to incur additional expenses. The qualification process for a new vendor could take months or even years, particularly if we are unable to locate an alternative supplier that has sufficient regulatory qualifications, and any such delay in qualification could materially adversely affect our business, financial condition, results of operations and prospects.

We rely on third parties to conduct aspects of our nonclinical studies and our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be unable to obtain regulatory approval for or commercialize QTORIN rapamycin for the treatment of microcystic LM or any other current or future product candidates.

We do not have the ability to independently conduct nonclinical studies and clinical trials. Although our employees manage the overall conduct of our preclinical studies and clinical trials and we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with applicable regulations and the investigational plan and protocol, we rely on third parties, such as CROs and academic institutions, to conduct aspects of our preclinical studies and clinical trials of QTORIN rapamycin for the treatment of microcystic LM. The third parties with whom we contract for execution of our preclinical studies and clinical trials play a significant role in the conduct of these studies and trials and the subsequent collection and analysis of data. These third parties are not our employees, and we expect CROs, independent clinical investigators and consultants to play a significant role in the conduct of our trials and the subsequent collection and analysis of data. Nevertheless, we are responsible for ensuring that each clinical trial is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the investigators, CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA for product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, clinical trial investigators and clinical trial sites. If we or any of our CROs or clinical trial sites fail to comply with applicable GCP requirements, the data generated in our clinical trials may be deemed unreliable, and the FDA may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that our clinical trials comply with GCPs. Our failure or the failure of third parties on whom we rely on to comply with these regulations may require us to delay, stop and/or repeat clinical trials, which would delay the marketing authorization process.

In addition, except for contractual duties and obligations, we have limited ability to control the amount or timing of resources that our third party CROs, investigators, and institutions devote to our programs. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, otherwise perform in a substandard manner, or terminate their engagements with us, the timelines for our development programs may be extended or delayed or our development activities may be suspended or terminated. If a clinical trial site terminates

for any reason, we may experience the loss of follow-up information on subjects enrolled in such clinical trial unless we are able to transfer those subjects to another qualified clinical trial site, which may be difficult or impossible.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of regulatory approval of our product candidates.

Our third parties may also have relationships with other commercial entities, some of which may compete with us. In some cases, these third parties could terminate their agreements with us without cause.

The execution of non-clinical studies and clinical trials and the subsequent compilation and analysis of the data produced requires coordination among various parties. In order for these functions to be carried out effectively and efficiently, it is imperative that these parties communicate and coordinate with one another. If the third parties conducting aspects of our clinical trials fail to communicate and coordinate with one another, do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our clinical trial protocols GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties, which could be difficult, costly or impossible, and our clinical trials may be extended, delayed or terminated or may need to be repeated, which would have a material adverse effect on our business.

We may rely on third parties to perform many essential services for any products that we commercialize. If these third parties fail to perform as expected or to comply with legal and regulatory requirements, our ability to commercialize our product candidates will be significantly impacted and we may be subject to regulatory sanctions.

We may retain third-party service providers to perform a variety of functions related to the sale and distribution of our product candidates, if approved, key aspects of which will be out of our direct control. These service providers may provide key services related to warehousing and inventory control, distribution, customer service, accounts receivable management, and cash collection. If these third-party service providers fail to comply with applicable laws and regulations, fail to meet expected deadlines, or otherwise do not carry out their contractual duties to us, or encounter physical or natural damage at their facilities, our ability to deliver adequate product to meet commercial demand would be significantly impaired and we may be subject to regulatory enforcement action.

In addition, we may engage third parties to perform various other services for us relating to pharmacovigilance and adverse event reporting, safety database management, fulfillment of requests for medical information regarding our product candidates and related services. If the quality or accuracy of the data maintained by these service providers is insufficient, or these third parties otherwise fail to comply with regulatory requirements, we could be subject to regulatory sanctions.

We may also contract with a third party to calculate and report pricing information mandated by various government programs. If a third party fails to timely report or adjust prices as required, or commits errors in calculating government pricing information from transactional data in our financial records, it could impact our discount and rebate liability, and potentially subject us to regulatory sanctions or False Claims Act lawsuits.

Our future commercial collaborators, as well as our independent contractors, including principal investigators, consultants, suppliers, service providers and other vendors, may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk that our employees and independent contractors, including principal investigators, consultants, suppliers, service providers and other vendors, may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate the laws and regulations of the FDA and other equivalent foreign regulatory authorities, including

those laws that require the reporting of true, complete and accurate information to such foreign regulatory authorities; manufacturing standards; U.S. federal and state healthcare fraud and abuse, data privacy laws and other similar non-U.S. laws; or laws that require the true, complete and accurate reporting of financial information or data. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials, or illegal misappropriation of product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending itself or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, integrity oversight and reporting obligations, possible exclusion from participation in Medicare, Medicaid and other U.S. healthcare programs, imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings and curtailment of our operations, any of which could materially adversely affect our business, financial condition, results of operations and prospects.

We intend to explore strategic collaborations with third parties for the development or commercialization of our product candidates, which collaborations may never materialize or may require that we relinquish rights to and control over the development and commercialization of our product candidates.

An element of our business strategy includes acquiring or in-licensing technologies or product candidates for the treatment of rare skin diseases. As a result, we intend to periodically explore a variety of possible strategic collaborations or licenses in an effort to gain access to additional product candidates, technologies, resources or markets. While we do not have a strategic collaboration in place with respect to QTORIN rapamycin or QTORIN pitavastatin and we intend to independently commercialize these product candidates in the United States, we may selectively seek collaborators to commercialize our products, if approved, outside of the U.S. market.

At this time, we cannot predict what form such strategic collaborations or licenses might take in the future. We are likely to face significant competition in seeking appropriate strategic collaborators, and strategic collaborations and licenses can be complicated and time-consuming to negotiate and document. We may not be able to negotiate strategic collaborations on acceptable terms, or at all. We are unable to predict when, if ever, it will enter into any additional strategic collaborations or licenses because of the numerous risks and uncertainties associated with establishing them. Any delays in entering into new strategic collaborations or licenses related to our product candidates could delay the development and commercialization of our product candidates in certain geographies or for certain indications, which could materially adversely affect our business, financial condition, results of operations and prospects.

Our current collaborators and future collaborations and licenses could subject us to a number of risks, including:

- we may be required to undertake the expenditure of substantial operational, financial and management resources;
- we may be required to issue equity securities that would dilute our stockholders' percentage ownership of our company;
- we may be required to assume substantial actual or contingent liabilities;
- we may not be able to control the amount and timing of resources that our strategic collaborators devote to the development or commercialization of our product candidates;
- we may not have the right to control the preparation, filing, prosecution and maintenance of patents and patent applications covering the technology that we license, and we cannot always be certain that these patents and patent applications will be prepared, filed, prosecuted and maintained in a manner consistent with the best interests of our business;
- collaborators may select indications or design clinical trials in a way that may be less successful than if we were doing so;

- collaborators may delay clinical trials, provide insufficient funding, terminate a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new version of a product candidate for clinical testing;
- collaborators may not commit adequate resources to the marketing and distribution of our product candidates, limiting our potential revenue from these products;
- disputes may arise between us and our collaborators that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management's attention and consumes resources;
- collaborators may experience financial difficulties;
- collaborators could decide to move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and
- collaborators could terminate the arrangement or allow it to expire, which would delay the development and may increase the cost of developing our product candidates.

Furthermore, license agreements we enter into in the future may not provide exclusive rights to use intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses.

Additionally, if any future collaborator of we are involved in a business combination, the collaborator might deemphasize or terminate development or commercialization of any product candidate licensed to it by us. If one of our collaborators terminate our agreement with us, we may find it more difficult to attract new collaborators and our reputation in the business and financial communities could be adversely affected.

Risks Related to Intellectual Property

We may not be able to obtain, maintain or enforce patent rights that cover our product candidates and technologies that are of sufficient breadth to prevent third parties from competing against us.

We rely upon a combination of patents, trade secret protection and CDAs to protect the intellectual property related to our product candidates, proprietary technologies and product candidate development programs. Our success depends in large part on our ability to secure and maintain patent protection in the United States and other countries with respect to our product candidates and proprietary technologies and any future product candidates. We seek to protect our proprietary position by filing or collaborating with our licensors to file patent applications in the United States and abroad related to our proprietary technologies, development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates, and proprietary technology in the United States or in other foreign countries, in whole or in part. Alternately, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from issuing from a pending patent application or later invalidate or narrow the scope of an issued patent. Even if patents do successfully issue, and even if such patents cover our existing product candidates or any future product candidate, third parties may challenge their validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful challenge to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their validity, breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our existing product candidates or any future product candidate, it could dissuade companies from collaborating with us to develop product candidates, encourage competitors to develop competing products or technologies and threaten our ability to commercialize future product candidates. Any such outcome could have a materially adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies is highly uncertain, involves complex legal and factual questions, and is characterized by the existence of large numbers of patents and frequent litigation based on allegations of patent or other intellectual property infringement or violation. In addition, the laws of jurisdictions outside the United States may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. Since patent applications in the United States and other jurisdictions are confidential for a period of time after filing, we cannot be certain that we were the first to file for patents covering our inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in the issuance of patents, or may result in the issuance of patents which fail to protect our technology or products, in whole or in part, or which fail to effectively prevent others from commercializing competitive technologies and products.

The issuance of a patent is not conclusive as to our inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity, patent term or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Thus, even if our patent applications issue as patents, they may not issue in a form that will provide us with meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage.

The patent rights we own covering QTORIN rapamycin and QTORIN pitavastatin are directed to specific formulations of rapamycin and specific methods of use. As a result, our ability to prevent others from marketing products related to QTORIN rapamycin and QTORIN pitavastatin may be limited by the lack of patent protection for the active ingredient itself and other rapamycin and pitavastatin formulations may be developed by competitors. No patent protection is available for rapamycin itself, the active ingredient in QTORIN rapamycin and no patent protection is available for pitavastatin itself, the active ingredient in QTORIN pitavastatin. As a result, competitors who develop and receive required regulatory approval for competing products using the same active ingredient as QTORIN rapamycin and QTORIN pitavastatin may market their competing products so long as they do not infringe any of the method or formulation patents owned by us.

Patent terms may be inadequate to protect our competitive position and if we do not obtain additional patent protection by issuing additional patents with longer patent terms for our product candidates, our business may be materially harmed.

Our commercial success will largely depend on our ability to obtain and maintain patent and other intellectual property in the United States and other countries with respect to our proprietary technology, product candidates and our target indications. The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from our application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. naturally expire as early as 2038. We also own pending applications in the US and other major markets directed to the use of QTORIN rapamycin for the treatment of microcystic LM and angiokeratomas that, if issued, would expire in 2042 and 2046, respectively. Our patent portfolio directed to QTORIN pitavastatin consists of one allowed U.S. application, that upon issuance, will expire in 2040 and one U.S. provisional that, if issued, will expire in 2046. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting our product candidates might expire before or shortly after such candidates begin to be commercialized.

If we are unable to obtain new patents with expiration dates further in the future, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing products following our patent expiration and launch their product earlier than might otherwise be the case.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States or may not be pursued at all outside of the United States. For example, we do not own or license any patent rights directed to QTORIN rapamycin outside of the United States Australia, China, Israel and Japan and we do not own or license any patent rights directed to QTORIN with any other mTOR inhibitors outside of the United States. The patent application that we exclusively license from Yale University relating to the use of HMG CoA reductase inhibitors for treating porokeratosis is only filed in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. In addition, certain jurisdictions do not protect to the same extent or at all inventions that constitute new methods of treatment. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the "America Invents Act", the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before we could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant of the time from invention to filing of a patent application. Since patent applications in the United States and most

other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications. The America Invents Act also includes a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO, during patent prosecution and additional procedures to attack the validity of a patent by USPTO-administered post-grant proceedings, including post-grant review, inter partes review and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business, financial condition, results of operations and prospects.

In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce patents that we own, have licensed or might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions, changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we own or have licensed or that we may obtain in the future.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for noncompliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and other foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign national or international patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of patent rights include, but are not limited to, failure to timely file national and regional stage patent applications based on our international patent application, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering any of our product candidates, our competitors might be able to enter the market earlier than anticipated, which could materially adversely affect our business, financial condition, results of operations and prospects.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we expect to rely on third parties to manufacture our product candidates, and we expect to continue to collaborate with third parties on the development of our current and future product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of collaboration or similar agreements. We seek to protect our proprietary technology in part by entering into CDAs and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others,

or are disclosed or used in violation of these agreements. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or third-party consultants and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity incident or data breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Further, adequate remedies may not exist in the event of unauthorized use or disclosure. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and could materially adversely affect our business, financial condition, results of operations and prospects.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Policing unauthorized use of our or our licensors' intellectual property is difficult, expensive and time-consuming, and we may be unable to determine the extent of any unauthorized use. Moreover, enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.

We may not be able to protect our proprietary information and technology adequately. Although we use reasonable efforts to protect our proprietary information, technology, and know-how, our employees, consultants, contractors, outside scientific advisors, licensors or licensees may unintentionally or willfully disclose our information to competitors. We rely, in part, on non-disclosure and CDAs with our employees, consultants and other parties to protect our proprietary information, technology, and know-how. These agreements may be breached and we may not have adequate remedies for any breach. Moreover, others may independently develop similar or equivalent proprietary information, and third parties may otherwise gain access to our proprietary knowledge.

We may be subject to claims challenging the inventorship or ownership of the patents and other intellectual property that we own or license.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an ownership interest in the patents and intellectual property that we own or license or that we may own or license in the future. While it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own; our licensors may face similar obstacles. We could be subject to ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against any claims challenging inventorship or ownership. If we or our licensors fail in defending any such claims, we may have to pay monetary damages and may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property, which could materially adversely affect our business, financial condition, results of operations and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may become subject to claims alleging infringement of third parties' patents or proprietary rights and/or claims seeking to invalidate our patents, which would be costly, time consuming and, if successfully asserted against us, delay or prevent the development and commercialization of our current product candidates or any future product candidates.

Our commercial success depends in part on us and our licensors avoiding infringement and other violations of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that it infringes or otherwise violates patents or other intellectual property rights owned or

controlled by third parties. There is a substantial amount of litigation, worldwide, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. Numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing product candidates.

We cannot assure that our exploitation of QTORIN rapamycin, QTORIN pitavastatin or any future product candidate will not infringe existing or future third-party patents. Because patent applications can take many years to issue and may be confidential for 18 months or more after filing, there may be applications now pending of which we are unaware and which may later result in issued patents that we may infringe by commercializing any of our product candidates, if approved. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization. Moreover, we may face claims from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may thus have no deterrent effect.

We may be subject to third-party claims in the future against us or our collaborators that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages, including treble damages and attorney's fees if we are found to be willfully infringing a third party's patents. We may be required to indemnify future collaborators against such claims. If a patent infringement suit were brought against us or our future collaborators, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtain a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent was to be held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable product candidate unless we obtain a license or until such patent expires. As a result of patent infringement claims, or in order to avoid potential claims, we or our collaborators may choose to seek, or be required to seek, a license from the third party and would most likely be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we or our future collaborators were able to obtain a license, the rights obtained may be nonexclusive, which would not confer a competitive advantage to us from an exclusivity perspective. Ultimately, we could be prevented from commercializing a product, or forced to redesign it, or to cease some aspect of our business operations if, as a result of actual or threatened patent infringement claims, we or our collaborators are unable to enter into licenses on acceptable terms to necessary third-party patent rights. Even if we are successful in defending against such claims, such litigation can be expensive and time consuming to litigate and would divert management's attention from our core business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Any of these events could materially adversely affect our business, financial condition, results of operations and prospects.

Some of our competitors may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. In addition, intellectual property litigation, regardless of our outcome, may cause negative publicity, adversely impact prospective customers, cause product shipment delays, or prohibit us from manufacturing, marketing or otherwise commercializing our products, services and technology. Any uncertainties resulting from the initiation and continuation of any litigation could adversely impact our ability to raise additional funds or otherwise materially adversely affect our business, financial condition, results of operations and prospects.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments, which could adversely impact the price of our common stock. If securities analysts or investors perceive these results to be negative, it could adversely impact the price of our common stock. The

occurrence of any of these events could materially adversely affect our business, financial condition, results of operations and prospects.

In addition to infringement claims against us, if third parties prepare and file patent applications in the United States that also claim technology similar or identical to ours, we may have to participate in interference or derivation proceedings in the USPTO, to determine which party is entitled to a patent on the disputed invention. We may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in derivation, reexamination, inter partes review, post-grant review or interference proceedings or similar opposition proceedings in the EPO or similar offices in other jurisdictions regarding our intellectual property rights with respect to our products and technology. Since patent applications are confidential for a period of time after filing, we cannot be certain that we were the first to file any patent application related to our product candidates.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our product candidates.

We cannot guarantee that any of us or our licensors' patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is or may be relevant to or necessary for the commercialization of our product candidates in any jurisdiction. Patent applications in the United States and elsewhere are not published until approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. In addition, U.S. patent applications filed before November 29, 2000 and certain U.S. patent applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Therefore, patent applications covering our products could have been filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use of our product candidates.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our product candidates. We may incorrectly determine that our product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that it considers relevant may be incorrect, and our failure to identify and correctly interpret relevant patents, may negatively impact our ability to develop and market our product candidates.

If we fail to identify and correctly interpret relevant patents, we may be subject to infringement claims. we cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay damages, we may be temporarily or permanently prohibited from commercializing any of our product candidates that are held to be infringing. We might, if possible, also be forced to redesign product candidates or services so that we no longer infringe upon the third-party intellectual property rights. Any of these events, even if we was ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

We may be subject to claims by third parties asserting that we, our employees or our licensors have misappropriated their intellectual property, including trade secrets, or claiming ownership of what we regard as our own intellectual property.

Many of our employees and our licensor's employees were previously employed at other biotechnology or pharmaceutical companies. Although we and our licensors try to ensure that our employees and our licensor's employees do not use the intellectual property rights, proprietary information or know-how of others in their work for us, including by contract, we or our licensors may be subject to claims that these employees, our licensors or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form

strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which could materially adversely affect our business, financial condition, results of operations and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may in the future be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensor fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products, which license may not be available on commercially reasonable terms, or at all, or such license may be non-exclusive. Even if we and our licensor are successful in prosecuting or defending against such claims, litigation could result in substantial costs.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property or the patents of our licensors, which could be expensive and time-consuming.

Competitors may infringe, misappropriate or otherwise violate our intellectual property, including our patents or the patents of our licensors. As a result, we may be required to file infringement claims or inform and cooperate with our licensors to stop third-party infringement or unauthorized use. This can be expensive, particularly for a company of our size, and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover our technology or that the factors necessary to grant an injunction against an infringer are not satisfied. An adverse determination of any litigation or other proceedings could put one or more of our patents at risk of being invalidated, interpreted narrowly or amended such that they do not cover our product candidates. Moreover, such adverse determinations could put our patent applications at risk of not issuing, or issuing with limited and potentially inadequate scope to cover our product candidates or to prevent others from marketing similar products.

Interference, derivation or other proceedings brought at the USPTO may be necessary to determine the priority or patentability of inventions with respect to our patent applications or those of our licensors or potential partners. Litigation or USPTO proceedings brought by us may fail or may be invoked against us by third parties. Even if we are successful, domestic or foreign litigation or USPTO or foreign patent office proceedings may result in substantial costs. We may not be able, alone or with our licensors or potential partners, to prevent misappropriation of our proprietary rights, particularly in countries where the laws may not protect such rights as fully as in the United States. The initiation of a claim against a third party may also cause the third party to bring counterclaims against us such as claims asserting that our patents are invalid or unenforceable.

In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement, lack of sufficient written description, obviousness-type double patenting or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable and could result in the revocation, cancelation, amendment or shortening of term of patents we own or license. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, We would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business. We

may find it impractical or undesirable to enforce our intellectual property against some third parties. For instance, we are aware of third parties that have marketed topical rapamycin formulations for the treatment of skin diseases over the internet or through compound pharmacies. These parties do not appear to have regulatory approval, and we have not authorized them in any way to market these products. However, to date we have refrained from seeking to enforce our intellectual property rights against these third parties due to the transient nature of their activities.

We may not be able to detect or prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other proceedings, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings. In addition, during the course of this kind of litigation or proceedings, there could be public announcements of the results of hearings, motions or other interim proceedings or developments or public access to related documents. If investors perceive these results to be negative, the market price for our common stock could be significantly harmed.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities, and have a harmful effect on the success of our business.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. In addition, the uncertainties associated with litigation could compromise our ability to compete in the marketplace or to raise the funds necessary to continue our clinical trials and internal research programs, or in-license needed technology or other product candidates. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property.

We may need to license additional intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

Additional third parties, apart from our current licensors, may hold intellectual property, including patent rights, that are important or necessary to the development of our product candidates. It may be necessary for us to use the patented or proprietary technology of these third parties to commercialize our product candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms. Such a license may not be available, or it may not be available on commercially reasonable terms, in which case our business would be harmed. The risks described elsewhere pertaining to our intellectual property rights also apply to the intellectual property rights that our in-licenses, and any failure by us or our licensors to obtain, maintain, defend and enforce these rights could harm our business. In some cases, we may not have control over the prosecution, maintenance or enforcement of the patents that we license, and may not have sufficient ability to provide input into the patent prosecution, maintenance and defense process with respect to such patents, and our licensors may fail to take the steps that we believe are necessary or desirable in order to obtain, maintain, defend and enforce the licensed patents.

Our intellectual property agreements with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

Certain provisions in our intellectual property agreements may be susceptible to multiple interpretations. Disputes may arise between us and any of these counterparties regarding intellectual property rights that are subject to such agreements, including, but not limited to:

- the scope of rights granted under the agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the agreement;
- our right to sublicense patent and other rights to third parties;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners;
- our right to transfer or assign our license;
- the effects of termination; and
- the priority of invention of patented technology.

The resolution of any contract interpretation disagreement that may arise could affect the scope of our rights to the relevant intellectual property or technology, or affect financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary or useful for developing and protecting our product candidates.

We have licensed, or may in the future license, certain intellectual property rights relating to our technology and indications of interest from third parties. If we materially breaches or fails to perform any provision under these license agreements, including failure to make payments to a licensor when due for royalties or milestones and failure to use commercially reasonable efforts to develop and commercialize the licensed technology, such licensors may have the right to terminate our license agreement. If, for any reason, one or more of our agreements with such third parties is terminated or we otherwise loses those rights, it could harm our business. Our license and other agreements impose, and any future collaboration agreements or license agreements we enter into are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement or other obligations on us. If we breach any such material obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license or otherwise acquire intellectual property rights from us, which could result in us being unable to develop, manufacture and sell products that are covered by the licensed technology, or having to negotiate new or reinstated licenses on less favorable terms, or enable a competitor to gain access to the licensed technology.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented, declared generic or conflict with third-party rights. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition by potential partners or customers in our markets of interest. In addition, third parties may file first for our trademarks in certain countries. If they succeeded in registering such trademarks, and if we were not successful in challenging such third-party rights, we may not be able to use these trademarks to market our products in those countries. In such cases, over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then our marketing abilities may be impacted.

We have not yet registered trademarks for a commercial trade name for our lead candidates in the United States or foreign jurisdictions and failure to secure such registrations could adversely affect our business.

We have not yet registered trademarks for a commercial trade name for our lead product candidates in the United States or any foreign jurisdiction. During trademark registration proceedings, we may receive rejections. Although we

are given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancelation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we proposes to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

Furthermore, QTORIN is our proprietary name for our technology platform. Any future commercial tradename for our lead product candidates will be subject to approval by the FDA for commercial use and will not include the QTORIN mark. Accordingly, any goodwill and recognition that we have built for the name in relation to future commercial drug products may be lost.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our product candidates, but that are not covered by the claims of the patents or other intellectual property rights that we own that we have exclusively licensed and has the right to enforce;
- we, our licensors or any collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or license;
- we or our licensors might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or licenses may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the U.S. may, under certain circumstances, force us or our licensors to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates;
- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;

- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we cannot ensure that our commercial activities or product candidates will not infringe upon the patents of others;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- we may not develop additional proprietary technologies that are patentable;
- we may not have sufficient time remaining on the term of our patents or the term of our marketing exclusivity to warrant commercialization of our product candidates;
- the patents or intellectual property rights of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Risks Related to Ownership of Our Common Stock

The market price of our common stock has been and is likely to continue to be volatile and fluctuate substantially.

The trading price of our common stock has been and is likely to continue to be highly volatile. Furthermore, the stock market in general and the market for biopharmaceutical and pharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares at or above the price they paid for their shares. The market price of our common stock may be influenced by many factors, including:

- results of clinical trials and preclinical studies of our product candidates, or those of our competitors or existing or future collaborators;
- failure to meet or exceed financial and development projections we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions or market conditions in the pharmaceutical and biotechnology sectors;

- sales of securities by us or other securityholders in the future;
- if we fail to raise an adequate amount of capital to fund our operations or continued development of our product candidates;
- trading volume of our common stock;
- announcements by competitors of new commercial products, clinical progress or lack thereof, significant contracts, commercial relationships or capital commitments;
- adverse publicity relating to precision medicine product candidates, including with respect to other products in such markets;
- the introduction of technological innovations or new therapies that compete or could compete with our product candidates;
- period-to-period fluctuations in our financial results; and
- the other factors described in this "*Risk Factors*" section.

Some companies that have experienced volatility in the trading price of their shares have been the subject of securities class action litigation. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our business practices. Defending against litigation is costly and time-consuming, and could divert our management's attention and our resources. Furthermore, during the course of litigation, there could be negative public announcements of the results of hearings, motions or other interim proceedings or developments, which could have a negative effect on the market price of our common stock.

Our executive officers, directors and principal stockholders have the ability to control or significantly influence all matters submitted to our stockholders for approval.

Our executive officers, directors and principal stockholders, in the aggregate, beneficially own a significant portion of our outstanding shares of common stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence any matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect to not provide research coverage of our common stock and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts, or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

We have broad discretion in the use of our cash and cash equivalents and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We have broad discretion over the use of our cash and cash equivalents. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. Our failure to apply these resources effectively

could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to our cash resources.

Because we do not anticipate paying any cash dividends on our share capital in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

You should not rely on an investment in our shares to provide dividend income. We have never declared or paid cash dividends on our share capital. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements or preferred equity may preclude us from paying dividends. As a result, capital appreciation, if any, of our common shares will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our shares.

Provisions in our charter and bylaws, as well as provisions of Nevada law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Our charter and bylaws and Nevada law contain provisions that may have the effect of delaying or preventing a change in control of the Company or changes in our management. Our charter and bylaws, include provisions that:

- authorize "blank check" preferred stock, which could be issued by the Board without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- advance notice for nominations of directors by stockholders and for stockholders to include matters to be considered at stockholder meetings;
- prohibit stockholder action by written consent;
- certain limitations on convening special stockholder meetings and the prohibition of stockholder action by written consent; and
- directors may only be removed for cause and only by the affirmative vote of the holders of at least 80% of the voting power of all of the then-outstanding shares of our capital stock entitled to vote at an election of directors, voting together as a single class.

The NRS contains provisions governing the acquisition of a controlling interest in certain Nevada corporations. Nevada's "acquisition of controlling interest" statutes (NRS 78.378 through 78.3793, inclusive) contain provisions governing the acquisition of a controlling interest in certain Nevada corporations. These "control share" laws provide generally that any person that acquires a "controlling interest" in certain Nevada corporations may be denied voting rights, unless a majority of the disinterested stockholders of the corporation elect to restore such voting rights. These laws would apply to us as of a particular date if we were to have 200 or more stockholders of record (at least 100 of whom have addresses in Nevada appearing on our stock ledger at all times during the 90 days immediately preceding that date) and do business in the State of Nevada directly or through an affiliated corporation, unless our Articles of Incorporation or Bylaws in effect on the tenth day after the acquisition of a controlling interest provide otherwise. These laws provide that a person acquires a "controlling interest" whenever a person acquires shares of a subject corporation that, but for the application of these provisions of the NRS, would enable that person to exercise (1) one-fifth or more, but less than one-third, (2) one-third or more, but less than a majority, or (3) a majority or more of all of the voting power of the corporation in the election of directors. Once an acquirer crosses one of these thresholds, shares which it acquired in the transaction taking it over the threshold and within the 90 days immediately preceding the date when the acquiring person acquired or offered to acquire a controlling interest become "control shares" to which the voting restrictions described above apply. Our Articles of Incorporation include a provision stating that these laws, or any successor statutes, relating to acquisitions of controlling interests in Palvella, shall not apply to us or to any acquisition of any shares of our capital stock. These laws may have a chilling effect on certain transactions if our Articles of Incorporation are amended to eliminate the foregoing provision and these laws otherwise apply according to their terms.

Nevada's "combinations with interested stockholders" statutes (NRS 78.411 through 78.444, inclusive) provide that specified types of business "combinations" between certain Nevada corporations and any person deemed to be an "interested stockholder" of the corporation are prohibited for two years after such person first becomes an "interested stockholder" unless the corporation's board of directors approves the combination (or the transaction by which such person becomes an "interested stockholder") in advance, or unless the combination is approved by the board of directors and 60% of the corporation's voting power not beneficially owned by the interested stockholder, its affiliates and associates. Furthermore, in the absence of prior approval certain restrictions may apply even after such two-year period. For purposes of these statutes, an "interested stockholder" is any person who is (1) the beneficial owner, directly or indirectly, of 10% or more of the voting power of the outstanding voting shares of the corporation, or (2) an affiliate or associate of the corporation and at any time within the two previous years was the beneficial owner, directly or indirectly, of 10% or more of the voting power of the then-outstanding shares of the corporation. The definition of the term "combination" is sufficiently broad to cover most significant transactions between a corporation and an "interested stockholder". These laws generally apply to Nevada corporations with 200 or more stockholders of record. However, a Nevada corporation may elect in its articles of incorporation not to be governed by these particular laws, but if such election is not made in the corporation's original articles of incorporation, the amendment (1) must be approved by the affirmative vote of the holders of stock representing a majority of the outstanding voting power of the corporation not beneficially owned by interested stockholders or their affiliates and associates, and (2) is not effective until 18 months after the vote approving the amendment and does not apply to any combination with a person who first became an interested stockholder on or before the effective date of the amendment. We have not made such an election in our original articles of incorporation, and we have not amended our Articles of Incorporation to so elect.

Further, NRS 78.139 provides that directors may resist a change or potential change in control of the corporation if the board of directors determines that the change or potential change is opposed to or not in the best interest of the corporation upon consideration of any relevant facts, circumstances, contingencies or constituencies pursuant to NRS 78.138(4).

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

Any provision of our charter, bylaws or Nevada law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our articles of incorporation contain exclusive forum provisions, which may limit a stockholder's ability to bring a claim in a judicial forum such stockholder finds favorable and may discourage lawsuits with respect to such claims.

Our amended and restated articles of incorporation provide that, to the fullest extent permitted by law, and unless we consent to the selection of an alternative forum, The Eighth Judicial District Court of Clark County, Nevada shall be the sole and exclusive forum for any (i) derivative action or proceeding brought in the name or right of the corporation or on our behalf, (ii) action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to the corporation or any of our stockholders, (iii) any action arising or asserting a claim arising pursuant to any provision of Chapters 78 or 92A of the NRS or any provision of its amended and restated articles of incorporation or amended and restated bylaws, (iv) any action to interpret, apply, enforce or determine the validity of its articles of incorporation or bylaws or (v) any action asserting a claim governed by the internal affairs doctrine. Our amended and restated articles of incorporation further provide that any person purchasing or otherwise acquiring any interest in shares of its capital stock shall be deemed, to the fullest extent permitted by law, to have notice of and consented to the foregoing provision.

Choice-of-forum provisions of the type and scope included in our amended and restated articles of incorporation are expressly permitted by Section 78.046 of the NRS, but application of these choice-of-forum provisions may be limited in some instances by law. Section 27 of the Exchange Act establishes exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder and therefore the choice-of-forum provision would not apply to actions arising under, or brought to enforce a duty or liability created by, the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction. We note that the choice-of-forum provision does not relieve us of our duty to comply with the federal securities laws

and the rules and regulations thereunder, and our stockholders will not be deemed to have waived compliance with these laws, rules and regulations.

We believe the choice-of-forum provision in our amended and restated articles of incorporation will help provide for the orderly, efficient and cost-effective resolution of the types of legal issues affecting us, as identified in the choice-of-forum provision, by designating courts located in the State of Nevada (our state of incorporation) as the exclusive forum for cases involving such issues. However, this provision may limit a stockholder's ability to bring a claim in a judicial forum that such stockholder believes to be favorable for disputes with us or our directors, officers, employees or agents, which may discourage such actions against us and our directors, officers, employees and agents, and could also increase the costs of stockholders in connection with bringing a claim and resolving such matters. If a court were to find the choice-of-forum provision in our amended and restated articles of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could adversely affect our business, financial condition or results of operations.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity.

Cybersecurity Risk Management and Strategy

In recognition of the evolving cybersecurity threat landscape, we acknowledge the increasing sophistication and frequency of cybersecurity incidents. While we cannot completely protect against the possibility of a cybersecurity incident occurring, we take measures designed to mitigate risks from cybersecurity threats, including those implemented by our third-party managed services provider.

As part of our cybersecurity procedures, we leverage a number of security controls, including network and device monitoring and system backup procedures. We work to mitigate risks from cybersecurity threats stemming from third-party vendors by providing them with access only to systems that they need to provide services to us.

We have not identified any cybersecurity incidents or threats that have materially affected us or are reasonably likely to materially affect us, including our business strategy, results of operations, or financial condition. However, like other companies in our industry, we and our third-party vendors have from time to time experienced threats that could affect our information or systems. For more information, please see "Item 1A, Risk Factors."

Cybersecurity Governance

Senior management, including the Chief Executive Officer and Chief Financial Officer, are responsible for implementation of the Company's risk management controls, including controls in connection with risks from cybersecurity threats.

The Audit Committee of our Board of Directors is primarily responsible for overseeing the Company's compliance and risk management obligations, including the management of risks from cybersecurity threats. Pursuant to its charter, the Audit Committee is responsible for monitoring the effectiveness of the Company's information system and cybersecurity controls.

On a quarterly basis, the Audit Committee discusses with senior management, and internal audit, if applicable, the Company's processes for assessing, identifying, and managing material risks from cybersecurity threats and the state of the Company's cybersecurity processes. The Audit Committee also receives updates on, and monitors, the Company's prevention, detection, mitigation and remediation of cybersecurity incidents.

Item 2. Properties.

Our principal executive office is located in Wayne, Pennsylvania, where we lease 6,853 square feet of space that we use for our administrative, research and development and other activities. We believe that our existing facilities are

adequate for our near-term needs but expect to need additional space as we grow. We believe that suitable additional or alternative space would be available as required in the future on commercially reasonable terms.

Item 3. Legal Proceedings.

From time to time, we may become involved in legal proceedings or be subject to claims arising in the ordinary course of our business. We are not currently subject to any material legal proceedings.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock is currently listed on the Nasdaq Capital Market under the symbol "PVLA." Prior to the consummation of the Merger, our common stock was listed on the Nasdaq Capital Market under the symbol "PIRS."

As of March 25, 2026, there were approximately 52 stockholders of record of our common stock. This number does not include beneficial owners whose shares are held by brokers or other nominees in street name. Because many of our shares are held by brokers and other institutions on behalf of stockholders, we are unable to estimate the total number of stockholders represented by these record holders.

Dividends

We have never declared or paid cash dividends on our capital stock. We intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Notwithstanding the foregoing, any determination to pay cash dividends will be at the discretion of our board of directors and will depend upon a number of factors, including our results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors our board of directors deems relevant.

Recent Sales of Unregistered Securities

We did not issue any equity securities during the year ended December 31, 2025 that were not registered under the Securities Act and that have not otherwise been described in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

Issuer Purchases of Equity Securities

We did not repurchase any of our equity securities during the fiscal year ended December 31, 2025.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read together with our consolidated audited financial statements and accompanying notes appearing elsewhere in this Form 10-K. In addition to historical information, this discussion and analysis includes forward-looking statements that are subject to risks and uncertainties, including those discussed in the section titled "Risk Factors," set forth in Part I, Item 1A of this Form 10-K, that could cause actual results to differ materially from historical results or anticipated results.

Unless otherwise indicated or the context otherwise requires, references in this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section to "the Company," "we," "us," and "our" refer to the business and operations of Palvella Therapeutics, Inc., a Delaware corporation (referred to as "Legacy Palvella") prior to the Merger, and the business and operations of Palvella Therapeutics, Inc., a Nevada Corporation (previously Pieris Pharmaceuticals, Inc., referred to as "Pieris") and its consolidated subsidiaries following the Merger.

Overview

We are a clinical-stage biopharmaceutical company whose vision is to become the leading rare disease biopharmaceutical company focused on developing and, if approved, commercializing novel therapies to treat patients suffering from serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies. We envision a future treatment paradigm in which individuals suffering from serious, rare skin diseases and vascular malformations, and the physicians treating those diseases, have significantly improved treatment options which address the underlying causes of those diseases. We intend to leverage our versatile QTORIN platform to minimize the challenges and timelines typically associated with generating novel topical product candidates. The QTORIN platform is specifically designed to reproducibly generate novel topical product candidates that penetrate the deep layers of the skin to locally treat a broad spectrum of rare skin diseases and vascular malformations. Our lead product candidate, QTORIN 3.9% rapamycin anhydrous gel ("QTORIN rapamycin"), is currently in clinical development for microcystic lymphatic malformations ("microcystic LMs") and cutaneous venous malformations ("cutaneous VMs"). QTORIN rapamycin contains the active pharmaceutical ingredient ("API") rapamycin, also known as sirolimus, which is an inhibitor of mTOR, a kinase that has been known to play a key role in cell growth and proliferation.

In February 2026, we announced positive topline results from SELVA, a Phase 3, single-arm, baseline-controlled study, which evaluated the safety and efficacy of QTORIN rapamycin for the treatment of microcystic LMs in patients 3 years and older. The study met the pre-specified primary endpoint, the mLM Investigator Global Assessment ("mLM-IGA"), with a +2.13 ($p<0.001$) improvement. The study also met its pre-specified key secondary and all four additional secondary endpoints with statistical significance (all $p<0.001$). In December 2025, we announced positive topline efficacy results from TOIVA, a Phase 2, single-arm, baseline-controlled study, which evaluated the safety and efficacy of QTORIN rapamycin for the treatment of cutaneous VMs in patients 6 years and older, which achieved nominal statistical significance ($p<0.001$) on multiple pre-specified clinician-reported and patient-reported efficacy endpoints, including dynamic change endpoints and static severity endpoints at Week 12.

In September 2025, we announced the expansion of our QTORIN rapamycin development program into clinically significant angiokeratomas.

In November 2025, we announced a new QTORIN product candidate, QTORIN pitavastatin, for the treatment of disseminated superficial actinic porokeratosis ("DSAP"). QTORIN pitavastatin leverages our proprietary QTORIN platform and is designed to be the first pathogenesis-directed therapy for DSAP by directly inhibiting the causal mevalonate pathway.

Our Novel Product Candidate: QTORIN rapamycin

Overview

We are developing QTORIN rapamycin, a novel, 3.9% anhydrous topical gel formulation containing rapamycin, for the treatment of microcystic LMs, cutaneous VMs, clinically significant angiokeratomas and other mTOR-driven skin diseases. If approved, we believe QTORIN rapamycin has the potential to become the standard of care in each of these diseases.

QTORIN rapamycin for the treatment of microcystic LMs

Microcystic LM is a serious, chronically debilitating, and lifelong genetic disease of the lymphatic system characterized by lymphorrhea and acute cellulitis. It is estimated that there are more than 30,000 diagnosed patients in the United States with microcystic LMs. The specific pathophysiology of microcystic LMs is primarily the result of somatic activating mutations in PIK3CA that result in increased activation of the PI3K/mTOR pathway and subsequent lymphatic hyperplasia. Because microcystic LMs have a well-understood pathophysiology and a well-defined disease course, we believe an appropriate clinical study for this rare disease is a baseline-controlled Phase 3 study using clinician assessments.

We recently completed SELVA, a Phase 3, single-arm, baseline-controlled clinical trial evaluating once-daily QTORIN rapamycin in individuals aged ≥ 3 years with microcystic LMs and announced positive topline results. Of the 51 participants enrolled, 50 initiated treatment, including 49 participants aged ≥ 6 years and 1 participant in the exploratory 3- to 5-year-old cohort. In accordance with the statistical analysis plan, efficacy results were reported for participants aged ≥ 6 years, which constituted the Intent-to-Treat ("ITT") population. The study was originally designed to enroll 40 participants across leading U.S. vascular anomaly centers and exceeded its target enrollment.

The primary endpoint, the mLM-IGA, is a 7-point clinician-assessed dynamic scale measuring change in disease severity from baseline ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the mLM-IGA in the ITT population (n=49), QTORIN rapamycin demonstrated a mean improvement of +2.13 points, meeting the study's primary endpoint ($p<0.001$). Of the participants aged ≥ 6 who completed the efficacy evaluation period, 95% (41/43) demonstrated at least a 1-point improvement, and 86% (37/43) were either "Much Improved" (+2) or "Very Much Improved" (+3). In the 3- to 5-year-old cohort, one participant enrolled and was "Very Much Improved" (+3) on the mLM-IGA at Week 24.

Similar to previous clinical trials of QTORIN rapamycin, in the Phase 3 SELVA study, QTORIN rapamycin was well-tolerated. Amongst the 50 participants who initiated treatment, 35 participants (70%) experienced treatment-emergent adverse events ("TEAEs"). Four experienced serious adverse events, of which one experienced a severe TEAE; all were deemed unrelated to study drug by investigators. Amongst the TEAEs, a total of 17 participants experienced treatment-related adverse events ("TRAEs"), all of which were rated mild or moderate. The most common TRAEs included application site acne, application site discoloration, and application site pruritus (all n=3, 6%). Rapamycin levels were below 2 ng/mL in systemic circulation for all participants at all timepoints in the study.

We previously announced topline Phase 2 clinical trial results from our multi-center, open-label, baseline-controlled study of 12 subjects receiving QTORIN rapamycin administered once daily for 12 weeks for the treatment of microcystic LMs. The Phase 2 clinical trial featured multiple pre-specified efficacy assessments, including clinician and patient global impression assessments as well as assessments of individual clinical manifestations that are important disease burdens for individuals living with microcystic LMs. All participants in the Phase 2 clinical trial demonstrated improvements on the Clinician Global Impression of Change scale, a 7-point clinician-rated change scale, with all participants in the study rated as either "Much Improved" (n=7, 58%) or "Very Much Improved" (n=5, 42%) after 12-weeks of treatment compared to the pre-treatment baseline period.

In the first quarter of 2026, we submitted a pre-NDA meeting request to the FDA. We anticipate the meeting to occur during the second quarter of 2026. We have received Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation from the FDA for QTORIN rapamycin for the treatment of microcystic LMs. Orphan Drug Designation has also been granted by the European Medicines Agency. In addition, we have been awarded an FDA Products Clinical Trials Grant for up to $2.6 million supporting the SELVA Phase 3 study. In May 2025, we received initial proceeds of $0.5 million from the FDA under such grant and received additional proceeds of $0.6 million in October 2025.

QTORIN rapamycin for the treatment of cutaneous VMs

Cutaneous venous malformation is a serious disease with a high unmet need characterized by dysregulated growth of malformed veins impacting the skin, causing functional impairment and deformity. It is estimated that there are more than 75,000 diagnosed patients in the United States with cutaneous VMs.

In December 2025, we announced positive topline efficacy results from TOIVA, a multicenter, single-arm,

open-label, baseline-controlled, Phase 2 clinical trial designed to evaluate the safety and efficacy of QTORIN rapamycin for the treatment of cutaneous VMs. The study enrolled 16 participants, ages six and older, at leading vascular anomaly centers across the U.S. Key findings from among the study's pre-specified efficacy endpoints at Week 12 demonstrated nominally statistically significant ($p<0.001$) improvements at Week 12 on several of the clinically relevant and important efficacy endpoints evaluated when compared to pre-treatment (baseline), including many of the static and impression of change global instruments evaluated, including the Overall Cutaneous VM Investigator Global Assessment ("Overall cVM-IGA") (Table 5). The Overall cVM-IGA is a 7-point, clinician-assessed, single-item efficacy endpoint measuring change in severity from baseline, with the numeric rating scale ranging from "Very Much Worse" (-3) to "Very Much Improved" (+3). On the Overall cVM-IGA at Week 12, 73% (11/15) participants improved, with 67% (10/15) either "Much Improved" (+2) or "Very Much Improved" (+3). No trial participants (0/15) were "Minimally Worse" (-1), "Much Worse" (-2), or "Very Much Worse" (-3).

Similar to previous clinical trials of QTORIN rapamycin, in the Phase 2 TOIVA study QTORIN rapamycin was generally well-tolerated, with the most common treatment-emergent adverse events being application site reactions (erythema, 25%). All treatment-related adverse events were moderate or mild, with no unexpected adverse events reported. Rapamycin levels were below 2 ng/mL in systemic circulation for all participants at all timepoints in the study.

In January 2026, we completed a Preliminary Breakthrough Therapy Designation Advice meeting with the FDA. Based on that meeting, our intent is to submit an application to the FDA for Breakthrough Therapy Designation in the second quarter of 2026. We plan to commence a Phase 3 pivotal study in the second half of 2026.

We have received Fast Track Designation from the FDA for our cutaneous VMs program.

QTORIN rapamycin for the treatment of Clinically Significant Angiokeratomas

In September 2025, we announced the expansion of our QTORIN rapamycin development program into clinically significant angiokeratomas. No FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.

Clinically significant angiokeratomas are superficial vascular malformations of lymphatic origin which can cause bleeding, pain, functional impairment, and risk of infection, with no tendency for spontaneous regression. Angiokeratomas were recently classified as an isolated lymphatic malformation in 2025 by the International Society for the Study of Vascular Anomalies ("ISSVA"). Current treatment options include potentially destructive procedural interventions that carry significant risks of pain, scarring, and recurrence. Despite the substantial disease burden, there are currently no FDA-approved treatments available for clinically significant angiokeratomas.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study of approximately 10-20 patients to evaluate QTORIN rapamycin for the treatment of clinically significant angiokeratomas. Study initiation is anticipated in the second quarter of 2026.

Fast Track Designation from the FDA has been granted for our angiokeratomas program.

QTORIN pitavastatin for the treatment of Disseminated Superficial Actinic Porokeratosis

In November 2025, we announced a new product candidate, QTORIN pitavastatin, for the treatment of disseminated superficial actinic porokeratosis (DSAP). QTORIN pitavastatin was developed leveraging our QTORIN platform.

DSAP is a premalignant genetic skin disease that presents as persistent, often extensive lesions that enlarge and increase in size, number, and extent over time, causing chronic loss of skin integrity which can severely impact quality-of-life; no FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.

We received written feedback from the FDA in the first quarter of 2026 on the proposed design of a Phase 2 study to evaluate QTORIN pitavastatin for the treatment of DSAP. Trial initiation is anticipated in the second half of 2026.

The Business Combination

On December 13, 2024 (the "Closing Date"), we consummated the previously announced business combination contemplated by that certain Agreement and Plan of Merger, dated July 23, 2024 (the "Merger Agreement"), by and among the Company, Polo Merger Sub, Inc. ("Merger Sub"), and Palvella Therapeutics, Inc. ("Legacy Palvella"). Pursuant to the Merger Agreement, on the Closing Date, (i) Merger Sub merged with and into Legacy Palvella, with Legacy Palvella as the surviving company in the merger and, after giving effect to such merger, continuing as a wholly owned subsidiary of the Company (the "Merger" and, together with the other transactions contemplated by the Merger Agreement, the "Business Combination") and (ii) the Company's name was changed from Pieris Pharmaceuticals, Inc. to Palvella Therapeutics, Inc.

The Business Combination was accounted for as a reverse recapitalization in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"). Under this method of accounting, Pieris was treated as the "acquired" company and Legacy Palvella is treated as the acquirer for financial reporting purposes as more fully explained in Note 1 and Note 3 of the accompanying notes to the consolidated financial statements contained elsewhere in this Annual Report on Form 10-K.

PIPE Financing

Concurrently with the execution of the Merger Agreement on July 23, 2024, Pieris entered into a securities purchase agreement (the "Purchase Agreement") with certain investors, including BVF Partners, L.P., an existing stockholder of Pieris (the "PIPE Investors"), pursuant to which, among other things, on the Closing Date and immediately following the consummation of the Merger, the PIPE Investors purchased (either for cash or in exchange for the termination and cancellation of outstanding convertible promissory notes issued by Legacy Palvella), and the Company issued and sold to the PIPE Investors, (i) 3,168,048 shares of common stock and (ii) Pre-Funded Warrants, exercisable for 2,466,456 shares of common stock, at a purchase price of $13.9965 per share or $13.9955 per Pre-Funded Warrant, which represents the per share purchase price of the common stock less the $0.001 per share exercise price for each Pre-Funded Warrant, for an aggregate purchase price of approximately $78.9 million, consisting of approximately $60.0 million in cash and the conversion of approximately $18.9 million of principal and interest under outstanding convertible notes issued by Legacy Palvella (the "PIPE Financing").

Contingent Value Rights Agreement

On December 13, 2024, immediately prior to closing of the Merger, we entered into a Contingent Value Rights Agreement (the "CVR Agreement") with a rights agent, pursuant to which our pre-Merger capital stockholders received one contingent value right (each, a "CVR") for each outstanding share of our Common Stock held by such stockholder, or share of Common Stock underlying preferred stock held by such stockholder, on such date. Each CVR represents the contractual right to receive payments upon the receipt of payments by us or any of its affiliates under certain strategic partner agreements, including existing collaboration agreements pursuant to which we may be entitled to milestones and royalties in the future and other out-licensing agreements for certain of Pieris' legacy assets, and upon the receipt of certain research and development tax credits in favor of us or any of its affiliates, in each case as set forth in, and subject to and in accordance with the terms and conditions of, the CVR Agreement. In January 2026, the Company paid out approximately $2.0 million to holders of CVRs related to receipt of certain research and development tax credits.

Convertible Note Financing

Between June and July 2024, Legacy Palvella issued convertible notes in the aggregate principal amount of approximately $18.4 million (the "Convertible Notes"). Simple interest accrued on the outstanding principal amount of the Convertible Notes at an annual rate of SOFR plus 2.0% per annum. Unless earlier converted, the maturity date was the earliest to occur of (i) the date that Legacy Palvella received approval of an NDA by the FDA of the QTORIN rapamycin in the United States, or (ii) the date that is June 3, 2027. Upon the closing of the PIPE Financing, the entire outstanding principal amount and unpaid accrued interest on the convertible notes automatically converted into an aggregate of 1,179,163 shares of common stock and 168,503 prefunded warrants. See Note 7 of the accompanying notes to the consolidated financial statements contained elsewhere in this Annual Report on Form 10-K.

Ligand Development Funding and Royalties Agreement

We are party to a Development Funding and Royalties Agreement with Ligand Pharmaceuticals, Inc. ("Ligand"), dated December 13, 2018, as amended May 22, 2020 and November 28, 2023 (the "Ligand Agreement"). Under the Ligand Agreement, Ligand has made payments totaling $15.0 million to fund the development of QTORIN rapamycin. As partial consideration for the funding received, we granted Ligand the right to receive up to $8.0 million in milestone payments upon achievement of certain corporate, financing and regulatory milestones by us related to QTORIN rapamycin for the treatment of any and all indications, of which $5.0 million of potential future milestone payments remain under the arrangement. In addition, we agreed to pay to Ligand tiered royalties ranging from 8.0% to 9.8% of any aggregate annual worldwide net product sales of any products based on QTORIN rapamycin. See Note 5 of the accompanying notes to the consolidated financial statements contained elsewhere in this Annual Report on Form 10-K.

Impact of Global Economic Events

Uncertainty in the global economy presents significant risks to our business. We are subject to continuing risks and uncertainties in connection with the current macroeconomic environment, including increases in inflation and geopolitical factors, including increases in inflation, increased U.S. trade tariffs and retaliatory tariffs, interest rate and currency rate fluctuations, new laws and regulations enacted by the Trump administration, including, but not limited to, the One Big Beautiful Bill Act, economic slowdown or recession, banking instability, monetary policy changes, and geopolitical factors, including the ongoing conflict between Russia and Ukraine, the current conflicts in Venezuela and the Middle East (including any escalation or expansion) and increasing tensions between China and Taiwan, rapid changes in our regulatory landscape in the United States, including significant staffing reductions and unexpected shifts in leadership of certain federal agencies, and an uncertain legislative environment and supply chain disruptions. While our management is closely monitoring the impact of the current macroeconomic conditions on all aspects of our business, including the impacts on its participants in its clinical trials, employees, suppliers, vendors and business partners, the ultimate extent of the impact on our business remains highly uncertain and will depend on future developments and factors that continue to evolve. Most of these developments and factors are outside our control and could exist for an extended period of time. Management will continue to evaluate the nature and extent of the potential impacts to our business, results of operations, liquidity and capital resources. For additional information, see Part I, Item 1A "Risk Factors."

Components of Operating Results

Operating Expenses

Our operating expenses since inception have consisted primarily of research and development expenses and general and administrative costs.

We expect to continue to incur significant operating losses for the foreseeable future and to incur increased expenses as we continue to advance our product candidates through clinical trials and regulatory submissions. We may also incur expenses in connection with the in-licensing or acquisition of additional product candidates. Furthermore, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that Legacy Palvella did not incur as a private company. If we receive regulatory approval for QTORIN rapamycin for treatment of microcystic LM, cutaneous VM, clinically significant angiokeratomas, QTORIN pitavastatin for the treatment of disseminated superficial actinic porokeratosis or any future product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Our losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

Research and Development Expenses

Our research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- costs related to production of preclinical and clinical materials, including CMC fees paid to CMOs;
- personnel costs, including salaries, related benefits and stock-based compensation expense for employees engaged in research and development functions;

- vendor expenses related to the execution of preclinical studies and clinical trials;
- expenses incurred under agreements with consultants that conduct research and development activities on our behalf;
- costs related to compliance with regulatory requirements; and
- allocated overhead, including rent, equipment and information technology costs.

We expense all research and development expenses in the periods in which they are incurred. Costs for certain research and development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and other service providers. This process involves reviewing open contracts, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. Any nonrefundable advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are expensed as the related goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

Our indirect research and development expenses are not currently tracked on a program-by-program basis. We use our personnel and infrastructure resources across multiple research and development programs to identify and develop product candidates.

Research and development activities account for a significant portion of our operating expenses. We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in advancing our programs and conducting clinical trials. In particular, we expect to incur substantial research and development expenses to continue late-stage clinical development and pursue regulatory approvals of QTORIN rapamycin for the treatment of microcystic LM venous malformations and the development of our preclinical programs. Product candidates in later stages of clinical development generally incur higher development costs than those in earlier stages, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect our research and development expenses to increase as our product candidates advance into later stages of clinical development.

Because of the numerous risks and uncertainties associated with product development and the current stage of development of our product candidates and programs, we cannot reasonably estimate or know the nature, timing and estimated costs necessary to complete the remainder of the development of our product candidates or programs. The duration, costs and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- timely completion of our preclinical studies and clinical trials, which may be significantly slower or cost more than we currently anticipate and may depend substantially upon the performance of certain third-party contractors;
- delays in validating, or inability to validate, any endpoints utilized in a clinical trial;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates, if any, or experienced by competitors who are developing topical rapamycin products or who are targeting the same indications in the rare skin diseases space;
- the ability of CMOs upon which we rely to manufacture clinical supplies of our product candidates or any future product candidates to remain in good standing with relevant regulatory authorities and to develop, validate and maintain commercially viable manufacturing processes that are compliant with cGMP;
- our ability to retain patients who have enrolled in a clinical study but may be prone to withdraw due to the rigors of the clinical trial, lack of efficacy, side effects, personal issues or loss of interest;

- our ability to establish and enforce intellectual property rights in and to our current product candidates or any future product candidates; and
- minimizing and managing any delay or disruption to our ongoing or planned clinical trials.

A change in the outcome of any of these factors with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

We may never succeed in achieving regulatory approval for any of our product candidates. Our preclinical studies and clinical trials may be unsuccessful. We may elect to discontinue, suspend or modify clinical trials of some product candidates or focus on others. A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA or another regulatory authority were to require us to conduct additional clinical trials beyond those that we currently anticipate will be required for the completion of any of our product candidates' clinical development, or if we experience significant delays in execution of or enrollment in any of our preclinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of preclinical and clinical development for such product candidates.

General and Administrative Expenses

Our general and administrative expenses consist primarily of the following costs:

- personnel costs, including salaries, related benefits, travel and stock-based compensation expense for personnel in executive, finance and administrative functions; and
- professional fees for legal, intellectual property, information technology, financial, human resources, consulting, audit and accounting services not otherwise included in research and development expenses.

We anticipate that our general and administrative expenses will increase substantially in the future as we increase our headcount to support our organizational growth. Following the completion of the Merger, we also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses associated with our operations as a public company. In addition, if we obtain regulatory approval for a product candidate and do not enter into a third-party commercialization collaboration, we expect to incur significant expenses related to building a sales and marketing organization to support product sales, marketing and distribution activities.

Other (Expense) Income

Our other (expense) income for the years ended December 31, 2025 and 2024 primarily consists of: (i) non-cash interest expense related to our obligation to make future royalty payments pursuant to the Amended Ligand Agreement, which was determined to be a debt instrument; (ii) interest expense related to the Convertible Notes; (iii) fair value adjustments related to our obligation to make future milestone payments under the Amended Ligand Agreement, which was determined to be a derivative liability; (iv) fair value adjustments related to the Convertible Notes, which were accounted for at fair value; (v) fair value adjustments related to the CVRs, which met the definition of a derivative, (vi) income related to a German R&D tax credit receivable, and (vii) interest income, net.

Our other (expense) income is subject to variability due to changes in the fair value of the derivative liabilities as well as the potential variability of the royalty agreement liability, both of which are based on significant estimates regarding the timing and success of future development and commercialization activities.

Income Taxes

Since May 2018, we have not recorded any income tax benefits for NOLs. We believe, based upon the weight of available evidence, that it is more likely than not that all of our NOLs and tax credits will not be realized. Accordingly, we have established a valuation allowance against such deferred tax assets for all periods since inception.

We assess our income tax positions and records tax benefits based upon management's evaluation of the facts, circumstances, and information available at the reporting date. For those tax positions where it is more likely than not that a tax benefit will be sustained, we record the amount of tax benefit with a greater than 50% likelihood of being realized upon ultimate settlement with a taxing authority having full knowledge of all relevant information. For those income tax positions for which it is not more likely than not that a tax benefit will be sustained, no tax benefit is recognized in the consolidated financial statements.

We had no provision for income taxes for the years ended December 31, 2025 and 2024. As of December 31, 2025, we had federal and state NOL carryforwards in the amount of $129.4 million and $113.3 million, respectively, which may be available to offset future taxable income. Federal tax loss carryforwards that were created prior to December 31, 2017 expire through 2037 and federal losses created after that date do not expire. State loss carryforwards expire starting in 2035.

As of December 31, 2025, we had German corporate income tax and trade tax net operating loss carryforwards of approximately $219.4 million and $215.1 million, respectively. Under current German laws, tax loss carryforwards may only be used to offset any relevant later assessment period (calendar year) of $1.2 million plus 60% of the exceeding taxable income and trade profit of such period and do not expire. In addition, certain transactions, including transfers of shares or interest in the loss holding entity, may result in the partial or total forfeiture of tax losses existing at that date. Partial or total forfeiture of tax losses may further occur in corporate reorganizations of the loss holding entity.

Results of Operations

Comparison of the Years Ended December 31, 2025 and 2024

The following sets forth our results of operations (in thousands):

	Year Ended December 31,		
	2025	2024	$ Change
Operating expenses:			
Research and development	$ 22,841	$ 8,151	$ 14,690
General and administrative	15,761	5,944	9,817
Total operating expenses	38,602	14,095	24,507
Operating loss	(38,602)	(14,095)	(24,507)
Other (expense) income:			
Interest expense – royalty agreement	(5,818)	(3,887)	(1,931)
Interest expense - convertible notes payable	—	(430)	430
Fair value adjustments on derivative liabilities – royalty agreement	(394)	(633)	239
Fair value adjustments on convertible notes payable	—	1,100	(1,100)
Fair value adjustments on derivative liabilities – contingent value right liability	(218)	(1,978)	1,760
Other income – German R&D tax credit receivable	—	1,978	(1,978)
Interest income, net	2,590	642	1,948
Other income (expense), net	727	(131)	858
Loss before income taxes	(41,715)	(17,434)	(24,281)
Income tax benefit (expense)	—	—	—
Net loss	$ (41,715)	$ (17,434)	$ (24,281)

Research and Development Expenses

The table below summarizes our research and development expenses incurred by development program (in thousands):

	Year Ended December 31,		
	2025	2024	$ Change
QTORIN rapamycin for microcystic LM	$ 4,755	$ 1,929	$ 2,826
QTORIN rapamycin for microcystic LM - Government grant income	(987)	$ (141)	(846)
QTORIN rapamycin for cutaneous VM	2,224	$ 296	1,928
QTORIN CMC	6,953	$ 1,601	5,352
Non-program specific and unallocated research and development expenses:			
Salaries and stock-based compensation	6,643	2,810	3,833
Consultants	1,910	1,040	870
Other	1,343	616	727
Total research and development expenses	$ 22,841	$ 8,151	$ 14,690

Research and development expenses for the year ended December 31, 2025 were $22.8 million, as compared to $8.2 million for the year ended December 31, 2024. The increase in research and development expenses during the year ended December 31, 2025 was primarily due to increased spending on the clinical development of QTORIN rapamycin for the treatment of microcystic LMs and cutaneous venous malformations, including conducting our Phase 3 SELVA and Phase 2 TOIVA trials, which were initiated in 2024. Additional increases include CMC costs for all programs and costs as a result of increased headcount in 2025.

General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2025 were $15.8 million, as compared to $5.9 million for the year ended December 31, 2024. The increase in general and administrative expenses during the year ended December 31, 2025, as compared to the year ended December 31, 2024 was primarily due to increased headcount in 2025, as well as increased professional services related to operating as a publicly-traded company.

Total Other (Expense) Income

Total other (expense) income, net for the year ended December 31, 2025 was $3.1 million of expense, as compared to $3.3 million of expense for the year ended December 31, 2024. The significant components of other (expense) income are more fully described below.

Interest (expense) income – royalty agreement

During the year ended December 31, 2025, we recorded interest expense of approximately $5.8 million, as compared to interest expense of approximately $3.9 million for the year ended December 31, 2024, related to the change in fair value of our royalty agreement liability.

Interest expense - convertible notes payable

During the year ended December 31, 2024, we recorded interest expense of approximately $0.4 million related to the Convertible Notes, which were issued during the year ended December 31, 2024. No such expense was incurred during 2025. As more fully described in Note 7 of the accompanying notes to the consolidated financial statements contained elsewhere in this Annual Report on Form 10-K, upon the closing of the PIPE Financing, the entire outstanding principal amount and unpaid accrued interest on the Convertible Notes automatically converted into our common stock (or prefunded warrants to purchase common stock). Accordingly, no interest expense was incurred subsequent to the Closing Date.

Fair value adjustments on derivative liabilities – royalty agreement

During the year ended December 31, 2025, we recorded a non-cash loss of approximately $0.4 million, as compared to a non-cash loss of approximately $0.6 million for the year ended December 31, 2024. The non-cash loss related to the change in fair value of our obligation to make future milestone payments under the Amended Ligand Agreement, which was determined to be a derivative liability.

Fair value adjustments on convertible notes payable

During the year ended December 31, 2024, we recorded $1.1 million of non-cash income from fair value adjustments on the Convertible Notes, which were issued in 2024 and converted to common stock (or prefunded warrants) upon closing of the PIPE Financing in December 2024.

Fair value adjustments on derivative liabilities – contingent value right liability

During the year ended December 31, 2025, we recorded a non-cash expense of approximately $0.2 million as compared to a non-cash expense of approximately $2.0 million from the year ended December 31, 2024, related to fair value adjustments related to the CVRs which were issued in 2024 in connection with the Business Combination and determined to be derivative liabilities. On the Closing Date, management concluded that there was no value associated with the CVRs as the likelihood of any payments received in connection with Pieris' legacy assets was remote. Subsequent to the Closing Date and as of December 31, 2024, a $2.0 million German research and development tax credit receivable was recorded by Pieris Pharmaceuticals GmbH resulting in a corresponding increase to the contingent value rights liability.

Other income – German R&D tax credit receivable

During the year ended December 31, 2024, we recorded $2.0 million of income related to a German research and development tax credit receivable recorded by Pieris Pharmaceuticals GmbH at December 31, 2024.

Interest income, net

During the year ended December 31, 2025, we recorded interest income, net of $2.6 million, as compared to $0.6 million for the year ended December 31, 2024. The increase was primarily due to increases in the average balances held in interest-bearing cash and money market funds.

Net Loss

As a result of the factors discussed above, our net loss for the years ended December 31, 2025 and 2024 was $41.7 million and $17.4 million, respectively.

Liquidity and Capital Resources

Sources of Liquidity

Since inception, we have incurred substantial losses, and have primarily funded our operations with proceeds from the Amended Ligand Agreement and the sale of debt and equity securities, including common stock, convertible preferred stock and convertible notes. During the year ended December 31, 2025, we incurred a net loss of $41.7 million and reported net cash used in operating activities of $25.0 million. As of December 31, 2025, we had an accumulated deficit of $135.5 million and cash and cash equivalents of $58.0 million. As discussed below, in February 2026, we completed an underwritten public offering of common stock resulting in net proceeds of approximately $215.8 million. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and, to a lesser extent, general and administrative expenditures.

We do not expect to generate commercial revenue or operating cash flows in the near-term, including the next two years. Our ability to continue as a going concern in the near term is largely dependent on our existing cash balance and our ability to obtain additional sources of financing in order to fund operating expenses, complete development of our product candidates, obtain regulatory approvals, launch, and commercialize our product candidates, and continue research and development programs.

February 2026 Public Offering

On February 27, 2026, we completed an underwritten public offering of 1,840,000 shares of our common stock, including the exercise in full of the underwriters' overallotment option to purchase additional shares of common stock, at a price to the public of $125.00 per share. The offering resulted in net proceeds of approximately $215.8 million, after deducting underwriting discounts and commissions and other offering expenses.

PIPE Financing

Concurrently with the execution of the Merger Agreement on July 23, 2024, Pieris entered into a securities purchase agreement (the "Purchase Agreement") with certain investors, including BVF Partners, L.P., an existing stockholder of Pieris (the "PIPE Investors"), pursuant to which, among other things, on the Closing Date and immediately following the consummation of the Merger, the PIPE Investors purchased (either for cash or in exchange for the termination and cancellation of outstanding convertible promissory notes issued by Legacy Palvella), and the Company issued and sold to the PIPE Investors, (i) 3,168,048 shares of Common Stock and (ii) Pre-Funded Warrants, exercisable for 2,466,456 shares of Common Stock, at a purchase price of $13.9965 per share or $13.9955 per Pre-Funded Warrant, which represents the per share purchase price of Common Stock less the $0.001 per share exercise price for each Pre-Funded Warrant, for an aggregate purchase price of approximately $78.9 million, consisting of approximately $60.0 million in cash and the conversion of approximately $18.9 million of principal and interest under outstanding convertible notes issued by Legacy Palvella (the "PIPE Financing").

Convertible Notes

On June 6, 2024, Legacy Palvella initiated a sequence of convertible notes with certain investors via a Convertible Note Purchase Agreement, pursuant to which the Company issued convertible notes in the aggregate principal amount of approximately $18.4 million (the "Convertible Notes") between June 2024 and December 2024. Simple interest accrued on the outstanding principal amount of the Convertible Notes at an annual rate of SOFR plus 2.0% per annum. Unless earlier converted, the maturity date of the Convertible Notes was the earliest to occur of (i) the date that Legacy Palvella received approval of an NDA by the FDA of QTORIN rapamycin in the United States, or (ii) June 3, 2027. Upon the closing of the PIPE Financing (defined above), the entire outstanding principal amount and unpaid accrued interest on the convertible notes automatically converted into an aggregate of 1,179,163 shares of Common Stock and 168,503 prefunded warrants.

Future Funding Requirements

We have not generated product revenue or achieved profitability since our inception and expect to continue to incur net losses for the foreseeable future. As of December 31, 2025, we had approximately $58.0 million in cash and cash equivalents. In February 2026, we completed a public offering of common stock resulting in net proceeds of approximately $215.8 million. Based on our current business plans, we believe that our existing cash and cash equivalents will be sufficient to fund our planned operations for at least the one year period following the date of the filing of this Form 10-K. Moreover, we expect our losses to increase as we continue to advance our product candidates through clinical trials and regulatory submissions. We may also incur expenses in connection with the in-licensing or acquisition of additional product candidates, which may not be currently contemplated in our planned operations. Furthermore, following the closing of the Merger, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Our primary uses of capital have been, and we expect will continue to be, compensation and related expenses, third-party clinical research, manufacturing and development services, license payments or milestone obligations that may arise, manufacturing costs, legal and other regulatory expenses and general overhead costs.

Based upon our current operating plan, we believe that our cash and cash equivalents on hand as of December 31, 2025 will be sufficient to fund our operating expenses for at least the next twelve months from the date of this Annual Report on Form 10-K. To continue to finance our operations beyond that point, we may need to raise additional capital, the success of which cannot be assured. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. If we receive regulatory approval for QTORIN rapamycin for the treatment of microcystic LM, cutaneous VMs, clinically significant angiokeratomas, QTORIN pitavastatin for the treatment of disseminated superficial actinic porokeratosis, or any of our future product candidates, we expect to incur significant commercialization expenses related to manufacturing, sales, marketing, and

distribution, or from any out-licensing of the product. We are also responsible for up to $5.0 million in milestone payments to Ligand under the Amended Ligand Agreement upon the achievement of certain regulatory milestones by us related to QTORIN rapamycin, which may be triggered prior to the commercialization of any of our product candidates and ability to generate revenue.

To the extent that we raise additional capital by issuing equity securities, our existing stockholders may experience substantial dilution, and the terms of these securities may include liquidation or other preferences detrimental to the rights of our common stockholders. Any agreements for future debt or preferred equity financings, if available, may involve covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. We may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future funding requirements depend on many factors, including, but not limited to:

- timing and outcome of regulatory review for QTORIN rapamycin for the treatment of microcystic LM, or our other product candidates;
- the cost of commercialization and manufacturing activities with respect to QTORIN rapamycin, QTORIN pitavastatin and our ability to successfully commercialize this product candidate, if approved;
- the scope, progress, results and costs of researching and developing QTORIN rapamycin, or any future product candidates, and conducting preclinical studies and clinical trials;
- the number and scope of clinical programs we decide to pursue;
- the cost of manufacturing our product candidates and any products we commercialize, including costs associated with developing our supply chain;
- the cost of commercialization activities if any of our product candidates are approved for sale, including marketing, sales and distribution costs;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such agreements that we may enter into;
- the timing and sales of any future approved products, if any;
- the potential size of the markets, degree of market acceptance, as well as the pricing and reimbursement for our approved products, if any;
- the timing and amount of milestone or royalty payments due under the Ligand Agreements or under similar arrangements with any future collaboration or licensing partners;
- the expenses needed to attract and retain skilled personnel;
- Our need to implement additional internal systems and infrastructure, including financial and reporting systems, and other costs associated with being a public company; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property portfolio.

Further, our development and commercialization operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities and commercialization of QTORIN rapamycin, if approved. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we may be unable to estimate the amounts of

increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

Cash Flows

The following table summarizes our cash flows for the years ended December 31, 2025 and 2024 (in thousands):

	Year Ended December 31,	
	2025	2024
Net cash used in operating activities	$ (25,006)	$ (10,840)
Net cash (used in) provided by financing activities	(660)	87,089
Effect of exchange rate change on cash and cash equivalents	46	3
Net (decrease) increase in cash and cash equivalents	$ (25,620)	$ 76,252

Net cash used in operating activities

Net cash used in operating activities for the years ended December 31, 2025 and 2024 consisted of net loss for the period adjusted for non-cash items and changes in components of operating assets and liabilities. The primary use of cash was to fund our operations related to the development of our product candidates, including general and administrative support, which increased due to greater research and development efforts in 2025, increased costs to operate as a public company, as well as the timing of payments and increase in accounts payable.

Net cash provided by financing activities

For the year ended December 31, 2025, net cash used in financing activities was $0.7 million, consisting primarily of payments of transaction costs incurred in connection with the Business Combination and proceeds from the exercise of options.

For the year ended December 31, 2024, net cash provided by financing activities was $87.1 million, which was primarily attributable to $13.8 million of cash acquired in connection with the Business Combination, $60.0 million in gross proceeds from the PIPE Financing, and $18.4 million in gross proceeds from the issuance of the Convertible Notes, partially offset by payment of transaction costs in connection with Business Combination of $2.5 million, payments of transaction costs associated with PIPE Financing of $2.5 million, and payments of transaction costs to issue the Convertible Notes of $0.1 million.

Contractual Obligations and Commitments

Leases

We lease office space in Wayne, Pennsylvania. Our future lease payments for these facilities are $0.7 million for the remaining term.

Ligand Agreement

We are party to a Development Funding and Royalties Agreement with Ligand Pharmaceuticals, Inc. (“Ligand”), dated December 13, 2018, as amended May 22, 2020 and November 28, 2023 (the “Ligand Agreement”). Under the Ligand Agreement, Ligand has made payments totaling $15.0 million to fund the development of QTORIN rapamycin. As partial consideration for the funding received, we granted Ligand the right to receive up to $8.0 million in milestone payments upon achievement of certain corporate, financing and regulatory milestones by us related to QTORIN rapamycin for the treatment of any and all indications, of which $5.0 million of potential future milestone payments remain under the arrangement. In addition, we agreed to pay to Ligand tiered royalties ranging from 8.0% to 9.8% of any aggregate annual worldwide net product sales of any products based on QTORIN rapamycin.

Other

Further, we enter into contracts in the normal course of business with service providers for clinical trials, preclinical research studies and testing, manufacturing, and other services and products for operating purposes. Our payment obligations under these contracts generally provide for termination upon notice and, therefore, we believe that our

non-cancelable obligations under these agreements are not material and we cannot reasonably estimate the timing of any such payments or if and when they will occur.

We may also enter into additional research, manufacturing, supplier and other agreements in the future, which may require up-front payments or long-term commitments of cash.

Critical Accounting Policies and Significant Judgments and Estimates

Management's discussion and analysis of our financial condition and results of operations is based on the audited consolidated financial statements included elsewhere in this Form 10-K, which have been prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in the notes to the audited consolidated financial statements included elsewhere in this Form 10-K, we believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Research and Development Expenses and Accruals

We estimate costs of research and development activities conducted by service providers, which include, the conduct of sponsored research, preclinical studies and contract manufacturing activities. We record the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and include these costs in accrued expenses and other current liabilities or prepaid expenses and other current assets on the balance sheets and within research and development expense on the statements of operations.

We estimate these costs based on factors such as estimates of the work completed and budget provided and in accordance with agreements established with our collaboration partners and third-party service providers. As actual costs become known, we adjust accrued liabilities or prepaid expenses. While our actual results could differ from their estimates, we have not experienced any material differences between accrued costs and actual costs incurred since our inception.

Our expenses related to clinical trials are based on estimates of patient enrollment and related expenses at clinical trial investigator sites as well as estimates for the services received and efforts expended pursuant to contracts with multiple research institutions that may be used to conduct and manage clinical trials on our behalf. We generally accrue expenses related to clinical trials based on contracted amounts applied to the level of patient enrollment and activity. If timelines or contracts are modified based upon changes in the clinical trial protocol or scope of work to be performed, we modify estimates of accrued expenses accordingly on a prospective basis.

Ligand Agreement

Under the terms of the Amended Ligand Agreement, we received $15.0 million to fund the development of QTORIN rapamycin, in exchange for Ligand's right to receive future payments based on the development and commercialization of products covered under the Amended Ligand Agreement. Ligand is entitled to receive up to an additional $5.0 million of milestone payments upon the achievement of certain regulatory milestones by us related to QTORIN rapamycin for the treatment of any and all indications. Our obligation to make milestone payments under the Amended Ligand Agreement was determined to be a derivative liability, and our obligation to make future royalty payments was determined to be a debt instrument.

The accounting for liabilities under the Amended Ligand Agreement requires us to make certain estimates and assumptions about the timing and probability of FDA approval and commercialization, and the amount of future net sales for any product containing QTORIN rapamycin. The estimated future net sales are based on subjective

assumptions that include the estimated size of the addressable patient population and the anticipated pricing of the Company's products. These assumptions are subject to significant variability, and are thus subject to significant uncertainty.

Royalty payments will be recorded as debt service payments on the royalty agreement liability. Interest expense is determined using the effective interest method based upon risk adjusted cash flow estimates of our expected future royalty payments, yielding an effective interest rate of 44.9% and 39.9% as of December 31, 2025 and 2024, respectively. The effective interest rate is estimated at each balance sheet date based on the rate that would enable the liability to be repaid in full over the anticipated life of the arrangement. This effective interest rate will likely be subject to variability as we continue the development and commercialization of our products. The derivative liabilities — royalty agreement is classified as long term on our balance sheet according to the estimated timing of the occurrence of potential payments.

The fair value of the derivative liabilities — royalty agreement with respect to the potential milestone payments is determined based upon the estimated probabilities and timing of the achievement of milestones, discounted to present value using our estimated weighted average cost of capital. The assumptions used to determine the fair value of the derivative liabilities — royalty agreement at December 31, 2025 and 2024 were (a) weighted cost of capital of 18.0% and 20.0%; and (b) 56.6% and 56.6% probability of achieving regulatory approval of a product by the FDA with a term of 1.50 years and 2.50 years, respectively. Gains and losses arising from changes in fair value of the derivative liabilities — royalty agreement are recognized within our statements of operations as fair value adjustments on the derivative liabilities — royalty agreement and in the balance sheet as a non-current liability for each financial reporting period.

Our estimates and assumptions with respect to the royalty agreement liability and derivative liabilities — royalty agreement are likely to change as we develop and commercialize QTORIN rapamycin, if approved. Any such adjustments that may become necessary will impact the recorded value of the royalty agreement liability and the derivative liabilities — royalty agreement, the accretion of interest expense on the royalty agreement liability and the fair value adjustments on derivative liabilities — royalty agreement.

CVR Agreement

Under the terms of the CVR Agreement, legacy Pieris stockholder's have the right to receive payments upon the receipt of payments by the us or any of our affiliates under certain strategic partner agreements, including existing collaboration agreements pursuant to which we may be entitled to milestones and royalties in the future and other out-licensing agreements for certain of Pieris's legacy assets, and upon the receipt of certain research and development tax credits in favor of us or any of its affiliates, in each case as set forth in, and subject to and in accordance with the terms and conditions of, the CVR Agreement.

Management concluded that the CVRs meet the definition of a derivative and will be initially measured at the aggregate estimated fair value of the CVRs and will be subsequently remeasured at estimated fair value on a recurring basis at each reporting period date. Changes in fair value of the CVR derivative are presented in the consolidated statements of operations and comprehensive income (loss). To determine the derivative value at each reporting period, management applies a scenario-based method and weighs them based on the possible likelihood of certain contingencies triggering payments due under the CVR for the liability recognized. The fair value measurements are based on significant inputs not observable in the market, such as estimated cash flows, estimated probabilities of success, and risk-adjustment discount rates, and thus represent a Level 3 measurement as defined in ASC 820, *Fair Value Measurement*. The estimated value of the CVR is based upon available information and certain assumptions, which our management believes are reasonable under the circumstances.

As of the Closing Date, management concluded that there was no value associated with the CVRs as the likelihood of any payments received in connection with Pieris' legacy assets was remote. As of December 31, 2025, management recorded a contingent rights value liability of approximately $2.2 million due to a receivable for research and development tax credits by Pieris Pharmaceuticals GmbH at December 31, 2025. In January 2026, the Company paid out approximately $2.0 million to holders of CVRs related to receipt of certain research and development tax credits.

Stock-Based Compensation

We account for stock-based compensation in accordance with ASC Topic 718, *Compensation – Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations based on their fair values. All of the stock-based awards are subject only to service-based vesting conditions. Management estimates the fair value of the stock option awards using the Black-Scholes option pricing model, which requires the input of assumptions, including (a) the fair value of the Company's common stock, (b) the expected stock price volatility, (c) the calculation of expected term of the award, (d) the risk-free interest rate and (e) expected dividends. Management estimates the fair value of the restricted stock awards using the fair value of the Company's common stock. Forfeitures are recognized as they are incurred.

Prior to the Merger, we periodically estimated the fair value of the our common stock considering, among other things, valuations of its common stock prepared by management with the assistance of a third-party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation.

Following the Merger, the fair value of our common stock is based on the closing stock price on the date of grant as reported on the Nasdaq Global Market. The expected life of the stock options in years is estimated using the "simplified method," as prescribed in SEC's Staff Accounting Bulletin (SAB) No. 107, as the Company has no historical information from which to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior for its stock option grants. The simplified method is the midpoint between the vesting period and the contractual term of the option. For stock price volatility, we use comparable public companies as a basis for its expected volatility to calculate the fair value of option grants. The risk-free rate is based on the U.S. Treasury yield curve commensurate with the expected life of the option. The expected dividend yield is zero as the Company has no history of paying dividends and no plans to do so in the near term. We classify stock-based compensation expense in our consolidated statement of operations and comprehensive (loss) income in the same manner of the award recipient's payroll costs.

Recently Adopted Accounting Pronouncements

Refer to Note 2, "*Summary of Significant Accounting Policies*," of the notes to our audited consolidated financial statements appearing elsewhere in this Form 10-K for a discussion of recent accounting pronouncements.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company as defined by Item 10 of Regulation S-K and are not required to provide the information otherwise required under this item.

Item 8. Financial Statements and Supplementary Data.

All financial statements required to be filed hereunder are filed under Item 15(a) of this Form 10-K and are incorporated herein by reference.

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and therefore we are permitted to provide a scaled Item 8 disclosure.

There have been no retrospective changes to our consolidated statements of operations for any of the quarters within the two years ended December 31, 2025.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of December 31, 2025. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applied its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on this evaluation, our principal executive officer and principal financial officer concluded that, as of December 31, 2025, our disclosure controls and procedures were (1) designed to ensure that material information relating to us is made known to our principal executive officer and principal financial officer by others, particularly during the period in which this report was prepared, and (2) effective, in that they provide reasonable assurance that information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the applicable SEC rules and forms.

Management's Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the 2013 framework in Internal Control–Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under that framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2025.

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm regarding internal control over financial reporting as we are a non-accelerated filer and a "smaller reporting company", as defined in Rule 12b-2 under the Exchange Act.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the three months ended December 31, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

(a) None.

(b) Rule 10b5-1 and Non-Rule 10b5-1 Trading Arrangements

During the quarter ended December 31, 2025, none of our directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" (as each term is defined in Item 408(a) of Regulation S-K).

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this item is incorporated by reference to our Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2025.

We have adopted insider trading policies and procedures governing the purchase, sale, and other dispositions of our securities by directors, officers, and employees that we believe are reasonably designed to promote compliance with insider trading laws, rules and regulations, and applicable Nasdaq listing standards. Our insider trading policy states, among other things, that our directors, officers, and employees are prohibited from trading in such securities while in possession of material, nonpublic information. The foregoing summary of our insider trading policies and procedures does not purport to be complete and is qualified by reference to our Insider Trading Policy filed as an exhibit to this Annual Report on Form 10-K. In addition, with regard to trading in our own securities, it is our policy to comply with the federal securities laws and the applicable exchange listing requirements.

Item 11. Executive Compensation.

The information required by this item is incorporated by reference to our Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2025.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item is incorporated by reference to our Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2025.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item is incorporated by reference to our Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2025.

Item 14. Principal Accounting Fees and Services.

The information required by this item is incorporated by reference to our Proxy Statement for the 2026 Annual Meeting of Stockholders to be filed with the SEC within 120 days of the fiscal year ended December 31, 2025.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) (1) Financial Statements.

(2) We are not filing any financial statement schedules as part of this Annual Report on Form 10-K because they are not applicable or the required information is included in the financial statements or notes thereto.

(3) The list of Exhibits filed as part of this Annual Report on Form 10-K is set forth on the Exhibit Index below.

(b) The list of Exhibits filed as part of this Annual Report on Form 10-K is set forth on the Exhibit Index below.

(c) None.

Item 16. Form 10-K Summary

Not applicable.

Exhibit Index

Exhibit Number	Exhibit Description	Incorporated by Reference (if applicable) Form	File No.	Exhibit	Filing Date	Filed Herewith
2.1	Agreement and Plan of Merger dated as of July 23, 2024, by and among Pieris Pharmaceuticals, Inc., Polo Merger Sub, Inc., and Palvella Therapeutics, Inc.	8-K	001-37471	2.1	July 24, 2024	
3.1	Amended and Restated Articles of Incorporation	8-K	333-190728	3.1	December 18, 2014	
3.2	Certificate of Change to Articles of Incorporation	8-K	001-37471	3.1	April 18, 2024	
3.3	Certificate of Amendment to Articles of Incorporation	8-K	001-37471	3.9	December 13, 2024	
3.4	Certificate of Withdrawal of the Series F Certificate of Designation	8-K	001-37471	3.10	December 13, 2024	
3.5	Certificate of Amendment to Articles of Incorporation	8-K	001-37471	3.11	December 13, 2024	
3.6	Amended and Restated Bylaws	8-K	333-190728	3.2	December 18, 2014	
3.7	Amendment to the Amended and Restated Bylaws	8-K	001-37471	3.1	September 3, 2019	
4.1	Description of Securities					X
4.2	Form of Common Stock Certificate	8-K	001-37471	4.1	December 13, 2024	
4.3	Form of Pre-Funded Warrant	8-K	001-37471	4.1	July 24, 2024	
10.1*	Form of Indemnification Agreement	8-K	333-190728	10.10	December 18, 2014	
10.2*	2018 Employee Stock Purchase Plan	8-K	001-37471	10.2	July 26, 2018	
10.3*	2023 Employee Stock Purchase Plan	10-Q	001-37471	10.2	August 10, 2023	
10.4*	2014 Employee, Director and Consultant Equity Incentive Plan	8-K	333-190728	10.1	December 18, 2014	
10.5*	2016 Employee, Director and Consultant Equity Incentive Plan	8-K	011-37471	10.1	July 1, 2016	
10.6*	2019 Employee, Director and Consultant Equity Incentive Plan	8-K	001-37471	10.1	July 31, 2019	
10.7*	2020 Employee, Director and Consultant Equity Incentive Plan	8-K	001-37471	10.1	June 29, 2020	
10.8*	2020 Employee, Director and Consultant Equity Incentive Plan, as amended	8-K	001-37471	10.1	June 29, 2021	
10.9*	2020 Employee, Director and Consultant Equity Incentive Plan, as amended	8-K	001-37471	10.1	June 27, 2022	
10.10*	2020 Employee, Director and Consultant Equity Incentive Plan, as amended	8-K	0001-37471	10.1	June 26, 2023	

10.11#	Development Funding and Royalties Agreement, dated December 13, 2018, by and between Ligand Pharmaceuticals, Inc. and Palvella Therapeutics, Inc.	S-4/A	333-281459	10.18	November 5, 2024
10.12	First Amendment to Development Funding and Royalties Agreement, dated May 22, 2020, by and between Ligand Pharmaceuticals, Inc. and Palvella Therapeutics, Inc.	S-4	333-281459	10.19	August 9, 2024
10.13#	Second Amendment to Development Funding and Royalties Agreement, dated November 28, 2023, by and between Ligand Pharmaceuticals, Inc. and Palvella Therapeutics, Inc.	S-4/A	333-281459	10.20	November 5, 2024
10.14*	Employment Agreement, dated May 20, 2020, by and between Wesley Kaupinen and Palvella Therapeutics, Inc.	S-4	333-281459	10.21	August 9, 2024
10.15*	Severance Agreement, dated May 22, 2020, by and between Kathleen Goin and Palvella Therapeutics, Inc.	S-4	333-281459	10.24	August 9, 2024
10.16*	Offer Letter, dated July 27, 2020, by and between Jeffrey Martini and Palvella Therapeutics, Inc.	S-4	333-281459	10.25	August 9, 2024
10.17*	Offer Letter, dated August 19, 2019, by and between Kathleen Goin and Palvella Therapeutics, Inc.	S-4	333-281459	10.26	August 9, 2019
10.18*+	Offer Letter, dated October 9, 2024, by and between Matthew Korenberg and Palvella Therapeutics, Inc.	S-4/A	333-281459	10.27	November 5, 2024
10.19*	Severance Agreement, dated October 10, 2024, by and between Matthew Korenberg and Palvella Therapeutics, Inc.	S-4/A	333-281459	10.28	November 5, 2024
10.20	Offer Letter dated May 21, 2025, by and between Ashley Kline and Palvella Therapeutics, Inc.	10-Q	001-37471	10.1	August 14, 2025
10.21	Severance Agreement, dated May 21, 2025, by and between Ashley Kline and Palvella Therapeutics, Inc.	10-Q	001-37471	10.2	August 14, 2025
10.22	Contingent Value Rights Agreement, dated as of December 13, 2024, by and among, Palvella Therapeutics, Inc. (formerly Pieris Pharmaceuticals, Inc.), Shareholder Representative Services LLC and Computershare Inc. and Computershare Trust Company, N.A.	8-K	001-37471	10.27	December 13, 2024
10.23*	Palvella Therapeutics, Inc. 2019 Equity Incentive Plan	S-4	333-281459	10.9	August 9, 2024
10.24*	Amendment No. 1 to Palvella Therapeutics, Inc. 2019 Equity Incentive Plan	S-4	333-281459	10.12	August 9, 2024
10.25*	Form of Incentive Stock Option Agreement under the Palvella Therapeutics, Inc. 2019	S-4	333-281459	10.10	August 9, 2024

10.26*	Form of Non-Qualified Stock Option Agreement under the Palvella Therapeutics, Inc. 2019 Equity Incentive Plan	S-4	333-281459	10.11	August 9, 2024	
10.27*	Palvella Therapeutics, Inc. 2024 Equity Incentive Plan	8-K	001-37471	10.1	December 12, 2024	
10.28*	Form of Stock Option Grant Notice and Stock Option Agreement	8-K	001-37471	10.29	December 13, 2024	
10.29*	Form of Notice of Grant of Restricted Stock Units Award	8-K	001-37471	10.30	December 13, 2024	
10.30	Office Building Lease, dated October 1, 2025, by and between LMP 353, LLC and Palvella Therapeutics, Inc.					X
19.1∞	Palvella Therapeutics, Inc. Insider Trading Policy	10-K	001-37471	19.1	March 31, 2025	
21.1	List of Subsidiaries of Palvella Therapeutics, Inc.					X
23.1	Consent of Ernst & Young LLP					X
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
97.1	Palvella Therapeutics, Inc. Clawback Policy	10-K	001-37471	97.1	March 31, 2025	
101.SCH	Inline XBRL Taxonomy Extension Schema Document					
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document					
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document					
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document					

101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (embedded within the Inline XBRL and contained in Exhibit 101)

∞ Exhibits and/or schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The registrant hereby undertakes to furnish supplementally copies of any of the omitted exhibits and schedules upon request by the SEC.

+ Portions of this exhibit have been redacted in compliance with Regulation S-K Item 601(a)(6).

Certain confidential information contained in this exhibit, marked by brackets, has been omitted pursuant to Item 601(b)(10)(iv) because the information (i) is not material and (ii) is the type of information that the Registrant both customarily and actually treats as private and confidential.

* Indicates management contract or compensatory plan

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized**.**

Palvella Therapeutics, Inc.

By: /s/ Wesley H. Kaupinen

Date: March 31, 2026

Wesley H. Kaupinen
President and Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ Wesley H. Kaupinen **Wesley H. Kaupinen**	President, Chief Executive Officer and Director *(principal executive officer)*	March 31, 2026
/s/ Matthew E. Korenberg **Matthew E. Korenberg**	Chief Financial Officer and Treasurer *(principal financial officer and principal accounting officer)*	March 31, 2026
/s/ George M. Jenkins **George M. Jenkins**	Chairman	March 31, 2026
/s/ Todd C. Davis **Todd C. Davis**	Director	March 31, 2026
/s/ Elaine J. Heron, PhD **Elaine J. Heron, PhD**	Director	March 31, 2026
/s/ Christopher Kiritsy **Christopher Kiritsy**	Director	March 31, 2026
/s/ Tadd S. Wessel **Tadd S. Wessel**	Director	March 31, 2026

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of Palvella Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Palvella Therapeutics, Inc. (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations and comprehensive loss, changes in convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the two years in the period ended December 31, 2025, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Royalty agreement liability and related interest expense

Description of the Matter	As described in Note 4 to the financial statements, the Company is party to a development funding agreement with Ligand Pharmaceuticals, Inc. (Ligand) (the Ligand Agreement). Pursuant to the Ligand Agreement, as partial consideration for the upfront payment received from Ligand, the Company agreed to pay to Ligand tiered future royalties based on any aggregate annual worldwide net product sales of any products based on QTORIN rapamycin. The Company recorded its obligation to pay tiered royalties under the Ligand Agreement as a debt instrument (royalty agreement liability) on the balance sheet at a carrying value of $11.9M as of December 31, 2025 and has recognized imputed interest expense of $3.9M for the year ended December 31, 2025 using the effective interest rate method. The effective interest rate is estimated at each balance sheet date based on the rate that would enable the liability to be repaid in full over the anticipated life of the arrangement. The effective interest rate may vary during the term of the Ligand Agreement

based on changes in the probability-adjusted cash flow estimates of the Company's potential future royalty payments under the Ligand Agreement.

Auditing the interest expense associated with the royalty agreement liability involved complex and subjective auditor judgment due to the estimation uncertainty involved in determining the probability-adjusted cash flow estimates of the Company's potential future royalty payments. The Company's effective interest rate calculation includes probability-adjusted revenue projections for which future royalties will be paid, which are sensitive to significant assumptions including the size of the addressable patient population, the anticipated pricing of the Company's products, and the probability of successful development and commercialization.

How We Addressed the Matter in Our Audit	To test the interest expense associated with the royalty agreement liability, our audit procedures included, among others, testing the significant assumptions used to develop the estimates and evaluating the completeness and accuracy of the underlying data used by the Company in its effective interest rate calculation. For example, we compared the estimated size of the addressable patient population to a third-party prevalence study and government census data, and we compared the anticipated pricing information to a third-party market analysis. We compared the probability of achieving development and commercial success to studies published in medical journals evaluating clinical advancement and approval rates for similar products. We tested that the revenue projections were updated based on the most recent clinical trial data received in 2025 and recalculated the current year interest expense.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2018.

Philadelphia, Pennsylvania

March 31, 2026

PALVELLA THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share amounts)

	December 31, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 57,982	$ 83,602
Accounts receivable	—	358
German research and development tax credit receivable	—	1,978
Prepaid expenses and other current assets	1,005	2,296
Total current assets	58,987	88,234
Operating lease right-of-use assets, net	572	—
Total assets	**$ 59,559**	**$ 88,234**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 4,595	$ 4,586
Derivative liabilities – contingent value right liability	2,196	1,978
Current portion of operating lease liability	202	—
Accrued expenses and other current liabilities	4,351	5,474
Total current liabilities	11,344	12,038
Long-term portion of operating lease liability	431	—
Royalty agreement liability	17,760	11,942
Derivative liabilities – royalty agreement	2,041	1,647
Total liabilities	31,576	25,627
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Preferred stock, $0.001; 10,000,000 shares authorized; 0 and 15,617 shares issued and outstanding at December 31, 2025 and 2024	—	—
Common stock, $0.001 par value; 200,000,000 shares authorized; 12,380,933 and 11,012,105 shares issued and outstanding at December 31, 2025 and 2024	12	11
Additional paid-in capital	163,338	156,328
Accumulated other comprehensive (loss) income	83	3
Accumulated deficit	(135,450)	(93,735)
Total stockholders' equity	27,983	62,607
Total liabilities and stockholders' equity	**$ 59,559**	**$ 88,234**

The accompanying notes are an integral part of these consolidated financial statements.

PALVELLA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2025	2024
Operating expenses:		
Research and development	$ 22,841	$ 8,151
General and administrative	15,761	5,944
Total operating expenses	38,602	14,095
Operating loss	(38,602)	(14,095)
Other (expense) income:		
Interest expense – royalty agreement	(5,818)	(3,887)
Interest expense – convertible notes payable	—	(430)
Fair value adjustments on derivative liabilities – royalty agreement	(394)	(633)
Fair value adjustments on convertible notes payable	—	1,100
Fair value adjustments on derivative liabilities – contingent value right liability	(218)	(1,978)
Other income – German R&D tax credit receivable	—	1,978
Interest income, net	2,590	642
Other income (expense), net	727	(131)
Loss before income taxes	(41,715)	(17,434)
Income tax benefit (expense)	—	—
Net loss	$ (41,715)	$ (17,434)
Other comprehensive loss:		
Foreign currency translation gain	80	3
Comprehensive loss	$ (41,635)	$ (17,431)
Net loss per share of Common Stock – basic and diluted	$ (3.71)	$ (7.83)
Weighted-average shares used in computing net loss per share of Common Stock – basic and diluted	11,251,250	2,225,934

The accompanying notes are an integral part of these consolidated financial statements.

PALVELLA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CHANGES IN CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
(in thousands, except share amounts)

	Convertible Preferred Stock		Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2023	**15,360,787**	**70,603**	**—**	**—**	**1,770,167**	**2**	**1,816**	**—**	**(76,301)**	**(74,483)**
Conversion of convertible preferred stock into common stock in connection with Reverse Merger	(15,360,787)	(70,603)	—	—	4,753,650	5	70,598	—	—	70,603
Conversion of convertible notes into common stock and prefunded warrants in connection with Reverse Merger	—	—	—	—	1,179,163	1	17,761	—	—	17,762
Issuance of common stock and prefunded warrants in connection with PIPE	—	—	—	—	1,988,885	2	56,187	—	—	56,189
Issuance of preferred stock and common stock to former stockholders of Pieris in connection with Reverse Merger	—	—	15,617	—	1,320,240	1	11,610	—	—	11,611
Transactions costs associated with the Reverse Merger	—	—	—	—	—	—	(2,474)	—	—	(2,474)
Stock-based compensation	—	—	—	—	—	—	830		—	830
Foreign currency translation gain	—	—	—	—	—	—	—	3	—	3
Net loss	—	—	—	—	—	—	—	—	(17,434)	(17,434)
Balance at December 31, 2024	**—**	**$ —**	**15,617**	**$ —**	**11,012,105**	**$ 11**	**$ 156,328**	**$ 3**	**$ (93,735)**	**$ 62,607**
Exercise of common stock options for cash, net of expense	—	—	—	—	88,828	—	763	—	—	763
Conversion of preferred stock into common stock	—	—	(15,617)	—	208,324	—	—	—	—	—
Conversion of prefunded warrants into common stock	—	—	—	—	1,071,676	1	—	—	—	1
Equity issuance costs	—	—	—	—	—	—	(154)	—	—	(154)
Stock-based compensation	—	—	—	—	—	—	6,401	—	—	6,401
Foreign currency translation gain	—	—	—	—	—	—	—	80	—	80
Net loss	—	—	—	—	—	—	—	—	(41,715)	(41,715)
Balance at December 31, 2025	**—**	**$ —**	**—**	**$ —**	**12,380,933**	**$ 12**	**$ 163,338**	**$ 83**	**$ (135,450)**	**$ 27,983**

The accompanying notes are an integral part of these consolidated financial statements.

PALVELLA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2025	2024
CASH FLOWS FROM OPERATING ACTIVITIES		
Net loss	$ (41,715)	$ (17,434)
Adjustments to reconcile net loss income to net cash used in operating activities:		
Non-cash interest expense – royalty agreement	5,818	3,887
Non-cash interest expense – convertible notes	—	430
Change in fair value of derivative liabilities – royalty agreement	394	633
Change in fair value of derivative liabilities – contingent value right liability	218	1,978
Change in fair value of convertible notes payable	—	(1,100)
Stock-based compensation	6,401	830
Non-cash lease operating expense	62	—
Costs to issue convertible notes	—	129
Change in operating assets and liabilities:		
Accounts receivable	387	—
German research and development tax credit receivable	2,105	(1,978)
Prepaid expenses and other current assets	1,364	(1,503)
Accounts payable	1,260	2,238
Accrued expenses and other current liabilities	(1,300)	1,050
Net cash used in operating activities	(25,006)	(10,840)
CASH FLOWS FROM FINANCING ACTIVITIES		
Cash acquired from Reverse Merger transaction	-	13,800
Payment of transaction costs in connection with Reverse Merger	(1,269)	(2,474)
Proceeds from PIPE Financing	—	60,001
Payments of transaction costs associated with PIPE Financing	—	(2,542)
Proceeds from issuance of convertible notes	—	18,433
Payment of transaction costs to issue convertible notes	—	(129)
Proceeds from exercise of stock options	763	—
Deferred equity issuance costs	(154)	—
Net cash (used in) provided by financing activities	(660)	87,089
Effect of exchange rate change on cash and cash equivalents	46	3
Net (decrease) increase in cash and cash equivalents	(25,620)	76,252
Cash and cash equivalents at beginning of period	83,602	7,350
Cash and cash equivalents at end of period	$ 57,982	$ 83,602
SUPPLEMENTARY DISCLOSURE OF NONCASH FINANCING ACTIVITIES:		
Transaction costs related to Reverse Merger included in accounts payable and accrued expenses	$ —	$ 1,269

The accompanying notes are an integral part of these consolidated financial statements.

PALVELLA THERAPEUTICS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Note 1. Description of Business, Organization and Liquidity

Business

Palvella Therapeutics, Inc. (the "Company," or "Palvella") (previously named Pieris Pharmaceuticals, Inc. ("Pieris")), a Nevada corporation, is a late clinical-stage biopharmaceutical company committed to serving individuals suffering from serious, rare skin diseases and vascular malformations without approved therapies. The Company's lead product candidate, QTORIN 3.9% rapamycin anhydrous gel ("QTORIN rapamycin"), is based on the Company's patented QTORIN platform. QTORIN rapamycin is in clinical development for microcystic lymphatic malformations ("microcystic LMs"), cutaneous venous malformations ("cutaneous VMs") and angiokeratomas. The Company is conducting IND-enabling development for QTORIN pitavastatin for disseminated superficial actinic porokeratosis ("DSAP"). Since inception, the Company has devoted substantially all of its resources to identifying, researching and conducting preclinical and clinical activities for its product candidates, and developing its platform technology, organizing and staffing the Company, business planning, raising capital and establishing its intellectual property portfolio. The Company's principal executive offices are located in Wayne, Pennsylvania.

Reverse Merger

On December 13, 2024 (the "Closing Date"), the Company consummated the previously announced merger pursuant to the terms of that certain Agreement and Plan of Merger, dated as of July 23, 2024 (the "Merger Agreement"), by and among the Company, Polo Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of Pieris (the "Merger Sub"), and Palvella Therapeutics, Inc., a Delaware corporation ("Legacy Palvella"). Pursuant to the Merger Agreement, on the Closing Date, (i) Merger Sub merged with and into Legacy Palvella, with Legacy Palvella as the surviving company in the merger and, after giving effect to such merger, continuing as a wholly owned subsidiary of the Company (the "Reverse Merger") and (ii) the Company's name was changed from Pieris Pharmaceuticals, Inc. to Palvella Therapeutics, Inc. (the "Reverse Merger" and, together with the other transactions contemplated by the Merger Agreement, the "Business Combination"). See Note 3 for additional details.

Liquidity

The Company follows the provisions of Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 205-40, *Presentation of Financial Statements—Going Concern*, which requires management to assess the Company's ability to continue as a going concern within one year after the date the financial statements are issued.

Since inception, the Company has incurred net losses and negative cash flows from operations. During the year ended December 31, 2025, the Company reported net loss of $41.7 million, and net cash used in operating activities of $25.0 million. At December 31, 2025, the Company had an accumulated deficit of $135.5 million.

Management does not expect to generate commercial revenue or operating cash flows in the near term, including in the next two years. The Company's ability to continue as a going concern in the near term is largely dependent on its existing cash and cash equivalents balance and ability to obtain additional sources of financing in order to fund operating expenses, complete development of its product candidates, obtain regulatory approvals, launch, and commercialize its product candidates, and continue research and development programs. Assuming no additional fund raising, the Company's forecasted cash required to fund operations indicates that the Company has sufficient funds to support operations through at least the one-year period from the issuance date of these consolidated financial statements.

Note 2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States ("U.S. GAAP"). Any references in these notes to applicable guidance is meant to refer to U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB"). The consolidated financial statements and notes as of December 31, 2025 and 2024 include the accounts of the Company and its subsidiaries. All intercompany balances and transactions are eliminated in consolidation.

Use of Estimates

The preparation of consolidated financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and reported amounts of revenues and expenses during the reporting period. Management considers many factors in selecting appropriate financial accounting policies and controls and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process and actual results could differ materially from those estimates.

Foreign Currency Translation

The financial statements of the Company's foreign subsidiaries are translated from local currency into reporting currency, which is U.S. dollars, using the current exchange rate at the balance sheet date for assets and liabilities, and the weighted average exchange rate prevailing during the period for revenues and expenses. The functional currency for foreign subsidiaries is considered to be the local currency for each entity and, accordingly, translation adjustments for these subsidiaries are included in accumulated other comprehensive loss within stockholders' equity.

Realized and unrealized gains and losses resulting from foreign currency transactions denominated in currencies other than the functional currency are reflected as other (expense) income, net in the consolidated statements of operations.

Concentration of Credit Risk and Other Risks and Uncertainties

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company holds cash at two accredited financial institutions in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships. In addition, the Company also maintains cash in a German bank account in denominations of Euros and U.S. dollars. The Company has not experienced any losses on its deposits of cash and cash equivalents.

The Company is dependent on contract manufacturing organizations ("CMOs") to supply products for research and development of its product candidates, including pre-clinical and clinical studies, and for commercialization of its product candidates, if approved. The Company's development programs could be adversely affected by any significant interruption in its CMOs' operations or by a significant interruption in the supply of active pharmaceutical ingredients and other components.

Products developed by the Company require approval from the U.S. Food and Drug Administration ("FDA") or other international regulatory agencies prior to commercial sales. There can be no assurance the Company's product candidates will receive the necessary approvals. If the Company is denied approvals, approvals are delayed, or it is unable to maintain approvals received, such events could have a materially adverse impact on the Company.

Comprehensive Loss and Accumulated Other Comprehensive (Loss) Income

Other comprehensive (loss) income is defined as the change in equity during a period from transactions and other events and circumstances from non-owner sources, including unrealized gains and losses on investments and foreign currency translation gains and losses.

Cash and Cash Equivalents

Cash and cash equivalents are held in accounts at multiple independent financial institutions. Cash equivalents are defined as money market funds with maturity from date of purchase of 90 days or less that are readily convertible into cash.

Accounts Receivable

Accounts receivable are amounts due from our vendors as a result of research and development and other services provided, as well as the shipment of clinical product.

Fair Value Measurements

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1 – Quoted prices in active markets for identical assets or liabilities.
- Level 2 – Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3 – Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies, and similar techniques.

At December 31, 2025 and 2024, the carrying amounts of financial instruments, which include cash and cash equivalents, accounts payable, and accrued expenses and other liabilities, approximate their fair value due to their short maturities. At December 31, 2025 and 2024, the fair value of the royalty agreement liability, which is based on Level 3 inputs (including probability-weighted cash flow estimates of the Company's potential future royalty payments and a weighted-average cost of capital 18.0% and 20.0%, respectively) is approximately $61.5 and $26.6 million, respectively. The Company records its derivative liabilities at fair value.

Derivative Instruments

The Company evaluates its contracts to determine if those contracts qualify as derivatives under ASC 815, *Derivatives and Hedging*. For derivative financial instruments that are accounted for as assets or liabilities, the derivative instrument is initially recorded at its fair value and is then re-valued at each reporting date. Any changes in fair value are recorded as other income or expense for each reporting period. Derivative instrument assets or liabilities are classified in the balance sheet as current or non-current based on whether or not net-cash settlement of the derivative instrument is probable within the next 12 months from the balance sheet date.

The Company has milestone payments which may be required in connection with the royalty agreement (see Note 4) that were determined to be derivative liabilities. The valuation of the derivative liabilities is based on unobservable inputs and, therefore, represent Level 3 financial liabilities. The fair value of the derivative liabilities – royalty agreement was calculated using the present value of the potential payments using a weighted-average cost of capital and an assessment of the probability of the achievement of the milestones as well as an assessment of the timing of the potential milestone payments.

The derivative liabilities – royalty agreement was initially recorded at fair value, with gains and losses arising for changes in fair value of the derivative liabilities – royalty agreement recognized within the consolidated statements of operations as fair value adjustments on the derivative liabilities at each financial reporting period.

The Company determined that certain contingent payments that may become payable under the CVR Agreement related to the asset sales prior to the Reverse Merger qualified as derivatives under ASC 815. Upon such time that these payments were assessed a fair value, they were recorded as a liability on the balance sheet. These values are then remeasured for future expected payout or receipt, as well as the increase in fair value due to the time value of money. These gains or losses, if any, are recognized in the consolidated statements of operations within other income, net.

The derivative liabilities – CVR agreement was initially recorded at fair value, with gains and losses arising for changes in fair value of the derivative liabilities – CVR recognized within the consolidated statements of operations as fair value adjustments on the derivative liabilities at each financial reporting period.

Research and Development Expenses

Research and development costs are charged to expense as incurred. Research and development expenses include, among other costs, salaries and benefits of scientific personnel and the external cost of producing and testing the clinical material for clinical trials.

The Company has entered various research and development and clinical trial-related contracts. The Company defers and capitalizes prepaid nonrefundable advance research and development payments to third parties for goods and services to be used in future research and development activities and recognizes to research and development expense over the period that the research and development activities are performed, or the services are provided. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research and clinical trial costs. When determining the accruals, at the end of a reporting period, the Company analyzes progress of its studies and clinical trials, including the phase or completion of events, invoices received and contracted costs. Actual results could differ from the Company's estimates.

Stock-Based Compensation

The Company accounts for stock-based compensation awards in accordance with ASC Topic 718, *Compensation – Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations based on their fair values. All of the stock-based awards are subject only to service-based vesting conditions. Management estimates the fair value of the stock option awards using the Black-Scholes option pricing model, which requires the input of assumptions, including (a) the fair value of the Company's common stock, (b) the expected stock price volatility, (c) the calculation of expected term of the award, (d) the risk-free interest rate and (e) expected dividends. Management estimates the fair value of the restricted stock awards using the fair value of the Company's common stock. Forfeitures are recognized as they are incurred.

Prior to the reverse merger, the Company periodically estimated the fair value of the Company's common stock considering, among other things, valuations of its common stock prepared by management with the assistance of a third-party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation.

Following the reverse merger, the fair value of the Company's common stock is based on the closing stock price on the date of grant as reported on the Nasdaq Global Market. The expected life of the stock options in years is estimated using the "simplified method," as prescribed in SEC's Staff Accounting Bulletin (SAB) No. 107, as the Company has no historical information from which to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior for its stock option grants. The simplified method is the midpoint between the vesting period and the contractual term of the option. For stock price volatility, the Company uses comparable public companies as a basis for its expected volatility to calculate the fair value of option grants. The risk-free rate is based on the U.S. Treasury yield curve commensurate with the expected life of the option. The expected dividend yield is zero as the Company has no history of paying dividends and no plans to do so in the near term.

The Company classifies stock-based compensation expense in its consolidated statement of operations and comprehensive loss in the same manner of the award recipient's payroll costs.

Leases

The Company recognizes right-of-use assets and lease liabilities for leases with original lease terms greater than one year based on the present value of lease payments over the lease term using its incremental borrowing rate on a collateralized basis. Short-term leases, with original lease terms of less than one year, are not recognized on the Consolidated Balance Sheets. The Company leases one office space. The Company is subject to the office lease offering a renewal option to extend the original lease term. The renewal option is included or excluded from right-of-use asset and lease liability based on the Company's assessment of the probability that the options will be exercised. See Note 11 Operating Lease Commitments.

Government Grants

The Company recognizes grants from governmental agencies when there is reasonable assurance that the Company will comply with the conditions attached to the grant arrangement and the grant will be received. The Company evaluates the conditions of each grant as of each reporting period to evaluate whether the Company has reached reasonable assurance of meeting the conditions of each grant arrangement and that it is expected that the grant will be received as a result of meeting the necessary conditions. Grants are recognized in the consolidated statements of operations on a systematic basis over the periods in which the Company recognizes the related costs for which the government grant is intended to compensate. Specifically, grant income related to research and development costs is recognized as such expenses are incurred. Grant income is recorded as a reduction of research and development costs in the consolidated statements of operations. In September 2024, the Company received a grant award notice from the Department of Health and Human Services in connection with its ongoing Phase 3 clinical trial, SELVA. For the year ended December 31, 2025 and 2024, the Company recognized $1.0 million and $0.1 million, respectively, of grant income as a reduction to research and development costs in the accompanying consolidated statements of operations. The Company received $0.5 million of grant proceeds from the Department of Health and Human Services in May 2025 and $0.6 million in October 2025.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the consolidated financial statements or tax returns. Deferred income tax assets and liabilities are determined based on differences between the financial statement reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. Deferred income tax assets are reduced, as necessary, by a valuation allowance when management determines it is more likely than not that some or all the tax benefits will not be realized. The effect on deferred income tax assets and liabilities of a change in tax rates is recognized in the period that such tax rate changes are enacted.

The Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not to be sustained upon examination based on the technical merits of the position. The amount of the accrued liability for which an exposure exists is measured as the largest amount of benefit determined on a cumulative probability basis that the Company believes is more likely than not to be realized upon ultimate settlement of the position. The Company has elected to treat interest and penalties, to the extent they arise, as a component of income taxes.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. The OBBBA includes significant provisions, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. The Company has evaluated the OBBBA enacted during the year and estimated its impact on the consolidated financial statements to be immaterial. The Company will continue to evaluate the full impact of these legislative changes as additional guidance becomes available.

German Research and Development Tax Credit Receivable

The Company recognizes income associated with research and development ("R&D") tax credits when the receipt of the R&D tax credit becomes probable. The Company evaluates the conditions of each R&D tax credit as of each reporting period to evaluate whether the Company has reached reasonable assurance of meeting the conditions of each R&D tax credit and that it is expected that the R&D tax credit will be received as a result of meeting the necessary conditions. R&D tax credits are recognized as a component of other income in the consolidated statements of operations once it becomes probable that the amounts will be received. Specifically, income related to the receipt of R&D tax credits is not recorded until it is probable that amounts will be received.

Related Party Transactions

The Company's board of directors reviews and approves transactions with directors, officers, and holders of 5% or more of its voting securities and their affiliates, each a related party. The material facts as to the related party's relationship or interest in the transaction are disclosed to its board of directors prior to their consideration of such transaction, and the transaction is not considered approved by its board of directors unless a majority of the directors who are not interested in the transaction approve the transaction.

Segments

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the chief operating decision maker ("CODM"), in deciding how to allocate resources to an individual segment and in assessing performance. The Company's CODM is its chief executive officer. The Company has determined it operates in one segment.

The Company follows ASU No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* ("ASU 2023-07") which applies to public entities with a single reportable segment and requires disclosures about each reportable segments' significant expenses and other segment items on an interim and annual basis. See Note 14 for related disclosures.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock and if-converted methods. For purposes of the diluted net loss per share calculation, preferred stock, stock options and warrants are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. For the years ended December 31, 2025 and 2024, basic and diluted net loss per share are the same.

Recently Adopted Accounting Standards

In December 2023, the FASB issued ASU No. 2023-09, *Improvements to Income Tax Disclosures (Topic 740)* ("ASU 2023-09"). The ASU requires disaggregated information about a reporting entity's effective tax rate reconciliation as well as additional information on income taxes paid. The ASU is effective on a prospective basis for annual periods beginning after December 15, 2024 and allows for adoption on a prospective or retrospective basis. The Company adopted this ASU for 2025 annual period on a retrospective basis. Refer to Note 12 to the consolidated financial statements for the inclusion of the required disclosures.

Recently Issued (Not Yet Adopted) Accounting Standards

In November 2024, the Financial Account Standards Board ("FASB") issued ASU 2024-03, *Income Statement – Disaggregation of Income Statement Expenses (DISE)*, which requires disaggregated disclosure of income statement expenses for public business entities ("PBEs"). The ASU does not change the expense captions an entity presents on the face of the income statement; rather, it requires disaggregation of certain expense captions into specified categories in disclosures within the footnotes to the consolidated financial statements. ASU 2024-03 is effective for all PBEs for

fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027. The Company is still evaluating the impacts the ASU has on its consolidated financial statements.

In December 2025, the FASB issued ASU No. 2025-10, *Government Grants (Topics 832) - Accounting for Government Grants Received by Business Entities*, which provides authoritative guidance for the recognition, measurement and presentation of government grants received by a business entity. The standard is effective for fiscal years beginning after December 15, 2028 and early adoption is permitted. The Company is currently evaluating the potential impact of this standard on its consolidated financial statements.

Note 3. Reverse Merger

As discussed in Note 1, on the Closing Date, the Company consummated the previously announced Business Combination with Legacy Palvella, as a result of which Legacy Palvella became a wholly-owned subsidiary of the Company. The Reverse Merger was contemplated and consummated, along with the PIPE Financing (defined below), to generate capital resources to support the advancement of the Company's pipeline and future operations.

At the consummation of the Merger Agreement on the Closing Date (the "Effective Time"), or immediately prior to where indicated, the following occurred:

- All of the then outstanding shares of Legacy Palvella common stock were converted into 1,770,167 shares of the Company's common stock, based on the exchange ratio of approximately 0.309469242 (the "Exchange Ratio").
- All of the then outstanding shares of the Legacy Palvella's convertible preferred stock were converted into 4,753,650 shares of the Company's common stock, based on the Exchange Ratio. Refer to Note 8, *Convertible Preferred Stock*, for further detail on the conversion of the Company preferred stock.
- All outstanding 15,617 shares of Pieris preferred stock remained outstanding through the completion of the Reverse Merger, with no changes to their terms and conditions.
- All of the then outstanding Convertible Notes of Legacy Palvella plus accrued interest were converted into 1,179,163 shares of the Company's common stock and 168,503 prefunded warrants based on the Exchange ratio.
- All of the then outstanding stock options of Legacy Palvella were exchanged for options to purchase common stock of the Company, subject to the Exchange Ratio.

While the Company was the legal acquirer of Legacy Palvella in the business combination, for accounting purposes, the Reverse Merger is treated as a reverse recapitalization whereby Legacy Palvella is deemed to be the accounting acquirer, and the historical financial statements of Legacy Palvella became the historical consolidated financial statements of the Company upon the closing of the Reverse Merger. Under this method of accounting, the Company was treated as the "acquired" company and Legacy Palvella was treated as the acquirer for financial reporting purposes. Accordingly, for accounting purposes, the Reverse Merger was treated as the equivalent of Legacy Palvella issuing stock for the net assets of the Company, accompanied by a recapitalization.

Based on the following factors, the Company determined under the ASC 805, *Business Combinations*, that the Reverse Merger should be accounted for as a reverse recapitalization:

- The Company stockholders owned approximately 60% of the voting rights in the Company and thus had sufficient voting rights to exert influence over the Company.
- The Company designated a majority of the Company's board of directors and maintained a majority of the composition of management.
- At the time of Closing, Pieris did not meet the definition of a business and had nominal assets, meeting the definition of a public shell company. As such, the Reverse Merger was treated as a reverse recapitalization in which Palvella is issuing stock for the net assets of Pieris.

The following table summarizes the fair value of identifiable assets acquired and liabilities assumed as part of the recapitalization (in thousands):

	As of December 13, 2024
Assets	
Current assets:	
Cash	$ 13,781
Accounts receivable	377
Prepaid expenses and other current assets	595
Total current assets	14,753
Total assets	$ 14,753
Liabilities	
Current liabilities:	
Accounts payable	$ 142
Accrued expenses and other current liabilities	3,000
Total current liabilities	3,142
Total liabilities	$ 3,142
Net assets acquired	**$ 11,611**

In connection with the Reverse Merger, the Company incurred transaction costs of $2.5 million. The $2.5 million of transaction costs were initially recorded as deferred financing costs on the consolidated balance sheets and then were reclassified to offset to equity upon closing of the reverse merger.

PIPE Financing

Concurrently with the execution of the Merger Agreement on July 23, 2024, Pieris entered into a securities purchase agreement (the "Purchase Agreement") with certain investors, including BVF Partners, L.P., an existing stockholder of Pieris (the "PIPE Investors"), pursuant to which, among other things, on the Closing Date and immediately following the consummation of the Merger, the PIPE Investors purchased (either for cash or in exchange for the termination and cancellation of outstanding convertible promissory notes issued by Legacy Palvella), and the Company issued and sold to the PIPE Investors, (i) 3,168,048 shares of common stock and (ii) Pre-Funded Warrants, exercisable for 2,466,456 shares of common stock, at a purchase price of $13.9965 per share or $13.9955 per Pre-Funded Warrant, which represents the per share purchase price of the common stock less the $0.001 per share exercise price for each Pre-Funded Warrant, for an aggregate purchase price of approximately $78.9 million, consisting of approximately $60.0 million in cash and the conversion of approximately $18.9 million of principal and interest under outstanding convertible notes issued by Legacy Palvella (the "PIPE Financing").

Contingent Value Rights Agreement

On December 13, 2024, immediately prior to the Effective Time, Pieris entered into a contingent value rights agreement (the "CVR Agreement") with a rights agent, pursuant to which holders of Pieris common stock prior to Closing received one non-transferable contingent value right (each, a "CVR") for each outstanding share of Pieris common stock held by such stockholder immediately prior to Closing. Each CVR represents the contractual right to receive payments upon the receipt of payments by Pieris or any of its affiliates under certain strategic partner agreements, including existing collaboration agreements pursuant to which Pieris may be entitled to milestones and royalties in the future and other out licensing agreements for certain of Pieris' legacy assets, and upon the receipt of certain research and development tax credits in favor of Pieris or any of its affiliates, in each case as set forth in, and subject to and in accordance with the terms and conditions of, the CVR Agreement. As no amounts related to the CVR Agreement were probable as of the time of the Reverse Merger, no contingencies for the CVR agreement had been recorded on the Closing Date.

Note 4. Fair Value Measurements

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	December 31, 2025			
	Level 1	Level 2	Level 3	Total
Current assets:				
Cash and cash equivalents	$ 57,982	$ —	$ —	$ 57,982
Total assets measured at fair value	$ 57,982	$ —	$ —	$ 57,982
Liabilities:				
Derivative liabilities – royalty agreement	$ —	$ —	$ 2,041	$ 2,041
Derivative liabilities – contingent value right liability	—	—	2,196	2,196
Total liabilities measured at fair value	$ —	$ —	$ 4,237	$ 4,237

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Current assets:				
Cash and cash equivalents	$ 83,602	$ —	$ —	$ 83,602
Total assets measured at fair value	$ 83,602	$ —	$ —	$ 83,602
Liabilities:				
Derivative liabilities – royalty agreement	$ —	$ —	$ 1,647	$ 1,647
Derivative liabilities – contingent value right liability	—	—	1,978	1,978
Total liabilities measured at fair value	$ —	$ —	$ 3,625	$ 3,625

Cash and cash equivalents as of December 31, 2025 and 2024 includes cash and investments in money market funds. Money market funds, which are cash equivalents, are highly liquid investments and are actively traded. The pricing information on the Company's money market funds is based on quoted prices in active markets for identical securities. This approach results in a classification of these securities as Level 1 of the fair value hierarchy.

Assumptions Used in Determining Fair Value of Derivative Liabilities

The key assumptions used to determine the fair value of the derivative liabilities – royalty agreement at December 31, 2025 and 2024 are as follows:

	December 31, 2025	December 31, 2024
Discount rate	18.0%	20.0%
Probability rate of achieving FDA approval of a product	56.6%	56.6%
Expected term to FDA regulatory approval of a product	1.50 years	2.50 years

Changes in Level 3 Liabilities Measured at Fair Value on a Recurring Basis

The following table provides a reconciliation of the liabilities measured at fair value on a recurring basis using significant unobservable inputs (Level 3) (in thousands):

Derivative Liabilities – Royalty Agreement

	2025	2024
Derivative liabilities - royalty agreement		
Balance at January 1	$ 1,647	$ 1,014
Fair value adjustments on derivative liabilities	394	633
Balance at December 31, 2025	$ 2,041	$ 1,647

The derivative liabilities – royalty agreement is classified as long term on the Company's consolidated balance sheets according to the estimated timing of the occurrence of the potential payments.

Derivative Liabilities – Contingent Value Right Liability

	2025	2024
Derivative liabilities - contingent value right liability		
Balance at January 1	$ 1,978	$ —
Fair value adjustments on derivative liabilities	218	1,978
Balance at December 31, 2025	$ 2,196	$ 1,978

The derivative liabilities – contingent value rights is classified as short term on the Company's consolidated balance sheets according to the estimated timing of the occurrence of the potential payments.

The Company applies a scenario-based method and weighs them based on the possible likelihood of certain contingencies triggering payments due under the CVR for the liability recognized. The fair value measurements are based on significant inputs not observable in the market and thus represent a Level 3 measurement as defined in ASC 820, *Fair Value Measurement*. The estimated value of the CVR is based upon available information and certain assumptions, which the Company's management believes are reasonable under the circumstances. As of December 31, 2025 and 2024, the fair value adjustment for the CVR is driven from foreign currency translation. The change in fair value has been recorded in fair value adjustments on derivative liabilities - contingent value right liability on the consolidated statement of operations.

Note 5. Strategic Agreements

Ligand Development Funding Agreement

The Company is party to a Development Funding and Royalties Agreement with Ligand Pharmaceuticals, Inc. ("Ligand"), dated December 13, 2018, as amended May 22, 2020 and November 28, 2023 (the "Ligand Agreement"). Under the Ligand Agreement, Ligand has made payments totaling $15.0 million to fund the development of QTORIN rapamycin. As partial consideration for the funding received, the Company granted Ligand the right to receive up to $8.0 million in milestone payments upon the achievement of certain corporate, financing and regulatory milestones by the Company related to QTORIN rapamycin for the treatment of any and all indications. In addition, the Company is currently obligated to pay to Ligand tiered royalties ranging from 8.0% to 9.8% of any aggregate annual worldwide net product sales of any products based on QTORIN rapamycin. On a licensed product-by-licensed product and country-by-country basis, the royalty period is from the date of first commercial sale of such licensed product in a country until the latest of (i) the expiration of the last valid claim within the licensed patent rights covering such licensed product in the country in which such licensed product is made, used or sold, (ii) the expiration of the regulatory exclusivity term conferred by the applicable regulatory authority in such country with respect to such licensed product, and (iii) the fifteenth anniversary of the first commercial sale of such licensed product in such country.

The Ligand Agreement may be terminated by the earlier of a mutual written agreement of the parties or when the royalties contemplated by the agreement are paid to Ligand. Additionally, Ligand may terminate the agreement for (i) any or no reason upon a 90-day notice to the Company, or (ii) cause in connection with a material breach that the Company does not cure within a certain period of time.

The total amount of potential future milestone payments remaining under the arrangement were $5.0 million as of December 31, 2025 and 2024. The potential future milestone payments represent derivative liabilities with a fair value of $2.0 million and $1.6 million as of December 31, 2025 and 2024, respectively, which are classified as "derivative liabilities – royalty agreement" on the accompanying consolidated balance sheets. See Note 4 for fair value measurements.

The Company's obligation to pay tiered royalties under the Ligand Agreement was determined to be a debt instrument based on the likelihood of repaying the amounts provided to fund the development of QTORIN rapamycin and that the Company has significant continuing involvement in the generation of the cash flows potentially due to Ligand. This obligation is reflected as royalty agreement liability which is classified as a long-term liability on the accompanying consolidated balance sheets and was $17.8 million and $11.9 million as of December 31, 2025 and 2024, respectively. Interest expense with respect to the royalty agreement liability is determined using the effective interest method based upon probability-adjusted cash flow estimates of the Company's potential future royalty payments under the Ligand Agreement, yielding an effective interest rate of 44.9% and 39.9% as of December 31, 2025 and 2024, respectively. Changes in these estimates impact the amount of interest expense recognized through the accompanying consolidated statements of operations. During the second quarter of 2024, the Company received data from certain of its clinical trials that reduced the projected net product sales related to QTORIN rapamycin and the corresponding probabilities of successful commercialization, resulting in a significant reduction in the future royalty agreement liability. In addition, in the fourth quarter of 2024, the Company began conducting a Phase 3 clinical trial in microcystic lymphatic malformations. In the fourth quarter of 2025, the Company reported positive topline data for its Phase 2 clinical trial for cutaneous venous malformations and increased the corresponding probability of successful commercialization. The Company incurred non-cash interest expense of $5.8 million and $3.9 million for the years ended December 31, 2025 and 2024, respectively, all of which is a component of the royalty agreement liability on the accompanying consolidated balance sheets.

The Ligand Agreement requires the Company to make certain estimates and assumptions about future development, FDA approval, commercialization, and net sales of any product containing QTORIN rapamycin. These estimates and assumptions are subject to significant variability and are thus subject to significant uncertainty. Therefore, these estimates and assumptions are likely to change as the Company develops and commercializes products containing QTORIN rapamycin that may result in significant future adjustments to the royalty agreement liability, the derivative liabilities, and the accretion of interest expense.

Note 6. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	December 31, 2025	December 31, 2024
Professional fees	$ 531	$ 808
Compensation expense (1)	1,915	3,073
Research and development expenses	1,200	404
Other	705	1,189
Total accrued expenses and other current liabilities	$ 4,351	$ 5,474

(1) Includes accrued severance, bonus, and retention payments of $2.1 million for current and former Pieris employees as of December 31, 2024.

Note 7. Convertible Notes Payable

To facilitate its ongoing operations, during the year ended December 31, 2024, the Company entered into the Convertible Notes, each with a maturity date of June 6, 2027 and interest rate of SOFR + 2.0%, as follows:

	Issuance Date	Original Issuance Amount
Note 1	6/4/2024	5,000,000
Note 2	6/26/2024	2,500,000
Note 3	6/26/2024	2,500,000
Note 4	7/17/2024	50,000
Note 5	7/17/2024	50,000
Note 6	7/19/2024	150,000
Note 7	7/19/2024	143,000
Note 8	7/19/2024	100,000
Note 9	7/19/2024	70,000
Note 10	7/19/2024	20,000
Note 11	7/22/2024	700,000
Note 12	7/22/2024	500,000
Note 13	7/22/2024	150,000
Note 14	8/20/2024	500,000
Note 15	12/10/2024	1,000,000
Note 16	12/6/2024	2,000,000
Note 17	12/6/2024	3,000,000
		$ 18,433,000

The convertible notes issued in 2024 contained a provision whereby the principal and accrued interest on the Convertible Notes would automatically convert into shares of the Company upon the occurrence of a Qualified Financing, defined as either the earlier to occur of a) issuance of shares of preferred stock resulting in aggregate gross proceeds of at least $20,000,000 or b) an initial public offering, in each case on or before the maturity date. As a result of the Reverse Merger, the principal and accrued interest of $18.4 million and $0.4 million, respectively, associated with the Convertible Notes were automatically converted into an aggregate of 1,179,163 shares of common stock and 168,503 prefunded warrants of the Company in accordance with this provision.

Note 8. Convertible Preferred Stock

In December 2022, the Company issued 1,835,227 shares of Series D Convertible Preferred Stock (“Series D Preferred”) at a price of $5.2879 per share. The Series D Preferred also contained a Milestone Closing option for additional shares to be issued following the Company’s receipt of clinical data for the top line Phase 3 data results for QTORIN rapamycin for pachyonychia congenita. Under the Milestone Closing, each Purchaser of the Series D Preferred had the right to purchase, and the Company agreed to sell and issue to each Purchaser at the Milestone Closing, up to that portion of 4,727,775 shares of Series D Preferred which equaled the proportion that the number of shares of Series D Preferred then held by such Purchaser beared to the total number of shares of Series D Preferred outstanding immediately prior to the Milestone Closing, at a purchase price of $5.2879 per share. The Company determined that the future tranche right to purchase additional shares of Series D Preferred was not a freestanding financial instrument as it was not separately exercisable and legally detachable. The future tranche right was evaluated as an embedded derivative and was not bifurcated from the Series D Preferred shares since it did not have a net settlement characteristic and therefore did not meet the definition of a derivative.

In connection with the issuance of the Series D Preferred, the Company amended and restated its certificate of incorporation (as amended, the “Amended Certificate”) such that it was authorized to issue 29,000,000 shares of common stock (25,500,000 voting and 3,500,000 non-voting) and 20,655,895 shares of preferred stock, with 2,241,903 shares designated as Series A-1 Convertible Preferred stock (“Series A-1 Preferred”), 1,240,134 shares designated as Series A-2 Convertible Preferred stock (“Series A-2 Preferred”), 1,533,528 shares designated as Series B Convertible Preferred stock (“Series B Preferred”), 8,509,995 shares designated as Series C Convertible Preferred stock (“Series C Preferred”) and 7,130,335 shares designated as Series D Preferred.

The Company's Series A-1, Series A-2 and Series B, Series C, and Series D preferred stock (collectively, "convertible preferred stock") was subject to certain pre-existing anti-dilution provisions that were triggered upon the closing of the PIPE Financing. The private placement in the Company closed immediately before the Closing. At the effective time of the Reverse Merger, each issued and outstanding share of the Company convertible preferred stock converted automatically into 0.309469242 shares of the Company's common stock, at the option of the preferred shareholder. At the Closing, the Company issued an aggregate of 4,753,650 shares of its common stock to the Company's preferred shareholders.

Note 9. Stockholders' Equity

Upon closing of the Reverse Merger, pursuant to the terms of the Amendment to the Company's Amended and Restated Articles of Incorporation with the Nevada Secretary of State, the Company was authorized to issue up to 200,000,000 shares of common stock, par value $0.001 per share ("Common Stock"). As of December 31, 2025 and 2024, 12,380,933 and 11,012,105 shares of Common Stock were issued and outstanding, respectively.

In connection with the Reverse Merger, 15,617 shares of Preferred Stock were issued and convert on a factor of 13.34 common shares for each preferred share:, and consisted of the following as of December 31, 2024:

- Series A Convertible, 85 shares issued and outstanding
- Series B Convertible, 4,026 shares issued and outstanding
- Series C Convertible, 3,506 shares issued and outstanding
- Series D Convertible, 3,000 shares issued and outstanding
- Series E Convertible, 5,000 shares issued and outstanding

In October 2025, 15,617 shares of Preferred Stock were converted into 208,324 shares of Common Stock. No shares of Preferred Stock were outstanding as of December 31, 2025.

Common Stock, Preferred Stock, and Pre-Funded Warrants

Each share of the Company's common stock is entitled to one vote and all shares rank equally as to voting and other matters. Dividends may be declared and paid on the common stock from funds legally available therefor, if, as and when determined by the Board of Directors.

The Company has pre-funded warrants outstanding to purchase an aggregate of 1,394,780 shares of common stock as of December 31, 2025. In October 2025, the Company issued to entities affiliated with BVF 535,837 shares of Common Stock upon the exercise of pre-funded warrants and issued to entities affiliated with BVF an additional 535,839 shares of Common Stock upon the exercise of pre-funded warrants in December 2025. The pre-funded warrants are exercisable at any time for an exercise price of $0.001, except that the pre-funded warrants cannot be exercised by the holders if, after giving effect thereto, the holders would beneficially own more than 9.99% of the outstanding common stock, subject to certain exceptions. However, any holder may increase or decrease such percentage to any other percentage (not in excess of 19.99%) upon at least 61 days' prior notice from the holder to the Company. The holders of the pre-funded warrants will not have the right to vote the shares underlying the pre-funded warrants on any matter except to the extent required by Delaware law. These warrants were classified as equity.

Prior to the Reverse Merger, Legacy Pieris had issued multiple series (Series A through E) of Preferred Stock to certain entities affiliated with Biotechnology Value Fund, L.P., or BVF. In each case, each share Preferred Stock is convertible into 13.34 shares of the Common Stock (subject to adjustment as provided in the Certificate of Designation for each series) at any time at the option of the holder, provided that the holder is prohibited from converting the Preferred Stock into shares of Common Stock if, as a result of such conversion, the holder, together with its affiliates, would own more than 9.99

% of the total number of shares of Common Stock then issued and outstanding, or the Beneficial Ownership Limitation. The holder may reset the Beneficial Ownership Limitation to a higher or lower number, not to exceed 19.99% of the total number of common shares issued and outstanding immediately after giving effect to a conversion, upon providing written notice to the Company. Any such notice providing for an increase to the Beneficial Ownership Limitation will be effective 61 days after delivery to the Company.

Series A, Series B, Series C, Series D and Series E Preferred Stock rank senior to Common Stock; senior to any class or series of capital stock of the Company created after the designation of and specifically ranking by its terms as junior to the five series of Preferred Stock; in parity with each other and with any class or series of capital stock of the Company created after the designation of and specifically ranking by its terms as in parity with the existing five series of Preferred Stock; and junior to any class or series of capital stock of the Company created after the designation of and specifically ranking by its terms as senior to the existing five series of Preferred Stock. In the event of the Company's liquidation, dissolution or winding up, subject to the rights of holders of Preferred Stock, holders are entitled to receive a payment equal to $0.001 per share of Preferred Stock pursuant to the rights and preferences discussed above, plus an additional amount equal to any dividends declared but unpaid on such shares. However, if the assets of the Company are insufficient to comply with the preceding sentence, then all remaining assets of the Company shall be distributed ratably to holders of the shares of the existing five series of Preferred Stock.

For each series of Preferred Stock, the Company designated the requisite number of shares of its authorized and unissued preferred stock as a specific series of Preferred Stock and filed a Certificate of Designation with the Nevada Secretary of State.

Shares of Preferred Stock generally have no voting rights, except as required by law and except that the consent of holders of a majority of the then outstanding Preferred Stock is required to amend the terms of the Certificate of Designation for each respective series of Preferred Stock. Holders of Preferred Stock are entitled to receive any dividends payable to holders of the Company's common stock subject to the rights and preferences discussed above, in each case, as to distributions of assets upon the Company's liquidation, dissolution or winding up whether voluntarily or involuntarily and/or the right to receive dividends.

Note 10. Equity Incentive Plans

In connection with the Reverse Merger, the Company stockholders approved the 2024 Equity Incentive Plan (the "2024 Plan") on December 13, 2024. The 2024 Plan provides for the grant of incentive stock options, nonqualified stock options, and stock awards, any of which may be performance-based, and for incentive bonuses, which may be paid in cash, Common Stock or a combination thereof.

The number of shares reserved for issuance under the 2024 Plan is equal to 3,455,433 shares of Common Stock.

As of December 31, 2025, 2,322,854 shares of Common Stock were issued under the 2024 Plan.

In connection with the Reverse Merger, all of the options outstanding under the Company's 2019 Equity Incentive Plan (the "2019 Plan") were adjusted with respect to the number of shares and exercise price to reflect the Exchange Ratio. As of December 31, 2025, 576,244 shares of Common Stock were outstanding under the 2019 Plan and no further grants will be made under the 2019 Plan.

For incentive stock options and non-statutory stock options, the option exercise price may not be less than 100% of the estimated fair value on the date of grant. Options granted to employees typically vest over a four-year period but may be granted with different vesting terms. The options expire ten years from the grant date.

The following table summarizes stock option activity for the Company's equity incentive plans for the year ended December 31, 2025:

	Common Shares Underlying Stock Options	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Life (in years)
Outstanding at January 1, 2024	489,283	$ 8.79	7.3
Granted	1,184,069	$ 12.70	
Exercised	—		
Forfeited / Cancelled	—		
Outstanding at December 31, 2024	1,673,352	$ 11.55	8.6
Granted	1,362,389	$ 28.31	
Exercised	(88,828)	$ 8.59	
Forfeited / Cancelled	(47,800)	$ 13.60	
Outstanding at December 31, 2025	2,899,113	$ 19.49	8.6
Exercisable at December 31, 2025	844,567	$ 11.31	7.4

The aggregate intrinsic value of options outstanding and options exercisable as of December 31, 2025 was $131.2 million and $43.4 million, respectively.

Total stock-based compensation expense recognized in the consolidated statements of operations for the years ended December 31, 2025 and 2024 is as follows (in thousands):

	Year Ended December 31,	
	2025	2024
Research and development	$ 2,226	$ 569
General and administrative	4,175	261
Total stock-based compensation expense	$ 6,401	$ 830

As of December 31, 2025, there was approximately $30.1 million of unrecognized compensation cost related to non-vested stock options, which is expected to be recognized over a remaining weighted-average service period of 2.89 years.

The weighted average fair value of stock options granted during the year ended December 31, 2025 was $28.31 per share which was estimated using the Black-Scholes option pricing model with the following weighted average assumptions:

Expected volatility	72.00% – 76.00%
Risk-free interest rate	3.72% – 4.47%
Expected term (years)	5.50 – 6.08
Expected dividend yield	—

Note 11. Operating Lease Commitments

The Company entered into an operating lease arrangement that commenced on October 1, 2025 with unrelated parties for office space located in Wayne, PA. The lease expires on September 30, 2028, and the Company has the option of extending the lease for two additional one-year terms. The Company did not include options to extend its lease terms as part of its right-of-use asset and lease liabilities. The lease is subject to additional variable charges, including common area maintenance and property insurance. Given the variable nature of such costs, they are recognized as expense as incurred. Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease right-of-use assets and lease liabilities are recognized based on the present value of lease payments over the lease term.

The Company has recorded right-of-use assets of $0.6 million and lease liabilities of $0.6 million for its operating lease on the consolidated balance sheet as of December 31, 2025.

The following are the components of lease cost recognized within general and administrative expense on the consolidated statements of operations and comprehensive loss for the year ended December 31, 2025:

	December 31, 2025
Operating lease cost	$ 61
Variable lease cost	12
Short-term lease cost	53

As of December 31, 2025, the weighted average remaining lease life and weighted average discount rate were as follows:

	December 31, 2025
Operating leases	
Weighted average remaining lease term (years)	2.75
Weighted average discount rate	11.58%

As of December 31, 2025, the future minimum rental payments due under non-cancelable leases are as follows:

	Operating Leases
Fiscal years ending December 31,	
2026	$ 262
2027	269
2028	205
2029	-
2030	-
Thereafter	-
Total gross minimum lease payments	736
Less: imputed interest	(103)
Subtotal	633
Less: current portion	(202)
Long-term portion of lease liability	$ 431

As of December 31, 2025, the Company had no leases that had not yet commenced. There were no material leases during the year ended December 31, 2024.

Note 12. Income Taxes

The Company reported a loss before income taxes consisting of the following (in thousands):

	December 31,	
	2025	2024
Domestic	$ (41,779)	$ (17,434)
Foreign	64	—
Loss before income taxes	$ (41,715)	$ (17,434)

The Company had no provision for income taxes for the years ended December 31, 2025 and 2024.

A reconciliation of the statutory U.S. federal income tax rate to the Company's effective tax rate is as follows:

	December 31,			
	2025		2024*	
U.S. federal statutory rate	$ (8,760)	21.0%	$ (3,661)	21.0%
Foreign tax effects	4	—	—	—
Tax credits	(564)	1.4	—	—
Change in valuation allowance	8,582	(20.6)	3,893	(22.3)
Nontaxable or nondeductible items	330	(0.8)	(229)	1.3
Other adjustments	408	(1.0)	(3)	—
Effective income tax rate	$ —	0.0%	$ —	0.0%

*While the underlying tax effects remain unchanged from the prior year, certain reconciling items have been reallocated consistent with the required ASU 2023-09 disclosure.

The components of deferred tax assets and liabilities related to net tax effects of temporary differences between the carrying amount of assets and liabilities for financial reporting purposes and the amounts used for income taxes purposes were as follows (in thousands):

	December 31,	
	2025	2024
Deferred tax assets:		
Royalty agreement liabilities	$ 5,415	$ 3,913
Net operating loss	91,962	81,971
Section 174 R&D capitalization	5,797	6,288
Stock-based compensation	1,791	419
Accrued expenses and other	690	744
Other tax credits	589	24
Orphan drug credit	199	199
Lease liabilities	158	—
Startup costs	76	86
Deferred tax assets before valuation allowance	$ 106,677	$ 93,644
Deferred tax liabilities		
Right of use assets	$ (142)	$ —
Other deferred tax liabilities	(400)	(390)
Net deferred tax	106,135	93,254
Valuation allowance	(106,135)	(93,254)
Net deferred tax assets	$ —	$ —
(Increase) decrease in valuation allowance	$ (12,881)	$ (77,240)

In assessing the realizability of the net deferred tax asset, the Company considers all relevant positive and negative evidence in determining whether it is more likely than not that some portion or all of the deferred income tax assets will not be realized. The realization of the gross deferred tax assets is dependent on several factors, including the generation of sufficient taxable income prior to the expiration of the NOLs. Management believes that it is more likely than not that the Company's deferred income tax assets will not be realized. As such, there is a full valuation allowance in the amount of $106.1 million and $93.3 million against the net deferred tax assets as of December 31, 2025 and 2024, respectively. The increase in the valuation allowance of deferred tax assets as of December 31, 2025 was primarily a result of operating losses.

The Company recognizes interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2025 and 2024, the company reported no liabilities for unrecognized tax benefits along with no related interest and penalty exposure as accrued income tax on the accompanying consolidated balance sheets. Income tax returns for the tax years 2019 through 2025 remain subject to examination by the taxing authority jurisdictions.

As of December 31, 2025, the Company had net operating loss carryforwards for U.S. federal income tax purposes of $129.4 million and net operating loss carryforwards for state income tax purposes of $113.3 million. Federal tax loss carryforwards that were created prior to December 31, 2017 expire through 2037 and federal losses created after that date do not expire. State loss carryforwards expire starting in 2035. In addition, the Company has orphan drug credits of $0.2 million to reduce future federal taxes through 2039. Pursuant to Section 382 of the Internal Revenue Code of 1986, or the Code, and similar state tax law, certain substantial changes in the Company's ownership may result in a limitation on the amount of net operating loss and tax credit carryforwards that may be used in future years. Utilization of the net operating loss and tax credit carryforwards may be subject to an annual limitation under Section 382 of the Code due to ownership change limitations that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating loss and tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. The Company completed a Section 382 study through December 31, 2020. Based on the study, the Company underwent an ownership change for Section 382 purposes which occurred in February 2018. As a result of the ownership change, the Company's net operating loss and tax credit carryforwards as of the ownership change dates are subject to limitation under Section 382; however, these limitations are not expected to result in any of the impacted net operating loss and tax credit carryforwards to expire unutilized. Any net operating losses or tax credits generated after the February 2018 change are not subject to this annual limitation. However, subsequent ownership changes, as defined by Section 382, may potentially further limit the amount of net operating loss and tax credit carryforwards that could be utilized to offset future taxable income and tax. The Company is currently open to examination under the statute of limitations by the Internal Revenue Service and state jurisdictions for the tax years ended 2019 through the current year. Carryforward tax attributes generated in years past may still be adjusted upon future examination if they have or will be used in a future period. The Company is not under examination by the Internal Revenue Service or any other jurisdictions for any tax years.

As of December 31, 2025, the Company had German corporate income tax and trade tax net operating loss carryforwards of approximately $219.4 million and $215.1 million, respectively. Under current German laws, tax loss carryforwards may only be used to offset any relevant later assessment period (calendar year) of $1.2 million plus 60% of the exceeding taxable income and trade profit of such period and do not expire. In addition, certain transactions, including transfers of shares or interest in the loss holding entity, may result in the partial or total forfeiture of tax losses existing at that date. Partial or total forfeiture of tax losses may further occur in corporate reorganizations of the loss holding entity.

Note 13. Commitments and Contingencies

From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a liability for such matters when future expenditures are probable and such expenditures can be reasonably estimated.

Legal Proceedings

From time to time, the Company may face legal claims or actions in the normal course of business. The Company is not currently subject to any material legal proceedings.

Note 14. Segment Information

The Company is comprised of one reportable segment, the Company operations. As of December 31, 2025, the Company operations segment has not generated any product revenue since inception, as it does not yet have approved products for sale. However, the Company operations segment anticipates future revenue generation upon the successful development and commercialization of product candidates either independently or through partnerships.

The Company expects to primarily generate revenue in North America, with its long-lived assets also concentrated in this region, and manages its business activities on a consolidated basis. Decisions concerning the allocation of the Company's resources are made by the Company's Chief Operating Decision Maker (CODM), which is the Company's

Chief Executive Officer (CEO). The CODM views the Company's operations as a single operating segment which is the business of discovering and developing products for individuals with serious and rare skin diseases.

The Company's significant segment expenses that are regularly provided to the CODM are related to:

- *Research and Development Expenses*: These expenses include costs incurred in conducting research and development activities for the specific clinical trials including facilities costs, license fee and other costs incurred in connection with preclinical research and development activities. Research and development also encompass non-program specific costs such as salaries and stock-based compensation costs, third party consulting fees, and other related costs.
- *General and Administrative Expenses*: These expenses include costs associated with the overall administration and management of the Company, including salaries and benefits for administrative personnel, professional service fees (e.g., accounting and legal services) and other office expenses.

The CODM assesses performance for the Company operations segment and decides how to allocate resources based on net income or loss that is also reported on the income statement as consolidated net income or loss and the measure of segment assets as reported on the balance sheet as total consolidated assets. Net income or loss is used to monitor budget versus actual results as well as assess the general performance of the segment in a given period.

The following table reconciles segment direct profit or loss to the Company's consolidated results:

	Year Ended December 31,	
(in thousands)	**2025**	**2024**
Research and development:		
QTORIN rapamycin for microcystic LM	$ 4,755	$ 1,929
QTORIN rapamycin for microcystic LM - Government grant income	(987)	(141)
QTORIN rapamycin for cutaneous VM	2,224	296
QTORIN CMC	6,953	1,601
Non-program specific and unallocated research and development expenses:		
Salaries and stock-based compensation	6,643	2,810
Consultants	1,910	1,040
Other	1,343	616
Total research and development	$ 22,841	$ 8,151
General and administrative:		
Salaries and stock-based compensation	$ 7,827	$ 1,935
Consultants	3,285	$ 2,961
Other	4,650	$ 1,048
Total general and administrative	$ 15,761	$ 5,944
Loss from operations	$ (38,602)	$ (14,095)

Note 15. Subsequent Events

In January 2026, the Company paid out approximately $2.0 million to holders of CVRs in accordance with the CVR Agreement entered into immediately prior to closing of the Merger on December 13, 2024.

On February 25, 2026, the Company entered into an underwriting agreement in connection with its previously announced underwritten public offering of 1,600,000 shares of its Common Stock, par value $0.001 per share, at a price to the public of $125.00 per share. Under the terms of the underwriting agreement, the Company granted the underwriters an option to purchase up to an additional 240,000 shares of Common Stock at the same price. The option was exercised and the offering closed on February 27, 2026.

The offering resulted in net proceeds of approximately $215.8 million, after deducting underwriting discounts and commissions and other offering expenses.

Exhibit 4.1

PALVELLA THERAPEUTICS, INC.
DESCRIPTION OF SECURITIES

The following description of the capital stock of Palvella Therapeutics, Inc. (the "Company," "we," "us," and "our") and provisions of our Amended and Restated Articles of Incorporation, as amended (the "Articles of Incorporation"), and Amended and Restated Bylaws, as amended (the "Bylaws"), are summaries and are qualified in their entirety by reference to such Articles of Incorporation and Bylaws and applicable provisions of Nevada law, including Chapters 78 and 92A of the Nevada Revised Statutes (the "NRS"). Copies of these documents are filed with the SEC as exhibits to our periodic filings.

General

Our authorized capital stock consists of 200,000,000 shares of our common stock, par value $0.001 per share, and 10,000,000 shares of our preferred stock, par value $0.001 per share, of which 85 shares are designated as Series A Convertible Preferred Stock, 4,026 shares are designated as Series B Convertible Preferred Stock, 3,506 shares are designated as Series C Convertible Preferred Stock, 3,000 shares are designated as Series D Convertible Preferred Stock, and 5,000 shares are designated as Series E Convertible Preferred Stock.

Common Stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of our stockholders and do not have cumulative voting rights. Holders of our common stock are entitled to receive any dividends as may be declared by our board of directors, subject to any preferential dividend or other rights of any then outstanding preferred stock.

Voting Rights

For all matters submitted to a vote of stockholders, each holder of our common stock is entitled to one vote for each share registered in the holder's name on our books. At all meetings of the stockholders, except where otherwise provided by applicable law, rules of any stock exchange upon which our securities are listed, our Articles of Incorporation or our Bylaws, the presence, in person, virtually or by duly authorized proxy, of the holders of a majority of the outstanding shares of our capital stock entitled to vote constitutes a quorum for the transaction of business. Except as otherwise provided by applicable law or by our Articles of Incorporation or our Bylaws, all matters, other than the election of directors, proposed at any meeting of the stockholders shall be determined by a majority of the votes cast affirmatively or negatively. Except as otherwise provided by law, our Articles of Incorporation, our Bylaws or the terms of any class or series of our preferred stock, directors are elected by a plurality of the votes cast by the holders of the shares of our common stock present virtually or by proxy at the meeting and entitled to vote generally on the election of directors.

Dividends

Subject to limitations under Nevada law, any provision of our Articles of Incorporation and any preferences that may be applicable to any then outstanding preferred stock, holders of our common stock are entitled to receive ratably those dividends, if any, as may be declared by our board of directors, in their sole discretion, out of legally available funds.

Liquidation

Upon our liquidation, dissolution or winding up, the holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all our debts and other liabilities of the company, subject to any prior rights of any preferred stock then outstanding.

Other Rights and Restrictions

Holders of our common stock do not have preemptive or subscription rights, and they have no right to convert our common stock into any other securities. There is no redemption or sinking fund provisions applicable to our

common stock. The rights, preferences and privileges of common stockholders are subject to the rights of the stockholders of any series of our preferred stock, including any which we may designate in the future. Our Articles of Incorporation and Bylaws do not restrict the ability of a holder of our common stock to transfer the holder's shares of our common stock.

Listing

Our common stock is listed on the Nasdaq Capital Market under the symbol "PVLA."

Transfer Agent and Registrar

The transfer agent and registrar for Palvella common stock is Computershare Investor Services, LLC, P.O. Box 43006, Providence, RI 02940-3078, telephone number: 1-877-373-6374.

Preferred Stock

Under the terms of our Articles of Incorporation, the Board of Directors of the Company (the "Board") is authorized to issue up to 10,000,000 shares of preferred stock in one or more series without stockholder approval by adopting a resolution or resolutions providing for the issuance of the shares thereof and by filing a certificate of designation relating thereto in accordance with the NRS. The Board has the discretion to determine the designations, rights, preferences, privileges and restrictions, including voting powers (full, limited or no voting powers), dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The authorization of the Board to issue preferred stock and determine the rights and preferences of that preferred stock eliminates delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock.

Anti-Takeover Effects of Nevada Law and Articles of Incorporation and Bylaws.

Nevada law and our Articles of Incorporation and Bylaws contain provisions that could have the effect of delaying, deferring or discouraging another party from acquiring control of Palvella. These provisions, which are summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of Palvella to first negotiate with the Board.

Staggered Board; Removal of Directors. Our Articles of Incorporation divides the Board into three classes with staggered three-year terms. In addition, a director may only be removed for cause and only by the affirmative vote of the holders of at least 80% of the votes that all of our stockholders would be entitled to cast in an annual election of directors. Any vacancy or newly-created directorship on the Board may only be filled by vote of a majority of our directors then in office, even though less than a quorum, or by a sole remaining director, and not by stockholders. The classification of the Board and the limitations on the removal of directors and filling of vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of Palvella.

No Stockholder Action by Written Consent; Special Meetings. Our Articles of Incorporation and Bylaws provide that any action required or permitted to be taken by Palvella stockholders must be effected at a duly called annual or special meeting of such holders and may not be effected by any consent in writing by such holders. Our Articles of Incorporation and Bylaws also provide that, except as otherwise required by law, special meetings of Palvella stockholders can only be called by the Board.

Advance Notice Requirements for Stockholder Proposals. Our Bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of Palvella stockholders, including proposed nominations of persons for election to the Board. Stockholders at a Palvella annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of the Board or by a Palvella stockholder of record on the record date for the meeting who is entitled to vote at the meeting

and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Amendment of Our Articles of Incorporation and Bylaws. The NRS provides generally that the affirmative vote of a majority of the outstanding shares entitled to vote thereon is required to amend a corporation's articles of incorporation, unless a corporation's articles of incorporation require a greater percentage. Our Bylaws may be amended or repealed by a majority vote of the Board or by the affirmative vote of the holders of at least 80% of the votes that all Palvella stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 80% of the votes that all Palvella stockholders would be entitled to cast in any annual election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our Articles of Incorporation described above under "-*Staggered Board; Removal of Directors*" and "-*No Stockholder Action by Written Consent; Special Meetings*," and under this section " -*Amendment of Our Articles of Incorporation and Bylaws*."

Acquisitions, Business Combinations and Change in Control. The NRS contains provisions governing the acquisition of a controlling interest in certain Nevada corporations. Nevada's "acquisition of controlling interest" statutes (NRS 78.378 through 78.3793, inclusive) contain provisions governing the acquisition of a controlling interest in certain Nevada corporations. These "control share" laws provide generally that any person that acquires a "controlling interest" in certain Nevada corporations may be denied voting rights, unless a majority of the disinterested stockholders of the corporation elect to restore such voting rights. These laws would apply to us as of a particular date if we were to have 200 or more stockholders of record (at least 100 of whom have addresses in Nevada appearing on our stock ledger at all times during the 90 days immediately preceding that date) and do business in the State of Nevada directly or through an affiliated corporation, unless our Articles of Incorporation or Bylaws in effect on the tenth day after the acquisition of a controlling interest provide otherwise. These laws provide that a person acquires a "controlling interest" whenever a person acquires shares of a subject corporation that, but for the application of these provisions of the NRS, would enable that person to exercise (1) one-fifth or more, but less than one-third, (2) one-third or more, but less than a majority, or (3) a majority or more of all of the voting power of the corporation in the election of directors. Once an acquirer crosses one of these thresholds, shares which it acquired in the transaction taking it over the threshold and within the 90 days immediately preceding the date when the acquiring person acquired or offered to acquire a controlling interest become "control shares" to which the voting restrictions described above apply. Our Articles of Incorporation include a provision stating that these laws, or any successor statutes, relating to acquisitions of controlling interests in Palvella, shall not apply to us or to any acquisition of any shares of our capital stock. These laws may have a chilling effect on certain transactions if our Articles of Incorporation are amended to eliminate the foregoing provision and these laws otherwise apply according to their terms.

Nevada's "combinations with interested stockholders" statutes (NRS 78.411 through 78.444, inclusive) provide that specified types of business "combinations" between certain Nevada corporations and any person deemed to be an "interested stockholder" of the corporation are prohibited for two years after such person first becomes an "interested stockholder" unless the corporation's board of directors approves the combination (or the transaction by which such person becomes an "interested stockholder") in advance, or unless the combination is approved by the board of directors and 60% of the corporation's voting power not beneficially owned by the interested stockholder, its affiliates and associates. Furthermore, in the absence of prior approval certain restrictions may apply even after such two-year period. For purposes of these statutes, an "interested stockholder" is any person who is (1) the beneficial owner, directly or indirectly, of 10% or more of the voting power of the outstanding voting shares of the corporation, or (2) an affiliate or associate of the corporation and at any time within the two previous years was the beneficial owner, directly or indirectly, of 10% or more of the voting power of the then-outstanding shares of the corporation. The definition of the term "combination" is sufficiently broad to cover most significant transactions between a corporation and an "interested stockholder". These laws generally apply to Nevada corporations with 200 or more stockholders of record. However, a Nevada corporation may elect in its articles of incorporation not to be governed by these particular laws, but if such election is not made in the corporation's original articles of incorporation, the amendment (1) must be approved by the affirmative vote of the holders of stock representing a majority of the outstanding voting power of the corporation not beneficially owned by interested stockholders or their affiliates and associates, and (2) is not effective until 18 months after the vote approving the amendment and does not apply to any combination with a person who first became an interested stockholder on or before the effective date of the amendment. We have not made such an election in our original articles of incorporation, and we have not amended our Articles of Incorporation to so elect.

Further, NRS 78.139 provides that directors may resist a change or potential change in control of the corporation if the board of directors determines that the change or potential change is opposed to or not in the best interest of the corporation upon consideration of any relevant facts, circumstances, contingencies or constituencies pursuant to NRS 78.138(4).

Exhibit 10.30

LEASE AGREEMENT

THIS LEASE AGREEMENT (this "Lease") is made and entered into this 2nd day of May, 2025, by and between **LMP 353, LLC**, a Pennsylvania limited liability company ("Landlord") and **PALVELLA THERAPEUTICS, INC.**, a Delaware corporation ("Tenant").

For and in consideration of the mutual agreements herein contained, Landlord hereby leases and demises to Tenant, and Tenant hereby leases and accepts from Landlord, a portion of the building located **at 353 W. Lancaster Avenue, Wayne, Pennsylvania 19087 (the "Building"), such portion consisting of approximately 6,853 rentable square feet, commonly referred to as Suite 200 covering the entire 2nd floor of the Building**, and on the land being more particularly described on the **Exhibit A** attached hereto and made a part hereof (the "Land"), and all other improvements thereon, all mechanical systems and components thereof, and all appurtenant rights thereto, including, without limitation, parking areas, easements, declarations and rights of way, if any (all of the foregoing shall be collectively referred to herein as the "Premises").

1. Term.

(A) Lease Term. The term of this Lease shall be three (3) years, commencing on October 1, 2025 (the "Commencement Date"), and expiring on the last day of the thirty-sixth (36th) month thereafter ("Expiration Date") (the period from the Commencement Date to the Expiration Date, herein the "Lease Term"). The Commencement Date and Expiration Date shall be confirmed by Landlord and Tenant by execution of a Confirmation of Lease Term in the form attached hereto as **Exhibit B**, provided that if Tenant fails to execute or object to said document within ten (10) days of its delivery, Landlord's determination of such dates shall be deemed accepted.

(B) Renewal Option. Provided that Tenant is not in default under this Lease, Landlord hereby grants to Tenant the option to extend the Lease Term (the "Renwal Option") for one (1) period of two (2) years (the "Renewal Period") by providing written notice to Landlord at least nine (9) months prior to the end of the initial Term. Upon such notice, the Tenant shall be legally bound to fulfill its obligations under this Lease for the Renwal Period subject to the same terms and conditions as set forth herein, except with respect to the Base Rent for the Renwal Period which shall be at the greater of (i) the then prevailing market rate for comparable office buildings in Wayne, Pennsylvania in Landlord's sole discretion (the "Fair Market Rent"), or (ii) one hundred three percent (103%) of the Base Rent payable by Tenant at the expiration of the initial Lease Term. For the purposes of this Lease, all references to the "Lease Term" shall include the initial Term and the Renewal Period to the extent actually extended by Tenant.

(C) Early Access. Subject to the terms below and the early termination of the lease by the existing tenant of the Premises, Landlord shall use commercially reasonable efforts to provide Tenant with early access to the Premises for a period of up to fifteen (15) days prior to the Commencement Date ("Early Access") for the purpose of installing Tenant's furniture, fixtures and equipment. Tenant's occupancy of the Premises during such period prior to the Commencement Date, if any, will be subject to all of the provisions of this Lease, except that no Rent will be payable by Tenant for such Early Access. Any occupancy or use of Tenant of the

Premises prior to the Commencement Date shall be at Tenant's sole risk, cost and expense, and Landlord shall not be liable for, and Tenant hereby releases Landlord from, any and all liability for theft thereof or any damage thereto occasioned by any act of God or by any acts, omissions or negligence of any persons. Tenant agrees to indemnify, defend (with counsel reasonably acceptable to Landlord), and hold harmless Landlord and its employees, officers, directors, agents and contractors from and against any and all claims, liabilities, losses, actions, causes of action, demands, costs and expenses (including, without limitation, attorneys' fees at the trial and appellate levels) of any and every nature arising out of or in any way relating to such occupancy or use.

(D) If requested by Tenant, Landlord shall provide a mailing address for Tenant at the Building from and after the Effective Date.

2. Acceptance of Premises. Landlord shall deliver the Premises to Tenant on the Commencement Date in good operating condition and repair and in compliance with all applicable laws and codes (including ADA), with the roof, structural elements and all Building systems in good working condition, with all necessary maintenance, repairs and replacements performed, and free and clean of debris and ready for Tenant's installation of its furniture, fixtures and equipment. Tenant affirms that the Premises is suitable for Tenant's intended use as described in Section 18. Landlord shall perform the work specifically set forth on **Exhibit C** attached hereto and made a part hereof within a reasonable time following the Commencement Date. Subject to this Section 2, Tenant acknowledges and agrees that the Premises shall be leased by Landlord to Tenant in an "AS IS, WHERE IS" condition, without representations or warranties whatsoever.

3. Base Rent. As consideration for the Premises, Tenant shall pay to Landlord rent ("Base Rent") in advance in equal monthly installments as set forth below, without deductions and setoffs and without prior demand therefor, on the first day of each calendar month during the Lease Term:

Lease Year	Per Square Foot	Annual Rent	Monthly Rent
1	$38.00	$260,414.00	$21,701.17
2	$38.95	$266,924.35	$22,243.70
3	$39.92	$273,571.76	$22,797.65

For the purposes of this Lease, "Lease Year" means each consecutive twelve (12) month period during the Lease Term, beginning on the Commencement Date, provided that the first Lease Year shall include any partial month during which the Commencement Date occurs, if applicable. For the purposes of this Lease, "Additional Rent" shall mean Tenant's Pro-Rata Share of the electricity charges due under Section 8 hereof, Tenant's Pro-Rata Share of Operating Expense increases due under Section 16 hereof, together with any other amounts owed by Tenant under this Lease. Base Rent and Additional Rent (collectively, "Rent) shall be paid to Landlord at 353 West Lancaster Avenue, Suite 300, Wayne, Pennsylvania 19087. The Base Rent for the first month during the Lease Term shall be due at the execution of this Lease.

Notwithstanding the foregoing and provided that Tenant is not in default of this Lease, Tenant shall be entitled to an abatement of Base Rent in the amount of $65,103.50 (the "Rent

Abatement") representing the Base Rent for the first three (3) months following the Commencement Date. During such abatement period, Tenant shall still be responsible for the payment of all electricity charges attributable to the Premises in accordance with Section 8 hereof. In the event of a default by Tenant under the terms of this Lease beyond the expiration of any applicable notice and cure period that results in early termination of this Lease, then any Base Rent abated hereunder shall be a part of the recovery set forth in Section 17 of this Lease, any Base Rent abated hereunder shall be immediately due and payable by Tenant to Landlord notwithstanding any subsequent cure of the breach by Tenant. Landlord's acceptance of Base Rent or the cure of such breach shall not be deemed a waiver by Landlord of the provisions of this Section 3.

4. Taxes. Subject to Section 16 hereof, Landlord shall pay all real estate taxes, assessments, state and local taxes, or any other governmental tax, assessment, or fee levied against the Premises.

5. Repairs and Future Improvements.

(A) Landlord's Repair and Maintenance Obligations. Landlord shall be responsible for all maintenance and repairs to the roof, foundation, and structural components of the Building, including structural walls and concrete sub flooring; maintenance, repair, and replacement of the HVAC system, electrical fixtures and equipment, underground utilities, and other such systems servicing the Premises; maintaining the landscaping and grounds of the Building in neat and clean condition, and any maintenance and/or repairs necessitated by the actions or negligence of Landlord, its agents and employees.

(B) Tenant's Repair and Maintenance Obligations. Except as expressly provided herein, Tenant, at its own cost and expense, shall be responsible for all maintenance and repairs of the Premises, including, but not limited to, keeping the interior of the Premises neat and clean and repairing any damage caused by Tenant or its agents, employees, contractors, or invitees.

(C) Tenant Improvements. Tenant shall not construct any improvements or make any alterations to the Premises without Landlord's prior written consent, not to be unreasonably withheld, conditioned or delayed. All such work, alterations, decorations, installations, additions or improvements (collectively, the "Improvements") shall be done at Tenant's sole expense in a good and workmanlike manner and in full compliance with all laws, rules, regulations, and requirements of all governmental bureaus and bodies having jurisdiction thereover. Tenant shall repair any damage which may occur from the Improvements to the Premises. Landlord reserves the right to require certificates evidencing insurance from Tenant's contractors in amounts reasonably determined by Landlord and naming Landlord as an additional insured party thereunder. All Improvements (other than communications equipment, trade fixtures as provided herein, or equipment leased by Tenant) shall become the property of Landlord and shall remain upon, and be surrendered with, the Premises as a part thereof at the expiration of the Lease Term or prior termination of this Lease, as the case may be. Tenant shall keep full and accurate records of the cost of any Improvements in and to the Premises made by Tenant and shall, if requested by Landlord, make the same available to Landlord for use in connection with any proceeding to review the assessed valuation of the Premises, any proceeding to acquire the Land

and Premises for public or quasi-public use, or otherwise. Tenant shall promptly pay and discharge any and all liens or other charges arising out of or in connection with the performance of any act required of or permitted Tenant hereunder, and Tenant shall keep the Premises free and clear from any and all such liens or charges, and any liens and charges shall exist only against the leasehold estate of Tenant and not against the fee. Additionally, Tenant agrees to indemnify and hold harmless Landlord from and against any and all losses, costs, damages or liabilities resulting from or attributable to any liens or claims of lien for such work and Tenant shall remove any such lien or claim of lien promptly upon notice of such lien or claim of lien.

(D) Landlord shall provide an allowance for Tenant to renovate and improve the Premises pursuant to the requirements of the Lease in an amount up to Thirty-Four Thousand Two Hundred Sixty-Five and No/100 Dollars ($34,265.00) ("TI Allowance"), Landlord shall pay the TI Allowance within thirty (30) days after Tenant delivers to Landlord the final lien waivers from all contractors and materialmen providing services or materials to the Premises, evidencing completion of the improvements, and copies of paid invoices (with canceled checks or other proof of payment for the same). Tenant agrees to waive and release Landlord from any obligation to pay any remaining, unspent portion of the TI Allowance atfter twenty-four (24) months from the Commencement Date. Tenant shall be responsible for all construction costs in excess of the TI Allowance. If Tenant defaults under the Lease beyond any applicable notice and cure period, Tenant shall immediately repay the TI Allowance to Landlord.

6. Fixtures. Provided Tenant is not in default hereunder, Tenant shall have the right upon the termination or early expiration of this Lease to remove from the Premises any movable trade fixtures that were purchased or installed by it, provided Tenant repairs any damage to the Premises caused by such removal. Title to such property shall remain in Tenant. This shall not include the right to remove any plumbing or wiring or structural alterations, additions or improvements to the Premises and shall, as a matter of course, not include any fixtures that were furnished or paid for by Landlord. No later than the last day of the Lease Term, Tenant shall remove all Tenant's personal property and repair all injury done by or in connection with the installation or removal of Tenant's personal property and/or trade fixtures.

7. Signs. Landlord shall provide Tenant with standard identification signage on Tenant's suite entry door, on all Building directories, and on the Building monument sign on Route 30. Any additional signage requested by Tenant must be approved by Landlord in writing, such approval to be in Landlord's sole discretion.

8. Utilities/Janitorial Services. Landlord shall contract for in its own name and shall pay the cost of providing all utilities required by Tenant in the use of the Premises including but not limited to water, gas, sewer, garbage service, electricity, and any other items required in order to provide heat, air conditioning, water, and any other utilities or services to the Premises; provided, however, (i) Tenant shall reimburse Landlord Tenant's Pro-Rata Share (as defined in Section 16 below) of all electricity charges within thirty (30) days after Tenant's receipt of paid invoices and (ii) Tenant shall either pay its Pro-Rata Share of any janitorial services for the Premises provided by Landlord, or contract for such janitorial services in its own name and shall pay directly all cost associated therewith. Landlord shall provide HVAC services to the Premises during normal business hours of the Building, which shall be 8:00 a.m. to 5:30 p.m. Monday

through Friday. Landlord shall provide a designated common area in the Building for all tenants to be used for the installation of information technology equipment. Landlord shall not be liable to Tenant for any discontinuance of utilities, telephone or other communication services caused by accident, breakage, strike or any other cause whatsoever that is outside of Landlord's control. Landlord reserves the right to interrupt or suspend any such services when necessary, whether because of accident, emergency or otherwise. Landlord shall, to the extent possible, give Tenant reasonable notice of any interruption or suspension or planned suspension of such services in the event of such interruption or suspension and shall use reasonable diligence to cause such service to be resumed promptly.

9. Parking. Tenant and its invitees shall have the non-exclusive right to the use of the parking area servicing the Building in common with others, without charge and to the use of the driveways appurtenant thereto for the purposes of ingress and egress, delivery and pickup, and parking of motor vehicles. Landlord shall reasonably monitor and enforce the parking area to ensure Tenant has parking for up to 24 spaces.

10. Indemnification of Landlord.

(A) Landlord shall not be liable for (and Tenant hereby releases Landlord from liability for) injury or damage to persons or property occurring within the Premises except to the extent caused by or resulting from the gross negligence or willful misconduct of, or breach of any provision of this Lease by, Landlord or any of Landlord's agents, servants or employees.

(B) Tenant hereby agrees to indemnify and hold harmless Landlord from and against any and all liability, claims, demands, expenses, fees, fines, penalties, suits, proceedings, actions and cause of action of every kind and nature arising or growing out of or in any way connected with Tenant's use, occupation, or control of the Premises and the furnishings, equipment and fixtures used in connection therewith or which may result from any breach, violation or nonperformance of any covenant, condition or agreement contained in this Lease on the part of Tenant to be kept in force. Except as otherwise herein provided, Tenant covenants and agrees that it will at its own cost and expense defend any and all actions, suits or proceedings which may be brought against Landlord or in which Landlord may be impleaded where alleged liability is based on Tenant's use, occupancy, or condition of the Premises.

11. Insurance. Throughout the Lease Term, Landlord, at its expense, shall maintain "all risk" property insurance covering the Building against loss or damage. Throughout the Lease Term, Tenant, at its expense, shall maintain commercial general public liability insurance covering claims arising out of liability for bodily injury, death, personal injury, and property damage occurring on the Premises or to the Building in amounts of not less than $2,000,000.00 per occurrence for injury to person, not less than $1,000,000.00 for property damage, and not less than $2,000,000.00 annual aggregate. The insurance policy to be maintained by each of Tenant and Landlord above shall (a) be issued by insurance companies authorized to do business in the state in which the Premises or Building/Land is located; (b) be issued by insurance companies with a Best rating of A or better; (c) provide that said insurance shall not be canceled or materially modified without first providing thirty (30) days' prior written notice to Landlord; and Tenant shall name Landlord as an additional insured on Tenant's commercial general public liability insurance

policy. Each party shall provide the other party with a copy of such insurance policy(ies) prior to the Commencement Date.

12.Casualty. In the event that, as a result of fire or other casualty, (a) more than fifty percent (50%) of the square footage of the Building is totally damaged or rendered substantially untenantable (as reasonably determined by Landlord), or (b) the Building is rendered structurally unsound or unstable (either being a "Major Casualty"), then either Landlord or Tenant may elect to cancel this Lease by written notice to the other party, in which event the accrued Rent with respect to the Premises shall be paid up to the time of fire or other casualty. If twenty percent (20%) or more of the Premises is rendered untenantable as a result of such casualty, then the Tenant shall have the right to cancel this Lease by written notice to the Landlord, in which event the accrued Rent with respect to the Premises shall be paid up to the time of such casualty. If neither Landlord nor Tenant terminates this Lease as above provided, then this Lease shall remain in force and effect, except that Rent shall abate pro rata in accordance with Tenant's actual loss of use of the Premises. In the event the Building is damaged by fire or other casualty, but less than a Major Casualty, then Landlord shall repair the damage with reasonable dispatch (provided that Landlord's financial obligation to make such repairs shall not exceed the insurance proceeds made available to Landlord as a result of such casualty), and the Lease shall remain in full force and effect, except that until such repairs shall be made, the Rent shall abate pro rata according to the portion of the Premises which is rendered unusable by Tenant.

13.Condemnation.

(A) If at any time during the Lease Term, the whole or any part of the Land shall be taken for any public or quasi-public purpose by any lawful power or authority by the exercise of the right of condemnation or eminent domain, or private purchase in lieu thereof by a public body vested with the power of eminent domain, Landlord shall be entitled to and shall receive any and all awards or private purchases that may be made in any such proceedings except for awards expressly made for Tenant's property or moving expenses. Except as otherwise provided, Tenant hereby assigns and transfers to Landlord any and all such awards that may be made to Tenant. Tenant shall not be entitled to any payment based inter alia upon the value of the unexpired term of this Lease, consequential damage to the land not so taken, or the use of the Premises.

(B) If such proceedings shall result in the taking of the whole or substantially all of the Premises or if Tenant cannot substantially conduct its operations on the part remaining, or if Landlord reasonably determines that restoration of the Premises is not practical or advisable, this Lease shall terminate and expire on the date of such taking, and the net Rent and other sums or charges provided in this Lease to be paid by Tenant shall be apportioned and paid to the date of such taking.

(C) If the Lease is not terminated as provided above, Landlord, with reasonable dispatch, shall repair the remaining portion of the Building taken so as to restore the Building, and the Rent shall be adjusted pro rata as the square footage remaining shall bear to the square footage of the Building prior to the taking. Landlord shall not be obligated to expend thereon more than the sum allowed to Landlord in such condemnation proceeding for damage to the Premises, less all expenses incurred by Landlord in such proceedings, and if the expense of such restoration

would be greater than the sum allowed to Landlord, less such expenses in such condemnation proceedings, then Landlord shall have an option, for a period of thirty (30) days after Landlord's receipt of the condemnation proceeds, within which to decide whether to make such restoration or to terminate this Lease.

14.Assignment or Sublease. Tenant shall not assign, mortgage or encumber this Lease nor sublet or permit the Premises or any part thereof to be used by others, without the prior written consent of Landlord in each instance, which consent shall not be unreasonably withheld, conditioned or delayed. The consent by Landlord to an assignment or subletting shall not be construed to relieve Tenant from obtaining the consent in writing of Landlord to any further assignment or subletting. Notwithstanding the foregoing, Tenant shall have the right to assign this Lease or sublet the Premises or any part thereof to any related entity, subsidiary, parent company or affiliate of Tenant, any company in which Tenant has a controlling interest, or to any successor corporation, whether by merger, consolidation or otherwise, or to any person who purchases all or substantially all of Tenant's assets, without Landlord's approval or consent (each a "Permitted Transfer"). No Permitted Transfer shall relieve Tenant's liability hereunder. Tenant shall remit to Landlord all excess rent derived from any transfer, except that Tenant may retain fifty percent (50%) of any excess rent derived from a Permitted Transfer.

15.Subordination. This Lease and all rights of Tenant hereunder are and shall be subject and subordinate to any mortgage, including a consolidated mortgage, constituting a first lien on the real property, whether such mortgage has heretofore been or may hereafter be placed upon the real property to secure an indebtedness to any savings bank, bank, trust company, or other institutional lender, private or public, and to any renewal modification, consolidation, replacement or extension of any such mortgage. Although no instrument or act on the part of Tenant shall be necessary to effect such subordination, Tenant will nevertheless execute and deliver such further instruments subordinating this Lease to the lien of any such mortgage.

16.Operating Expenses.

(A) Increase in Operating Expenses. In the event there is any increase during any year of the Lease Term in the "Operating Expenses" for the Building (as such expenses are described hereinafter and are customarily and consistently applied to all tenants of the Building) over and above the amount of such Operating Expenses during the calendar year which the Lease Term commences (2025 "base year") and upon presentation of proof of the increased expenses and paid invoices provided by Landlord to Tenant, Tenant shall pay to Landlord, within thirty (30) days, an amount equal to 40.59% of the increase in the Operating Expenses ("Tenant's Pro-Rata Share"), which is calculated by dividing the Tenant's rentable share of the Building (6,853 SF) by the entire rentable area of the Building (16,883 SF). Notwithstanding the foregoing, if at any time the Building is less than ninety-five percent (95%) leased, Tenant's Pro-Rata Share shall be recalculated to be a percentage equal to the ratio of rentable area in the Premises to the leased rentable area in the Building.

(B) Definition of Operating Expenses. "Operating Expenses," which are included in the "Rent" amount, shall mean the amount of all of Landlord's costs and expenses paid or incurred in operating, repairing and maintaining the Building (including the Common Areas (as

defined herein)) in good condition and repair for a particular calendar year, including all costs and expenses which Landlord reasonably determines that it would have paid or incurred during such year if the Building had been fully occupied, including, but not limited to, all real estate taxes, assessments and fees levied against the Building; the annual cost of Landlord's property/casualty and liability insurance on the Building; utilities serving the Building (other than those reimbursable by Tenant under Section 8 hereof); service and other charges incurred in the repair, replacement, operation and maintenance of the heating, ventilation and air-conditioning system that services the Building; cleaning and other janitorial services of Common Areas; tools and supplies; repair costs; landscape maintenance costs; security services; license, permit and inspection fees; management or administrative fees; supplies, costs, wages and related employee benefits payable for the management, maintenance and operation of the Building; maintenance, repair and replacement of the driveways, parking and sidewalk areas (including snow and ice removal), landscaped areas, and lighting; maintenance and repair costs, dues, fees and assessments incurred under any covenants, trust indenture or Landlords association. The cost of any capital improvement shall be amortized over the useful life of such improvement (as reasonably determined by Landlord), and only the amortized portion shall be included in Operating Expenses.

(C) Definition of Common Areas. "Common Areas" shall mean the areas of the Building and the Land which are designed for use in common by all tenants of the Building and their respective employees, agents, customers, invitees and others, and includes, by way of illustration and not limitation, entrances and exits, hallways and stairwells, elevators, restrooms, sidewalks, driveways, parking areas, landscaped areas and other areas as may be designated by Landlord as part of the Common Areas of the Building. Tenant shall have the non-exclusive right, in common with others, to the use of the Common Areas.

17. Default.

(A) The occurrence of any of the following is deemed to be an event of default under this Lease:

(1) Any installment of Rent required to be paid by Tenant which is not paid within three (3) days of when due and after notice of such non-payment is received by Tenant.

(2) The making by Tenant of an assignment for the benefit of its creditors;

(3) The levying of a writ of execution or attachment on or against the property of Tenant and the same not being released or discharged within thirty (30) days thereafter;

(4) The institution of proceedings for the reorganization, liquidation or involuntary dissolution of Tenant, or for its adjudication as a bankrupt or insolvent, or for the appointment of a receiver of the property of Tenant, and said proceeding not being dismissed, and any receiver, trustee or liquidator appointed therein discharged within thirty (30) days after the institution of such proceedings;

(5) The doing, or permitting to be done of any act by Tenant which creates a claim or a lien therefore against the Premises and the same not being released or otherwise

provided for by indemnification reasonably satisfactory to Landlord within thirty (30) days thereafter;

(6) Failure of Tenant to cure non-compliance with any other covenant or provision of this Lease within thirty (30) days after written notice of such failure is given by Landlord, or if it is not feasible to cure such failure within such period, to advise Landlord in writing of Tenant's intention duly to institute all steps necessary to cure such failure or violation and to begin performance of such covenants within such period and diligently to pursue performance to completion in a reasonable time thereafter.

(B) If an event of default shall occur, the following provisions shall apply and Landlord shall have, in addition to all other rights and remedies available at law or in equity, including the right to terminate this Lease, the rights and remedies set forth herein, which may be exercised upon or at any time following the occurrence of an event of default:

(1) By notice to Tenant, Landlord shall have the right to accelerate all Rent and all expense due hereunder and otherwise payable in installments over the remainder of the Lease Term; and the amount of accelerated rent to the termination date, without further notice or demand for payment, shall be due and payable by Tenant within five (5) days after Landlord has so notified Tenant, such amount collected from Tenant shall be discounted to present value using an interest rate of six percent (6%) per annum.

(2) The damages which Landlord shall be entitled to recover from Tenant shall be the sum of: (i) all Rent accrued and unpaid as of the termination date; and (ii)(1) all costs and expenses incurred by Landlord in recovering possession of the Premises, including legal fees, and removal and storage of Tenant's property, (2) the costs and expenses of restoring the Premises to the condition in which the same were to have been surrendered by Tenant as of the expiration of the Lease Term, and (3) the costs of reletting commissions; and (iii) all Rent otherwise payable by Tenant over the remainder of the Lease Term as reduced to present value; and all consequential damages relating to Tenant's breach of this Lease; provided, however, all Rent which Landlord receives from other tenant(s) by reason of the leasing of the Premises during any period falling within the otherwise remainder of the Lease Term shall be deducted from the total determined above under subsections (i), (ii), and (iii).

(3) Without limiting the generality of the foregoing, if Tenant shall fail to perform any of its obligations hereunder, Landlord may, in addition to any other rights it may have in law or in equity, cure such default on behalf of Tenant, and Tenant shall reimburse Landlord upon demand for any sums paid or costs incurred by Landlord in curing such default, including reasonable attorneys' fees and other legal expenses, together with interest at 12% per annum ("Default Rate") from the dates of Landlord's incurring of costs or expenses.

(4) Any sums payable by Tenant hereunder, which have not been paid three days after Tenant's receipt of notice by Landlord that such sums are outstanding, shall bear interest at the Default Rate.

(5) No delay or forbearance by Landlord in exercising any right or remedy hereunder, or Landlord's undertaking or performing any act or matter which is not expressly required to be undertaken by Landlord shall be construed, respectively, to be a waiver of Landlord's rights or to represent any agreement by Landlord to undertake or perform such act or matter thereafter. Waiver by Landlord of any breach by Tenant of any covenant or condition herein contained (which waiver shall be effective only if so expressed in writing by Landlord) or failure by Landlord to exercise any right or remedy in respect of any such breach shall not constitute a waiver or relinquishment for the future of Landlord's right to have any such covenant or condition duly performed or observed by Tenant, or of Landlord's rights arising because of any subsequent breach of any such covenant or condition nor bar any right or remedy of Landlord in respect of such breach or any subsequent breach.

(C) **CONFESSION OF JUDGMENT.**

(1) If Tenant shall default in the payment of the rent or any other sums due hereunder by Tenant, or in the event of any other default as defined herein, Tenant hereby irrevocably authorizes and empowers any prothonotary or attorney of any court of record within the United States of America, or elsewhere, to appear for Tenant, with or without complaint filed; and in said suits or actions to confess judgment, or a series of judgments, against Tenant and all persons claiming through or under Tenant, in favor of Landlord, for all or any part of said rental and/or said other sums, including, but not limited to, the amounts due from Tenant to Landlord under (B) of this section, and including any amount to which Landlord would be entitled as damages under the provisions of this Lease, and for interest and costs, and a reasonable attorney's commission not to exceed fifteen percent (15%) for collection, for which this Lease, or a true and correct copy thereof, shall be sufficient warrant, and such powers may be exercised as well after the termination or expiration of the term of this Lease.

Tenant hereby acknowledges that by agreeing to the foregoing confession of judgment and warrant of attorney, Tenant waives the right to notice and a prior judicial proceeding to determine its rights and liabilities, and further acknowledges that Landlord may, on default by Tenant under this Lease, subject to such notice requirements, if any, as are herein expressly provided, obtain a judgment against Tenant for all sums due hereunder, and levy execution on such judgment against any and all property of Tenant without any opportunity of Tenant to raise any defense, setoff, counterclaim or other claim that Tenant may have, and that Tenant knowingly, voluntarily and intelligently grants Landlord the foregoing right to confess judgment and warrant of attorney as an explicit and material part of the consideration bargained for between Tenant and Landlord. Tenant certifies that it has been represented by (or has had the opportunity to be represented) at the signing of this Lease and in the granting of this confession of judgment and warrant of attorney by independent legal counsel, selected of its own free will, and that it has had the opportunity to discuss the confession of judgment and warrant of attorney with counsel. Tenant further certifies that it has read and understands the meaning and effect of the foregoing confession of judgment and warrant of attorney.

Please Initial _____ Date: ___

(2) When this Lease or Tenant's right of possession shall be terminated by reason of the breach of any provision of this Lease, or in the event of a deliberate event of default as defined herein, either during the Term or any renewal or extension thereof, and also when and as soon as the term shall have expired or been terminated, Tenant hereby irrevocably authorizes and empowers any prothonotary or attorney of any court of record as attorney for Tenant and any persons claiming through or under Tenant, with or without complaint filed, to confess judgment in ejectment against Tenant and all persons claiming through or under Tenant, in favor of Landlord, for the recovery by Landlord of possession of the Premises, for which this Lease, or a true and correct copy thereof, shall be sufficient warrant, whereupon if Landlord so desires, a writ of execution or of possession may issue forthwith, without any prior writ or proceedings whatsoever, and provided that if for any reason after such action shall have been commenced the same shall be determined, canceled or suspended and possession of the Premises remain in or be restored to Tenant or any person claiming through or under Tenant, Landlord shall have the right upon any subsequent default or defaults, or upon any subsequent termination or expiration of this Lease, or any renewal or extension hereof, or of Tenant's right of possession as hereinbefore set forth, to confess judgment in ejectment as hereinbefore set forth one or more additional times to recover possession of the Premises.

Tenant hereby acknowledges that by agreeing to the foregoing confession of judgment and warrant of attorney, Tenant waives the right to notice and a prior judicial proceeding to determine its rights and liabilities, and further acknowledges that Landlord may, on default by Tenant under this Lease, subject to such notice requirements, if any, as are herein expressly provided, obtain a judgment against Tenant for possession of the Premises without any opportunity of Tenant to raise any defense, setoff, counterclaim or other claim that Tenant may have, and that Tenant knowingly, voluntarily and intelligently grants Landlord the foregoing right to confess judgment and warrant of attorney as an explicit and material part of the consideration bargained for between Tenant and Landlord. Tenant certifies that it has been represented by (or has had the opportunity to be represented) at the signing of this Lease and in the granting of this confession of judgment and warrant of attorney by independent legal counsel, selected of its own free will, and that it has had the opportunity to discuss the confession of judgment and warrant of attorney with counsel. Tenant further certifies that it has read and understands the meaning and effect of the foregoing confession of judgment and warrant of attorney.

Please Initial _____ Date: ___

In any action of or for ejectment or for rent or other sums, if Landlord shall first cause to be filed in such action an affidavit made by it or someone acting for it setting forth the facts necessary to authorize the entry of judgment, such affidavit shall be conclusive evidence of such facts; and if a true copy of this Lease (and of the truth of the copy such affidavit shall be sufficient evidence) be filed in such action, it shall not be necessary to file the original as a warrant of attorney, any rule of court, custom or practice to the contrary notwithstanding. Tenant hereby waives and releases to Landlord, and to any and all attorneys who may appear for Landlord, all procedural errors in any proceedings taken by Landlord, whether by virtue of the warrants of attorney contained in this Lease or not, stay of execution and extension of time of payment, all laws exempting real or personal property from execution and all liability therefor, and no benefit of exemption will be claimed under and by virtue of any exemption law now in force or which

may hereafter be passed.

Pursuit of any of the other remedies herein provided shall not preclude pursuit of any other remedies provided by law, nor shall pursuit by Landlord of any remedy herein provided constitute a forfeiture or waiver of any Rent due to Landlord hereunder or of any damages accruing to Landlord by reason of the violation of any of the covenants and provisions herein contained.

(D) Tenant hereby appoints as its agent to receive service of all dispossessory or distraint proceedings and notices thereunder the person in charge of the Premises at the time.

18.Use of Premises; Compliance with Laws. Tenant shall use the Premises exclusively for general office and other related uses permitted by zoning, and shall not use or occupy or permit the Premises to be used or occupied, nor do or permit anything to be done in or on the Premises, in a manner which will cause or be likely to cause structural damage to the Building, or any part thereof or which will constitute a public or private nuisance, and Tenant shall not use or occupy or permit the Premises to be used or occupied in any manner which will violate any present or future laws or regulations of any governmental authority. Tenant shall have access to the Premises 7 days per week, 24 hours per day. Tenat's use of the Premises shall be limited to general office use and storage incidental thereto. Tenant shall be responsible for keeping the Premises at all times in compliance with all laws, ordinances, regulations and code requirements, including but not limited ADA.

19.Landlord's Access to the Premises. Landlord, its agents and employees, shall have the right to access and enter upon the Premises upon twenty-four (24) hours prior written notice to Tenant for purposes of performing such maintenance and repairs as Landlord deems reasonably necessary, showing the Premises to prospective purchasers thereof and, during the last six months of the Lease Term, showing the Premises to prospective tenants thereof. Landlord also shall have right to access and enter upon the Premises without prior notice to Tenant in the event of an emergency.

20.Quiet Enjoyment. Tenant, upon the payment of the Rent hereind described, and upon the performance of all of the terms of this Lease, shall at all times during the terms of this Lease quietly enjoy the Premises without any disturbance from Landlord or from any person claiming through Landlord; subject, however, to the terms and conditions of this Lease.

21.Surrender; Holdover. Upon the Expiration Date or the prior termination of the Lease Term, Tenant shall surrender the Premises to Landlord in as good condition as it was in at the beginning of the Lease Term, reasonable use and wear and damages by the elements or casualty excepted. Any holdover by Tenant beyond the expiration or prior termination of this Lease shall be deemed to create a tenancy-at-will, subject to all other terms and conditions of this Lease, except tha Rent shall be increased to 125% of the Rent in effect immediately prior to the expiration or earlier termination of this Lease.

22.Rights of Successors and Assigns. The terms, covenants and conditions contained in this Lease shall apply to and inure to the benefit of and be binding upon the parties hereto and upon their respective successors, legal representatives and assigns.

23.Effect of Instrument. It is expressly understood and agreed that this instrument contains the entire contract between the parties hereto and that all covenants, agreements and conditions herein shall be binding and may be legally enforced by the said parties, their successors, personal representatives and assigns, respectively.

24.Controlling Law. This Lease shall be governed by the laws of the Commonwealth of Pennsylvania.

25.Amendment; Severability. This Lease may not be amended or modified except by written agreement signed by both Landlord and Tenant. All provisions of this Lease are severable from all others, and if any provision of this Lease shall be deemed unenforceable, such shall not affect the enforceability of the remainder of this Lease.

26.Notices. All notices to be given under this Lease shall be in writing and shall either be personally delivered, or sent by certified mail, return receipt requested, or sent by Federal Express or other comparable commercial overnight delivery service, if to Landlord then at the address provided for payment of Rent, and if to Tenant, then at the Premises. Notices shall be deemed to have been given on the day sent or deposited; provided, however, that any time period for a response or responsive action to such notice shall be measured from the date such notice is actually received (any notice actually received after 5:00 PM at the site of receipt shall be deemed received on the following business day).

27.Brokerage. Tenant covenants, warrants and represents that no broker represents Tenant in the negotiation of this Lease other than Re:Align, Inc., which shall be compensated by Landlord in accordance with a separate agreement. Tenant shall indemnify and hold Landlord harmless from any liability, cost, fees (including attorneys' fees) incurred by any broker claiming through Tenant.

28.Security Deposit. Upon the execution of this Lease, Tenant shall deliver to Landlord a security deposit in the amount of Twenty-One Thousand Seven Hundred One and 17/100 Dollars ($21,701.17) ("Security Deposit"). Landlord may use, apply on Tenant's behalf or retain (without liability for interest) during the Lease Term the whole or any part of the Security Deposit to the extent required for the payment of any Rent or other sum as to which Tenant may be in default hereunder or for any sum which Landlord may expend by reason of Tenant's default, including, but not limited to, any deficiency or damage incurred in reletting the Leased Premises. The covenants in this Section are personal covenants between Landlord and Tenant and not covenants running with the land, and in no event will Landlord's mortgagees or any purchaser at a foreclosure sale or sale in lieu of foreclosure be liable to Tenant for the return of the Security Deposit. After each application from Tenant's Security Deposit, Tenant shall, upon demand by Landlord, replenish the Security Deposit to the full amount set forth in this Section. Provided Tenant shall comply with all of the terms of this Lease, such Security Deposit shall be returned to Tenant upon termination of this Lease and after surrender of possession of the Leased Premises to Landlord. In the event of a sale of the Premises or assignment of this Lease by Landlord to person other than a mortgagee, Landlord shall the right to transfer the Security Deposit to its assignee, subject to Tenant's aforesaid rights upon termination, and thereupon Landlord shall be released from any liability with respect to the return of such Security Deposit to Tenant, such assignee to

be solely responsible to Tenant therefore. Tenant shall not assign or encumber its interest in the Security Deposit, and neither Landlord nor its successors and assigns shall be bound by any attempted assignment or encumbrance.

29. Force Majeure Events. "Force Majeure" shall mean and include those situations beyond either parties control, including by way of example and not by way of limitation, acts of God, accidents, repairs, strikes, shortages of labor, supplies or materials, inclement weather, epidemic, pandemic, public health crisis, government-imposed quarantine or lock-down orders, which render performance of a party's obligations under this Lease (other than the payment of money) impossible. Any time limits required to be met by either party hereunder, whether specifically made subject to Force Majeure, (except those related to the payment of Rent, Operating Expenses and any other amounts due and owing hereunder), unless specifically stated to the contrary elsewhere in this Lease, will be automatically extended by the number of days by which any performance called for is delayed due to Force Majeure. Force Majeure which renders Tenant's access to the Premises impossible for a continuous period of four (4) months or longer or which renders Landlord's ability to provide the Premises to Tenant impossible for a continuous period of 4 months or longer, will be grounds for either party to terminate this Lease.

30. Right of First Refusal and Right of First Offer to Lease.

(A) If, during the Lease Term, Landlord receives a bona fide third party offer to lease space in the Building (the "Offer"), which Offer Landlord is willing to accept, Landlord will give written notice to Tenant of the Offer and Tenant shall have a period of fifteen (15) business days following receipt of such written notice from Landlord to elect, by providing written notice to Landlord prior to the expiration of such fifteen (15) business day period, to lease such space in the Building on the same economic terms set forth in the Offer and with a term which will be coterminous with the Lease Term of this Lease. Should Tenant fail to elect to lease such space within such fifteen (15) business period, Tenant will have waived its right to lease such space and Landlord will be free to lease such space to any third party on terms acceptable to Landlord.

(B) Additionally, should any space become available for lease in the Building during the Lease Term, before marketing or offering such space to lease to any third party, Landlord will provide written notice to Tenant of such available space, which notice will include the economic and other terms on which Landlord is willing to lease such space to Tenant, and Tenant will have a period of fifteen (15) business days from receipt of such written notice from Landlord in which to elect to lease such space on the terms and conditions set forth in Landlord's notice by providing written notice to Landlord. Should Tenant fail to elect to lease such space within such fifteen (15) business period (i) Tenant will have waived its rights under this Section 31(B), (ii) Landlord will be free to lease such space to any third party, and (iii) the provisions of Section 31(A)will not apply to such space.

{SIGNATURES ARE ON THE FOLLOWING PAGE}

IN WITNESS WHEREOF, the parties have executed this Lease as of the date first above written.

LANDLORD:

LMP 353, LLC, a Pennsylvania limited liability company

By: /s/ Timothy B. MacColl
Timothy B. MacColl, Manager
Date: May 5, 2025

TENANT:

PALVELLA THERAPEUTICS, INC., a Delaware corporation

By: /s/ Wesley Kaupinen
Name: Wesley Kaupinen
Title: Chief Executive Officer
Date: May 5, 2025

EXHIBIT A
(Legal Description)

ALL THAT CERTAIN parcel or tract of land, situate in Radnor Township, Delaware County, Pennsylvania, according to an ALTA/ACSM Land Title Survey prepared by Momenee Survey Group, Inc., Bryn Mawr, Pennsylvania dated March 25, 2007, last revised May 9, 2007 File No. 07-092 describes as follows, to wit:

BEGINNING at a point of intersection of the center line of Lancaster Avenue (50' wide) and the Westerly side of Farm Road (44' wide) extended; thence from said beginning point along the center line of Lancaster Avenue, South 88 degrees 43 minutes 00 seconds West, 193.98' to a point, a corner of land now or formerly of the Philadelphia Saving Fund Society; thence leaving Lancaster Avenue by land of The Philadelphia Saving Fund Society, North 01 degree, 17 minutes 00 seconds West, 220.00' to an iron pin, (passing over a marble monument being distant 25.33' herefrom); thence by land now or formerly of Leslie P. Morgan, North 88 degrees 43 minutes 00 seconds East 158.81' to an iron pin on the Westerly side of Farm Road; thence along said Westerly side of Farm Road, South 10 degrees, 22 minutes, 20 seconds East, crossing the Northerly side of Lancaster Avenue, 222.79' to the first mentioned point and place of beginning.

BEING known as 353 W. Lancaster Avenue. BEING Folio No. 36-01-00228-00.

EXHIBIT B

CONFIRMATION OF LEASE TERM

By executing this Confirmation of Lease Term, **LMP 353, LLC**, a Pennsylvania limited liability company ("Landlord") and **PALVELLA THERAPEUTICS, INC.**, a Delaware corporation ("Tenant") confirm that the Commencement Date of the Lease Agreement dated _____ ___, 2025, by and between the same, is _____ ____, 202_, and the Expiration Date of the Lease Agreement is _____, 202_.

LANDLORD:

LMP 353, LLC, a Pennsylvania limited liability company

By: /s/ Timothy B. MacColl
Timothy B. MacColl, Manager
Date: May 5, 2025

TENANT:

PALVELLA THERAPEUTICS, INC., a Delaware corporation

By: /s/ Wesley Kaupinen
Name: Wesley Kaupinen
Title: Chief Executive Officer
Date: May 5, 2025

EXHIBIT C

WORK LETTER

1. **Scope of TI Work**. Landlord agrees to perform on Tenant's behalf the following improvements within the Premises (collectively, "TI Work") at Landlord's cost in accordance with the provisions below:

(a) demolition of one (1) mutually agreed upon existing office and repair and/or patching of the affected floor and ceiling area;

(b) installation of security measures of a key card limiting access to the Premises via the elevator.

Tenant's approval of the plans and specifications ("TI Work Plans") shall be required for all TI Work. Landlord shall promptly prepare TI Work Plans for the TI Work and submit the same to Tenant for approval. The parties shall thereafter act in good faith to make mutually acceptable modifications to the TI Work Plans until the same have been approved. Once approved by Tenant, the TI Work Plans will be deemed final, subject only to change by Change Order as described below.

2. **Change Orders**.

(a) General. Any change in the TI Work Plans requested by Tenant (any such change being herein referred to as a "Change Order") will be at Tenant's sole cost and expense and subject to Landlord's written approval, which approval will not be unreasonably withheld, conditioned or delayed.

(b) Notice of Approved Change Order. In the event Landlord approves any such requested Change Order, Landlord will promptly give written notice thereof to Tenant, which notice will specify the Change Order approved by Landlord as well as the estimated incremental cost thereof and any estimated delay in the completion of the TI Work.

(c) Payment of Change Order Cost. Landlord will be under no obligation to proceed with any work related to an approved Change Order unless and until Tenant delivers to Landlord an amount equal to the full estimated incremental cost of such approved Change Order as set forth in Landlord's notice. When the final incremental cost of any such Change Order has been determined and incurred, Landlord and Tenant each agree to pay or refund the amounts owed to the other with respect to such Change Order, based on the estimated payment made to Landlord.

Exhibit 19.1

PALVELLA THERAPEUTICS, INC.

INSIDER TRADING POLICY

Effective March 28, 2025

I. Purpose

It is illegal for any employee, officer or director of Palvella Therapeutics, Inc. (the "Company") to trade in the securities of the Company while in the possession of material non-public information about the Company. It is also illegal for any employee, officer or director of the Company to give (deliberately or inadvertently) material non-public information to others who may trade on the basis of that information. The Company has adopted this Insider Trading Policy (this "Policy") in order to take an active role in the prevention of violations of such insider trading laws and to provide guidelines to all of the Company's officers, directors and employees, and to members of their immediate families and others living in their households or under their control, as well as the Company's outside advisors.

II. Persons Subject to the Policy

This Policy applies to (i) all officers, directors and employees of the Company and any of its subsidiaries, (ii) immediate family members (as defined below) and any persons that reside in the same household as any of the foregoing persons and (iii) any other person whose transactions in Company Securities (as defined below) are directed by, or subject to influence and control by the foregoing persons, and any trust, partnership, corporation or other entity over which such persons have investment control (collectively, "Insiders"). Individuals subject to this Policy are responsible for ensuring that immediate family members and members of their households comply with this Policy and therefore should make them aware of the need to confer with you before they trade in Company Securities (as defined below) and should treat all such transactions for the purposes of this Policy and applicable securities laws as if the transactions were for your own account.

This Policy does not, however, apply to personal securities transactions of your immediate family members (as defined below) where the purchase or sale decision is made by a third party not controlled by, influenced by or related to you or your immediate family members.

For purposes of this Policy, "immediate family member" means any spouse, domestic partner, child, stepchild, grandchild, parent, stepparent, grandparent, sibling, mother or father-in-law, son or daughter-in-law, or brother or sister-in-law (as well as other adoptive relationships), whether or not sharing the same household as the persons described in item (i) above.

All consultants and outside advisors assisting the Company on sensitive matters are expected to abide by this Policy, although the Company assumes no responsibility with respect to the actions of persons who are not under its direct control. Consultants and outside advisors are not Company employees, and nothing in this Policy should be construed to the contrary.

Persons in possession of material, non-public information related to, affecting or regarding the Company or its subsidiaries when their employment or service terminates may not trade in

Company Securities (as defined below) until that information has become public or is no longer material.

III. Transactions Subject to the Policy

This Policy applies to all transactions in securities of the Company (collectively referred to in this Policy as "Company Securities"), including common stock, options to purchase common stock, preferred stock, convertible debt and warrants, restricted stock units or any other type of securities that the Company has or may issue, as well as derivative securities that are not issued by the Company, such as exchange-traded put or call options or swaps relating to the Company Securities.

IV. General Policy

No Insider who is in possession of material, non-public information may, either directly or indirectly, (i) purchase or sell Company Securities, or, (ii) without the consent of the Company, provide material non-public information to any other person outside of the Company, including family and friends, provided that notwithstanding the foregoing (y) a director may provide material non-public information to their employer provided such employer either (1) otherwise complies with this paragraph or (2) has established its own insider trading controls and procedures in compliance with applicable security laws or (z) an Insider may disclose material, non-public information as required by law.

Insiders may not disclose, convey or "tip" material non-public information to any person (including family members) by providing them with material, non-public information other than to disclose on a "need to know" basis to directors, officers and employees of the Company or outside advisors in the course of performing their duties for the Company (it being understood that directors may disclose material non-public information to their employers; provided that such employer either (i) complies with the requirements of the first paragraph of this Section IV or (ii) has established its own insider trading controls and procedures in compliance with applicable securities laws). When sharing material, non-public information with other directors, officers and employees of the Company or outside advisors, or other persons involved in the business and affairs of the Company, such information should be confined to as small a group as possible. Unlawful tipping includes passing on material non-public information to friends, family members or acquaintances under circumstances that suggest that persons subject to this Policy were trying to help the recipients of such information to make a profit or avoid a loss by trading in Company Securities based on such information. Additionally, Insiders shall not make recommendations or express opinions on the basis of material, non-public information as to trading in the Company's securities.

Insiders who have access to material, non-public information regarding a public company other than the Company, or about such other company's products or activities, are prohibited from trading in the securities of these other public companies. In considering whether confidential or proprietary information of another company is material, Insiders should remember that the threshold for what is considered material may be different for other companies than it is for the Company.

V. Definition of Material Non-Public Information

Material Information. Information is considered "material" if a reasonable investor would consider that information important in making a decision to buy, hold or sell Company Securities or the securities of another public company. Any information that could be expected to affect the Company's stock price, whether it is positive or negative, should be considered material. Determining whether information is material is not always straightforward; rather, materiality is based on an assessment of all of the facts and circumstances, and is often evaluated by enforcement authorities with the benefit of hindsight. When doubt exists as to whether information would be considered "material," the information should be presumed to be material. While it is not possible to identify in advance all information that will be deemed to be material, some examples of such information would include the following:

- information concerning clinical trials and their results, intellectual property, regulatory approvals or other developments (positive or negative), product or technological plans, developments or agreements;
- annual or quarterly financial results that are known but have not been publicly disclosed;
- projections of future earnings or losses, or other earnings guidance;
- earnings or revenue that are inconsistent with the consensus expectations of the investment community;
- merger, acquisition, joint venture, partnerships, strategic alliances, collaborations or investment proposals, or the cancellation of such a strategic relationship;
- changes in strategic plans;
- the status of the Company's progress toward achieving significant Company goals;
- significant expansion or curtailment of operations;
- material information regarding an existing or potential customer or supplier;
- unusual borrowings;
- public or private securities offerings;
- litigation (pending or threatened);
- changes in certain senior management or members of the Board of Directors;
- communications to or from regulatory agencies or other significant regulatory developments;
- new product launches or the introduction of new business strategies;
- a stock split;
- listing on or delisting from a stock exchange;
- new major contracts, customers, distributors or suppliers, or the loss of any of the foregoing;
- material changes in the Company's pricing or cost structure for its products;
- a company restructuring;
- a significant cybersecurity incident;
- significant related-party transactions; or
- similar information concerning a significant subsidiary, business unit or investment.

Non-Public Information. Information that has not been widely disseminated to the public is generally considered to be non-public information. Information generally becomes available to the public when it has been disclosed by the Company or third parties in a press release or other authorized public statement, including any filing with the Securities and Exchange Commission (the "SEC") that is available on the SEC's website, through the Dow Jones "broad tape," newswire services, a broadcast on widely-available radio or television programs or publication in a widely-available newspaper, magazine or news website. By contrast, information would likely not be considered widely disseminated if it is available only to the Company's employees, or if it is only available to a select group of analysts, brokers and institutional investors.

Once information is widely disseminated, it is still necessary to afford the investing public with sufficient time to absorb the information. As a general rule, information should not be considered fully absorbed by the marketplace until ***after the first full trading day after the information is released***. If, for example, the Company were to make an announcement prior to the start of trading on a Monday, a person covered by this Policy should not trade in Company Securities until the start of trading on Tuesday. Depending on the particular circumstances, the Company may determine that a longer or shorter period should apply to the release of specific material non-public information.

If you are unsure whether you are in possession of material non-public information, you should consult with the Chief Executive Officer prior to engaging in, or entering into an agreement, understanding or arrangement to engage in, a purchase or sale transaction of any Company Securities. However, you are responsible for determining whether you are in possession of material, non-public information and any action on the part of the Company, the Chief Executive Officer or any other employee or director pursuant to this Policy or otherwise does not in any way constitute legal advice or insulate you from liability under applicable securities laws.

VI. Special and Prohibited Transactions

Both for the protection of Insiders and the Company, it is important to avoid even the appearance of insider trading or disclosure of material, non-public information. Therefore, it is against this Policy for Insiders to directly or indirectly participate in transactions involving trading activities that by their nature are aggressive or speculative, or may give rise to an appearance of impropriety. In addition to the other restrictions set forth in this Policy, the following transactions are strictly prohibited at all times:

- any sales of a class of Company Securities within six months after the purchase of that class of Company Securities, except pursuant to a properly authorized 10b5-1 trading plan (as defined below);
- trading in call or put options involving Company Securities and other derivative securities;
- engaging in short sales of Company Securities (i.e., sale of stock that the seller does not own or a sale that is completed by delivery of borrowed stock);
- holding Company Securities in a margin account;
- entering into any "equity" or "performance" swap or exchange agreements or similar arrangements;
- all forms of hedging or monetization transactions, such as zero-cost collars and forward sale contracts; and

- pledging Company Securities to secure margin or other loans.

If you are unsure whether a particular transaction is prohibited under this Policy, you should consult with the Chief Executive Officer prior to engaging in, or entering into, an agreement, understanding or arrangement to engage in, such transaction.

VII. Transactions Not Subject to Trading Restrictions Under the Policy

The trading restriction prohibitions in this Policy do not apply to:

- the granting of options or other equity awards;
- exercises of Company stock options provided that none of the underlying shares received are sold while in possession of material non-public information, except pursuant to a properly authorized 10b5-1 trading plan (as defined below);
- transactions between Insiders and the Company with respect to grants under its equity incentive plan (or, to the extent applicable, granted outside such plan), including the exercise of stock options for cash, the vesting of restricted stock or restricted stock units ("RSUs") or the exercise of a tax withholding right pursuant to which a person has elected to have the Company withhold shares to satisfy tax withholding upon the exercise of stock options or the vesting of any restricted stock or RSUs;
- bona fide gifts of Company Securities; provided that no Restricted Person may donate or make any gift during a period when they are not permitted to trade unless the donee agrees not to sell the Company Securities until such time as the Restricted Person is permitted to sell;
- investments in publicly traded mutual funds; or
- purchases or sales of Company Securities made pursuant to any binding contract, specific instruction or written plan entered into outside of a blackout period and while the purchaser or seller, as applicable, was unaware of any material non-public information and which contract, instruction or plan (i) meets all of the requirements of the affirmative defense provided by Rule 10b5-1 promulgated under the Securities Exchange Act of 1934, as amended (a "10b5-1 trading plan"), (ii) was pre-cleared in advance pursuant to this Policy and (iii) has not been amended or modified in any respect after such initial pre-clearance without such amendment or modification being pre-cleared in advance pursuant to this Policy.

Although exercises of Company stock options are not subject to the restrictions on trading in this Policy, such restrictions do apply to any sale of the underlying stock on the market in a "cashless exercise" effected through a broker or a "same day sale" of an option, except to the extent such sales are made pursuant to a properly authorized 10b5-1 trading plan. In addition, any sale of the underlying securities acquired upon the exercise of an option or RSU is subject to this Policy.

VIII. Additional Procedures Applicable to Restricted Persons

Blackout Periods. All officers and directors of the Company, as well as certain key employees listed on Annex A hereto (as may be amended from time to time), as well as any family members or other persons that reside in the same household as those persons (all of the foregoing being "Restricted Persons") are subject to additional restrictions on their ability to engage in

purchase or sale transactions involving Company Securities. Restricted Persons are more likely to have access to Inside Information regarding the Company because of their positions or affiliations with the Company and, as a result, their trades in Company Securities are more likely to be subject to greater scrutiny. Accordingly, Restricted Persons are prohibited from trading in Company Securities beginning twenty-one days prior to the filing of each of the Company's quarterly reports on Form 10-Q or annual reports on Form 10-K with the SEC and ending on the beginning of the first trading day following public disclosure of the financial results for that quarter or the full year. Attached hereto as Annex B is a list of the blackout periods for the current and next fiscal year, which shall be updated from time to time.

Special Blackout Periods. In addition, from time to time, the Company may impose special blackout periods on Restricted Persons and/or other employees if, in the judgment of the Chief Executive Officer and external legal counsel, it is likely that such person or persons have become aware of significant corporate developments that have not yet been disclosed to the public, even when trading otherwise may be permitted. If certain Restricted Persons or other employees of the Company become subject to a special blackout period, such persons are prohibited from (i) trading in Company Securities and (ii) without the consent of the Company, disclosing to others the fact that they are subject to such special blackout period. These special blackout periods may vary in length and may or may not be broadly communicated to Insiders. Unless otherwise specified, the Company will re-open trading after the second full trading day following the date of public disclosure of such significant corporate developments. If, for example, the Company imposed a special blackout period in connection with a significant corporate development that was disclosed during trading hours on Monday, the Company will not re-open trading until pre-market on Thursday, unless otherwise specified by the Company.

Pre-Clearance Procedures. Restricted Persons must obtain prior clearance from the Chief Executive Officer, or the Chief Financial Officer, or such persons' designee(s), by submitting (in writing or via email) the information contained in the Request for Clearance to Trade as set forth on Annex C attached hereto, before such person makes any purchases, sales or gifts of Company Securities, regardless of whether a blackout period is then in effect. A Request for Clearance to Trade should be submitted at least two business days in advance of the proposed transaction. In evaluating each proposed transaction, the Chief Executive Officer, or the Chief Financial Officer, or such persons' designee(s), will consult with senior management and outside counsel, if necessary, before clearing any proposed trade. The Chief Executive Officer is under no obligation to approve a transaction submitted for pre-clearance, and may determine not to permit the transaction. If a person seeks pre-clearance and permission to engage in the transaction is denied, then he or she should refrain from initiating any transaction in Company Securities. Clearance of a transaction is valid for no more than the 5-business day period (or such shorter period as may be prescribed in the pre-clearance form) immediately following receipt by such person of such clearance.

IX. Rule 10b5-1 Plans

The Company permits all directors, officers and other employees to adopt a 10b5-1 trading plan pursuant to the Company's procedure for adopting such a trading plan. All directors, officers and other employees must obtain pre-clearance prior to entering into, modifying or terminating a 10b5-1 trading plan. The restrictions on trading set forth in this Policy shall not apply to trades made pursuant to a 10b5-1 trading plan.

X. Section 16 Requirements

All officers and directors are required to comply with Section 16 of the Securities and Exchange Act of 1934, as amended, and related rules and regulations which set forth reporting obligations as well as limitations on "short swing" transactions. The Company will make all required Section 16 reports on behalf of its directors and officers, however, the obligation to comply with Section 16 is personal. Please direct any inquiries concerning Section 16 compliance to the Chief Executive Officer.

XI. Consequences of Violations

The purchase or sale of Company Securities while aware of material non-public information, or the disclosure of material, non-public information to others who then trade in Company Securities, is prohibited by federal and state securities laws. Insider trading violations are pursued vigorously by the SEC, U.S. Attorneys and state enforcement authorities, as well as the laws of foreign jurisdictions. Punishment for insider trading violations is severe, and could include significant fines and imprisonment. While the regulatory authorities concentrate their efforts on the individuals who trade, or who tip material non-public information to others who trade, the federal securities laws also impose potential liability on companies and other "controlling persons" within the organization if they fail to take reasonable steps to prevent insider trading by company personnel.

In addition, an individual's failure to comply with this Policy may subject the individual to Company-imposed sanctions, including dismissal for cause, whether or not the employee's failure to comply results in a violation of law. A violation of law, or even an SEC investigation that does not result in prosecution, can tarnish a person's reputation and irreparably damage a career.

XII. Administration of the Policy

The Company's Chief Executive Officer, and in such person's absence, an employee designated by the Chief Executive Officer, in consultation with internal and external legal counsel, shall be responsible for administration of this Policy. All determinations and interpretations by the Chief Executive Officer (or his or her designees) shall be final and not subject to further review.

Any person who has a question about this Policy or its application to any proposed transaction may obtain additional guidance from the Chief Executive Officer.

XIII. Certification

You must sign, date and return the Certification set forth on Annex D attached hereto (or such other certification as the Chief Executive Officer may deem appropriate) stating that you have received, read, understand and agree to comply with this Policy. The Company may require you to sign such a Certification on an annual basis, which Certification may be in electronic format. Please note that you are bound by the Policy whether or not you sign the Certification.

Annex A

Restricted Persons

- All Employees

Directors:

- Wesley Kaupinen
- George M. Jenkins
- Todd C. Davis
- Tadd Wessel
- Christopher Kiritsy
- Elaine J. Heron

Annex B

[***]

Annex C

Request for Clearance to Trade

To: Palvella Therapeutics, Inc.
125 Strafford Avenue, Suite 360
Wayne, PA 19087

Attention: Chief Executive Officer
Phone Number:
E-mail:

Name: _____ Title: _____

I hereby request clearance for myself (or a member of my immediate family or household) to execute the following transaction relating to the securities of Palvella Therapeutics, Inc.

Type of Transaction:

☐ I wish to purchase shares of common stock. Number of shares of common stock to be purchased: _____

☐ I wish to sell shares of common stock. Number of shares of common stock to be sold: _____

☐ I wish to gift shares of common stock. Number of shares of common stock to be gifted: _____

☐ Other: _____

Expiration Date for Transaction: _____

If the request is for a member of my immediate family or household:

Name of Person: _____ Relationship: _____

I hereby represent that I am not aware of any material, non-public information concerning Palvella Therapeutics, Inc. or its subsidiaries at the time of submitting this request and I agree that should I become aware of any material, non-public information concerning Palvella Therapeutics, Inc. or its subsidiaries prior to consummating the approved transaction, I will not consummate such transaction.

I understand that once approved, the authorization is valid on the date of approval and during the remaining term of the trading window in which it is approved. I further understand that the approval will lapse if, in the judgment of the Chief Executive Officer, I am likely to be aware of material, non-public information or at the expiration of the trading window in which approval is granted, whichever is the first to occur.

Date	Signature

Approved by:

Chief Executive Officer	Date

Annex D

Certification

I hereby certify that:

1. I have read and understand Palvella Therapeutics, Inc.'s (the "Company") Insider Trading Policy (the "Policy"). I understand that the Chief Executive Officer is available to answer any questions I have regarding the Policy.

2. Since I have been affiliated with the Company, I have complied with the Policy.

3. I will continue to comply with the Policy for as long as I am subject to the Policy.

Print name: _____

Signature: _____

Date: _____

Exhibit 21.1

Subsidiaries

Entity	Jurisdiction of Organization
Palvella Therapeutics, Inc.	Delaware
Pieris Pharmaceuticals GmbH	Germany

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1) Registration Statement (Form S-3 No. 333-292544) of Palvella Therapeutics, Inc.; and
2) Registration Statement (Form S-8 No. 333-285029) pertaining to the 2019 Equity Incentive Plan and 2024 Equity Incentive Plan of Palvella Therapeutics, Inc.

of our report dated March 31, 2026, with respect to the consolidated financial statements of Palvella Therapeutics, Inc. included in this Annual Report (Form 10-K) of Palvella Therapeutics, Inc. for the year ended December 31, 2025.

/s/ Ernst & Young LLP
Philadelphia, Pennsylvania
March 31, 2026

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Wesley Kaupinen, certify that:

1. I have reviewed this Annual Report on Form 10-K of Palvella Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2026

By: /s/ Wesley H. Kaupinen

Wesley H. Kaupinen
President and Chief Executive Officer

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew Korenberg, certify that:

1. I have reviewed this Annual Report on Form 10-K of Palvella Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 31, 2026

By: /s/ Matthew E. Korenberg

Matthew E. Korenberg
Chief Financial Officer and Treasurer

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Palvella Therapeutics, Inc. (the "Company") on Form 10-K for the year ended December 31, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to such officer's knowledge:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)); and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 31, 2026

By: /s/ Wesley H. Kaupinen

Wesley H. Kaupinen
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Palvella Therapeutics, Inc. (the "Company") on Form 10-K for the year ended December 31, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer of the Company certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to such officer's knowledge:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)); and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 31, 2026

By: /s/ Matthew E. Korenberg
Matthew E. Korenberg
Chief Financial Officer and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 97.1

PALVELLA THERAPEUTICS, INC.

DODD-FRANK CLAWBACK POLICY

The Board of Directors (the "Board") of Palvella Therapeutics, Inc. (the "Company") has adopted the following Dodd-Frank Clawback Policy (this "Policy") on March 28, 2025 (the "Effective Date").

1. Purpose. The purpose of this Policy is to provide for the recoupment of certain incentive compensation pursuant to Section 954 of the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, in the manner required by Section 10D of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), Rule 10D-1 promulgated thereunder, and the Applicable Listing Standards (as defined below) (collectively, the "Dodd-Frank Rules").

2. Administration. This Policy shall be administered by the Compensation Committee of the Board (the "Compensation Committee"). Any determinations made by the Compensation Committee shall be recommended to the Board for approval. Upon full Board approval, the determinations will be final and binding on all affected individuals.

3. Definitions. For purposes of this Policy, the following capitalized terms shall have the meanings set forth below.

(a) "***Accounting Restatement***" shall mean an accounting restatement of the Company's financial statements due to the material noncompliance of the Company with any financial reporting requirement under the securities laws, including any required accounting restatement (i) to correct an error in previously issued financial statements that is material to the previously issued financial statements (*i.e.,* a "Big R" restatement), or (ii) that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period (*i.e.,* a "little r" restatement).

(b) "***Affiliate***" shall mean each entity that directly or indirectly controls, is controlled by, or is under common control with the Company.

(c) "***Applicable Exchange"*** shall mean (i) The Nasdaq Stock Market, if the Company's securities are listed on such national stock exchange, (ii) the New York Stock Exchange, if the Company's securities are listed on such national stock exchange or (iii) the NYSE American, if the Company's securities are listed on such national stock exchange.

(d) "***Applicable Listing Standards***" shall mean (i) Nasdaq Listing Rule 5608, if the Company's securities are listed on The Nasdaq Stock Market, (ii) Section 303A.14 of the New York Stock Exchange Listed Company Manual, if the Company's securities are listed on the New York Stock Exchange or (iii) Section 811 to the NYSE American Company Guide if the Company's securities are listed on the NYSE American.

(e) "***Clawback Eligible Incentive Compensation***" shall mean Incentive-Based Compensation Received by a Covered Executive (i) on or after the Effective Date, (ii) after beginning service as a Covered Executive, (iii) if such individual served as a Covered Executive at any time during the performance period for such Incentive-Based Compensation (irrespective

of whether such individual continued to serve as a Covered Executive upon or following the Restatement Trigger Date), (iv) while the Company has a class of securities listed on a national securities exchange or a national securities association, and (v) during the applicable Clawback Period. For the avoidance of doubt, Incentive-Based Compensation Received by a Covered Executive on or after the Effective Date could, by the terms of this Policy, include amounts approved, awarded, or granted prior to such date.

(f) "***Clawback Period***" shall mean, with respect to any Accounting Restatement, the three completed fiscal years of the Company immediately preceding the Restatement Trigger Date and any transition period (that results from a change in the Company's fiscal year) within or immediately following those three completed fiscal years (except that a transition period between the last day of the Company's previous fiscal year end and the first day of its new fiscal year that comprises a period of at least nine months shall count as a completed fiscal year).

(g)"***Company Group***" shall mean the Company and its Affiliates.

(h)"***Covered Executive***" shall mean any "executive officer" of the Company as defined under the Dodd-Frank Rules, and, for the avoidance of doubt, includes each individual identified as an executive officer of the Company in accordance with Item 401(b) of Regulation S-K under the Exchange Act.

(i) "***Erroneously Awarded Compensation***" shall mean the amount of Clawback Eligible Incentive Compensation that exceeds the amount of Incentive-Based Compensation that otherwise would have been Received had it been determined based on the restated amounts, computed without regard to any taxes paid. With respect to any compensation plan or program that takes into account Incentive-Based Compensation, the amount contributed to a notional account that exceeds the amount that otherwise would have been contributed had it been determined based on the restated amount, computed without regard to any taxes paid, shall be considered Erroneously Awarded Compensation, along with earnings accrued on that notional amount.

(j) "***Financial Reporting Measures***" shall mean measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and all other measures that are derived wholly or in part from such measures. Stock price and total shareholder return (and any measures that are derived wholly or in part from stock price or total shareholder return) shall for purposes of this Policy be considered Financial Reporting Measures. For the avoidance of doubt, a measure need not be presented in the Company's financial statements or included in a filing with the U.S. Securities and Exchange Commission (the "SEC") in order to be considered a Financial Reporting Measure.

(k)"***Incentive-Based Compensation***" shall mean any compensation that is granted, earned or vested based wholly or in part upon the attainment of a Financial Reporting Measure.

(l) "***Received***" shall mean the deemed receipt of Incentive-Based Compensation. Incentive-Based Compensation shall be deemed received for this purpose in the Company's fiscal period during which the Financial Reporting Measure specified in the applicable Incentive-Based Compensation award is attained, even if payment or grant of the Incentive-Based Compensation occurs after the end of that period.

(m)"***Restatement Trigger Date***" shall mean the earlier to occur of (i) the date the Board, a committee of the Board, or the officer(s) of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare an Accounting Restatement, or (ii) the date a court, regulator or other legally authorized body directs the Company to prepare an Accounting Restatement.

4. Recoupment of Erroneously Awarded Compensation. Upon the occurrence of a Restatement Trigger Date, the Company shall recoup Erroneously Awarded Compensation reasonably promptly, in the manner described below. For the avoidance of doubt, the Company's obligation to recover Erroneously Awarded Compensation under this Policy is not dependent on if or when restated financial statements are filed following the Restatement Trigger Date.

(a) ***Process.*** The Compensation Committee shall use the following process for recoupment:

(i) First, the Compensation Committee will determine the amount of any Erroneously Awarded Compensation for each Covered Executive in connection with such Accounting Restatement. For Incentive-Based Compensation based on (or derived from) stock price or total shareholder return where the amount of Erroneously Awarded Compensation is not subject to mathematical recalculation directly from the information in the applicable Accounting Restatement, the amount shall be determined by the Compensation Committee based on a reasonable estimate of the effect of the Accounting Restatement on the stock price or total shareholder return upon which the Incentive-Based Compensation was Received (in which case, the Company shall maintain documentation of such determination of that reasonable estimate and provide such documentation to the Applicable Exchange).

(ii) Second, the Compensation Committee will inform the Board of its determination of any Erroneously Awarded Compensation. Following Board approval, the Compensation Committee will provide each affected Covered Executive with a written notice stating the amount of the Erroneously Awarded Compensation, a demand for recoupment, and the means of recoupment that the Company will accept.

(b) ***Means of Recoupment.*** The Compensation Committee shall have discretion to determine the appropriate means of recoupment of Erroneously Awarded Compensation, which may include without limitation: (i) recoupment of cash or shares of Company stock, (ii) forfeiture of unvested cash or equity awards (including those subject to service-based and/or performance-based vesting conditions), (iii) cancellation of outstanding vested cash or equity awards (including those for which service-based and/or performance-based vesting conditions have been satisfied), (iv) to the extent consistent with Section 409A of the Internal Revenue Code of 1986, as amended ("Section 409A"), offset of other amounts owed to the Covered Executive or forfeiture of deferred compensation, (v) reduction of future compensation, and (vi) any other remedial or recovery action permitted by law. Notwithstanding the foregoing, the Company Group makes no guarantee as to the treatment of such amounts under Section 409A, and shall have no liability with respect thereto. For the avoidance of doubt, appropriate means of recoupment may include amounts approved, awarded, or granted prior to the Effective Date. Except as set forth in Section 4(d) below, in no event may the Company Group accept an amount that is less than the amount of

Erroneously Awarded Compensation in satisfaction of a Covered Executive's obligations hereunder.

(c) ***Failure to Repay.*** To the extent that a Covered Executive fails to repay all Erroneously Awarded Compensation to the Company Group when due (as determined in accordance with Section 4(a) above), the Company shall, or shall cause one or more other members of the Company Group to, take all actions reasonable and appropriate to recoup such Erroneously Awarded Compensation from the applicable Covered Executive. The applicable Covered Executive shall be required to reimburse the Company Group for any and all expenses reasonably incurred (including legal fees) by the Company Group in recouping such Erroneously Awarded Compensation.

(d) ***Exceptions.*** Notwithstanding anything herein to the contrary, the Company shall not be required to recoup Erroneously Awarded Compensation if one of the following conditions is met and the Compensation Committee determines that recoupment would be impracticable:

(i) The direct expense paid to a third party to assist in enforcing this Policy against a Covered Executive would exceed the amount to be recouped, after the Company has made a reasonable attempt to recoup the applicable Erroneously Awarded Compensation, documented such attempts, and provided such documentation to the Applicable Exchange;

(ii) Recoupment would violate home country law where that law was adopted prior to November 28, 2022, provided that, before determining that it would be impracticable to recoup any amount of Erroneously Awarded Compensation based on violation of home country law, the Company has obtained an opinion of home country counsel, acceptable to the Applicable Exchange, that recoupment would result in such a violation and a copy of the opinion is provided to the Applicable Exchange; or

(iii) Recoupment would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees, to fail to meet the requirements of 26 U.S.C. 401(a)(13) or 26 U.S.C. 411(a) and regulations thereunder.

5. Reporting and Disclosure. The Company shall file all disclosures with respect to this Policy in accordance with the requirements of the Dodd-Frank Rules.

6. Indemnification Prohibition. No member of the Company Group shall be permitted to indemnify any current or former Covered Executive against (i) the loss of any Erroneously Awarded Compensation that is recouped pursuant to the terms of this Policy, or (ii) any claims relating to the Company Group's enforcement of its rights under this Policy. The Company may not pay or reimburse any Covered Executive for the cost of third-party insurance purchased by a Covered Executive to fund potential recoupment obligations under this Policy.

7. Acknowledgment. To the extent required by the Compensation Committee, each Covered Executive shall be required to sign and return to the Company the acknowledgement form attached hereto as Exhibit A pursuant to which such Covered Executive will agree to be bound by the terms of, and comply with, this Policy. For the avoidance of doubt, each Covered Executive will be fully bound by, and must comply with, the Policy, whether or not such Covered Executive has executed and returned such acknowledgment form to the Company.

8. Interpretation. The Compensation Committee is authorized to interpret and construe this Policy and to make all determinations necessary, appropriate, or advisable for the administration of this Policy. The Compensation Committee intends that this Policy be interpreted consistent with the Dodd-Frank Rules.

9. Amendment; **Termination**. The Board may amend or terminate this Policy from time to time in its discretion, including as and when it determines that it is legally required to do so by any federal securities laws, SEC rule or the rules of any national securities exchange or national securities association on which the Company's securities are listed.

10. Other Recoupment Rights. The Compensation Committee intends that this Policy be applied to the fullest extent of the law. The Compensation Committee may require that any employment agreement, equity award, cash incentive award, or any other agreement entered into be conditioned upon the Covered Executive's agreement to abide by the terms of this Policy. Any right of recoupment under this Policy is in addition to, and not in lieu of, any other remedies or rights of recoupment that may be available to the Company Group, whether arising under applicable law, regulation or rule, pursuant to the terms of any other policy of the Company Group, pursuant to any employment agreement, equity award, cash incentive award, or other agreement applicable to a Covered Executive, or otherwise (the "Separate Clawback Rights"). Notwithstanding the foregoing, there shall be no duplication of recovery of the same Erroneously Awarded Compensation under this Policy and the Separate Clawback Rights, unless required by applicable law.

11. Successors. This Policy shall be binding and enforceable against all Covered Executives and their beneficiaries, heirs, executors, administrators or other legal representatives.

Exhibit A

PALVELLA THERAPEUTICS, INC. DODD-FRANK CLAWBACK POLICY

ACKNOWLEDGEMENT FORM

By signing below, the undersigned acknowledges and confirms that the undersigned has received and reviewed a copy of the Palvella Therapeutics, Inc. Dodd-Frank Clawback Policy (the "***Policy***"). Capitalized terms used but not otherwise defined in this Acknowledgement Form (this "***Acknowledgement Form***") shall have the meanings ascribed to such terms in the Policy.

By signing this Acknowledgement Form, the undersigned acknowledges and agrees that the undersigned is and will continue to be subject to the Policy and that the Policy will apply both during and after the undersigned's employment with the Company Group. Further, by signing below, the undersigned agrees to abide by the terms of the Policy, including, without limitation, by returning any Erroneously Awarded Compensation to the Company Group reasonably promptly to the extent required by, and in a manner permitted by, the Policy, as determined by the Compensation Committee of the Company's Board of Directors in its sole discretion.

Sign: _____
Name: [Employee]

Date: _____