Palvella Therapeutics Announces Positive Topline Results from Phase 3 SELVA Clinical Study of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) in Microcystic Lymphatic Malformations

February 24, 2026

Primary endpoint met with statistically significant improvement (mean change of +2.13; p<0.001) on the Microcystic Lymphatic Malformation Investigator Global Assessment (mLM-IGA)

Achieved statistical significance on pre-specified key secondary endpoint (p<0.001) and all four secondary efficacy endpoints (all p<0.001)

95% of trial participants aged ≥ 6 who completed the efficacy evaluation period improved on the mLM-IGA at Week 24

86% of trial participants aged ≥ 6 who completed the efficacy evaluation period were rated as "Much Improved" (+2) or "Very Much Improved" (+3) on the mLM-IGA at Week 24

QTORIN™ rapamycin was well-tolerated, with no drug-related serious adverse events reported and systemic rapamycin levels below 2ng/mL at all timepoints for all participants

98% of participants who completed the efficacy evaluation period elected to continue to receive QTORIN™ rapamycin in the ongoing treatment extension period

Palvella plans to submit a New Drug Application to FDA in the second half of 2026

QTORIN™ rapamycin has the potential to become the first FDA-approved therapy and standard of care for the estimated more than 30,000 individuals with microcystic lymphatic malformations in the U.S.

Company to host webcast conference call at 8:00am ET today

WAYNE, Pa., Feb. 24, 2026 (GLOBE NEWSWIRE) -- (Nasdaq: PVLA) Palvella Therapeutics, Inc. (Palvella or "the Company"), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat participants suffering from serious, rare skin diseases and vascular malformations for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced positive topline results from the Company's Phase 3 SELVA study of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs). The Phase 3 trial met its primary endpoint with statistically significant improvement on the Microcystic Lymphatic Malformation Investigator Global Assessment (mLM-IGA) (p<0.001). The study also met its pre-specified key secondary endpoint (p<0.001) and all four additional secondary endpoints with statistical significance (all p<0.001).

Based on these results, Palvella plans to submit a New Drug Application (NDA) to FDA for QTORIN™ rapamycin for patients with microcystic LMs in the second half of 2026, with potential U.S. approval for QTORIN™ rapamycin in the first half of 2027. If approved, QTORIN™ rapamycin would be the first approved therapy for patients with microcystic LMs.

SELVA is 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 are 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.

Topline efficacy results from SELVA are as follows:

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)

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.

“The SELVA Phase 3 results represent an important milestone for individuals living with microcystic lymphatic malformations,” said Joyce M. Teng, M.D., Ph.D., Professor of Dermatology and Pediatrics at Stanford University School of Medicine and one of the SELVA Principal Investigators. “For the first time, we have robust, statistically significant Phase 3 data showing that a pharmacologic targeted therapy can meaningfully improve disease severity in this chronically debilitating condition. Microcystic LMs are a congenital, progressive disease. Lesions can cause leakage, recurrent infections, and functional impairment, which can have a profound impact on patients’ quality of life. Interventions such as surgery and laser are painful and associated with recurrence, and therefore repeated treatments are often needed. The SELVA results highlight QTORIN™ rapamycin’s potential to be a much-needed therapy for children and adults with microcystic LMs who currently have no FDA-approved treatment.”

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.

Of the 50 participants who initiated treatment, 44 participants (88%) completed the 24-week efficacy evaluation period. Four participants discontinued for reasons unrelated to adverse events, one participant discontinued due to an adverse event not related to study drug, and one participant discontinued due to an adverse event (lymphorrhea) possibly related to study drug. Following completion of the efficacy evaluation period, 43 of 44 eligible participants (98%, inclusive of those aged ≥ 3 years) elected to continue QTORIN™ rapamycin in the ongoing treatment extension period.

“We are deeply grateful to the participants, families, caregivers, investigators, and study teams who made the SELVA trial possible,” said Wes Kaupinen, Founder and Chief Executive Officer of Palvella Therapeutics. “The positive topline results from SELVA mark a significant milestone for Palvella and for the estimated more than 30,000 diagnosed patients in the U.S. living with microcystic LMs, a serious, rare, and chronically debilitating disease with no FDA-approved therapies. These data support the potential for QTORIN™ rapamycin to become the first FDA-approved therapy for microcystic LMs as we advance toward submission of a planned NDA in the second half of 2026. The results reinforce our conviction in QTORIN™ rapamycin, validate the QTORIN™ platform, and advance our vision to become the leading rare disease biopharma company serving patients with serious, rare skin diseases and vascular malformations.”

QTORIN™ rapamycin has received Breakthrough Therapy, Orphan Drug, and Fast Track designations from the FDA for the treatment of microcystic LMs, as well as an FDA Orphan Products Development grant. Palvella plans to present detailed results from the SELVA study at upcoming medical meetings.

Palvella is also advancing QTORIN™ rapamycin in other serious, rare skin diseases and vascular malformations driven by hyperactivation of the mammalian target of rapamycin (mTOR) pathway, including cutaneous venous malformations and clinically significant angiokeratomas, both of which have been granted FDA Fast Track Designation.

Conference Call Details

Palvella will host a conference call and live audiovisual webcast to discuss the Phase 3 SELVA topline results at 8:00 a.m. ET today. To access the live webcast of the call with slides, please click here or visit the "Events & Presentations" section of

Palvella's website. To access the call by phone, please use this registration link, and you will be provided with dial in details. A replay of the webcast will be available approximately 2 hours after the conclusion of the call and archived for 90 days under the "Events & Presentations" section of the Company's website at www.palvellatx.com.

About Microcystic Lymphatic Malformations

Microcystic LMs are a rare, chronically debilitating genetic disease caused by dysregulation of the phosphatidylinositol 3-kinase (PI3K)/mammalian target of rapamycin (mTOR) pathway. The condition is characterized by malformed lymphatic vessels that can protrude through the skin and persistently leak lymph fluid (lymphorrhea) and bleed, often leading to recurrent serious infections and cellulitis that can cause hospitalization. The natural history of microcystic LMs is persistent and progressive without spontaneous resolution, with symptoms generally worsening over time, including increases in the number and size of malformed vessels that lead to complications and lifetime morbidity. There are currently no FDA-approved treatments for the estimated more than 30,000 diagnosed patients with microcystic LMs in the United States.

About Palvella Therapeutics

Founded and led by rare disease drug development veterans, Palvella Therapeutics, Inc. (Nasdaq: PVLA) is a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat participants suffering from serious, rare skin diseases and vascular malformations for which there are no FDA-approved therapies. Palvella is developing a broad pipeline of product candidates based on its patented QTORIN™ platform, with an initial focus on serious, rare skin diseases, many of which are lifelong in nature. Palvella's lead product candidate, QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin), is currently being developed for the treatment of microcystic lymphatic malformations, cutaneous venous malformations, and clinically significant angiokeratomas. Palvella's second product candidate, QTORIN™ pitavastatin, is currently being developed for the topical treatment of disseminated superficial actinic porokeratosis. For more information, please visit www.palvellatx.com or follow Palvella on LinkedIn or X (formerly known as Twitter).

QTORIN™ rapamycin and QTORIN™ pitavastatin are for investigational use only and neither has been approved by the FDA o by any other regulatory agency for any indication.

Forward-Looking Statements

This press release contains forward-looking statements (including within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended (Securities Act)). These statements may discuss goals, intentions, and expectations as to future plans, trends, events, results of operations or financial condition, or otherwise, based on current beliefs of the management of Palvella, as well as assumptions made by, and information currently available to, the management of Palvella. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions or the negative or plural of these words, or other similar expressions that are predictions or indicate future events or prospects, although not all forward-looking statements contain these words. Statements that are not historical facts are forward-looking statements. Forward-looking statements include, but are not limited to, statements regarding the expected timing of the presentation of data from ongoing clinical trials, including the TOIVA study, Palvella's clinical development plans and related anticipated development milestones, Palvella's plans to pursue Breakthrough Therapy Designation, Palvella's plans to meet with regulatory authorities, Palvella's cash, financial resources and expected runway, Palvella's expectations regarding its programs, including QTORIN™ rapamycin and QTORIN™ pitavastatin, and its research-stage opportunities, including its expected therapeutic potential and market opportunity. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: the ability to raise additional capital to finance operations; the ability to advance product candidates through preclinical and clinical development; the ability to obtain regulatory approval for, and ultimately commercialize, Palvella's product candidates, including QTORIN™ rapamycin and QTORIN™ pitavastatin; the outcome of early clinical trials for Palvella's product candidates, including the ability of those trials to satisfy relevant governmental or regulatory requirements; the fact that data and results from clinical studies may not necessarily be indicative of future results; Palvella's limited experience in designing clinical trials and lack of experience in conducting clinical trials; the ability to identify and pivot to other programs, product candidates, or indications that may be more profitable or successful than Palvella's current product candidates; the substantial competition Palvella faces in discovering, developing, or commercializing products; the negative impacts of global events on operations, including ongoing and planned clinical trials and ongoing and planned preclinical studies; the ability to attract, hire, and retain skilled executive officers and employees; the ability of Palvella to protect its intellectual property and proprietary technologies; reliance on third parties, contract manufacturers, and contract research organizations; and the risks and uncertainties described in the filings made by Palvella with the Securities and Exchange Commission (SEC), including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the SEC and available at www.sec.gov. The events and circumstances reflected in our forward-looking statements may not be achieved or occur, and actual results could differ materially from those projected in the forward-looking statements. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties that Palvella may face. Except as required by applicable law, Palvella does not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. This press release contains hyperlinks to information that is not deemed to be incorporated by reference into this press release.

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