Palvella Therapeutics Reports Second Quarter 2025 Financial Results and Provides Corporate Update

August 14, 2025

Phase 3 SELVA trial evaluating QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for microcystic lymphatic malformations completed enrollment, exceeding enrollment target by over 25%; top-line results on track for the first quarter of 2026

Top-line results for Phase 2 TOIVA trial evaluating QTORIN™ rapamycin for cutaneous venous malformations remain on track for the fourth quarter of 2025

QTORIN™ rapamycin has the potential to be the first approved therapy and standard of care in the U.S. for microcystic lymphatic malformations and cutaneous venous malformations

Company plans to announce both a third clinical indication for QTORIN™ rapamycin and a second QTORIN™ platform candidate before year-end 2025

Cash and cash equivalents of $70.4 million as of June 30, 2025 are expected to fund operations into the second half of 2027

Company to host conference call at 8:30 a.m. ET today

WAYNE, Pa., Aug. 14, 2025 (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 patients suffering from serious, rare genetic skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, reported financial results for the second quarter ending June 30, 2025 and provided a corporate update.

"In the second quarter of 2025, we continued to advance Palvella's mission of pioneering first-in-disease therapies for individuals with serious, rare genetic skin diseases," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "We remain on track for two near-term data readouts for QTORIN™ rapamycin, including our Phase 3 SELVA study in microcystic LMs which we believe will support a New Drug Application submission in 2026. In parallel, we are preparing to announce two new development programs in 2025, further expanding our rare disease pipeline. With strong momentum and a solid financial foundation, Palvella is well-positioned for continued growth and execution on our vision."

Recent Research and Development Highlights

QTORIN™ rapamycin for the treatment of microcystic lymphatic malformations (microcystic LMs)

- Completed enrollment in the Phase 3 SELVA trial, a single-arm, baseline-controlled clinical trial of QTORIN™ rapamycin for the treatment of microcystic LMs; trial enrolled 51 subjects, exceeding its original target of 40 subjects by over 25%.
- The Company received initial proceeds from an FDA Orphan Products Grant which could total up to $2.6 million to support the Phase 3 SELVA trial. The FDA Orphan Products Grants Program provides non-dilutive funding to advance the development of promising therapies for rare diseases.
- Key presentations and posters featured at major scientific meetings included:
  - Oral presentation highlighting QTORIN™ rapamycin for the treatment of microcystic LMs was presented by Amy Paller, M.S., M.D., Chair of Dermatology at Northwestern University's Feinberg School of Medicine, at the 15th World Congress of Pediatric Dermatology; the presentation highlighted the Phase 3 SELVA trial design and reviewed Phase 2 results.
  - Poster highlighting the estimated diagnosed U.S. prevalence and U.S. annual incidence of lymphatic malformations was presented at the 82nd Annual Meeting of the Society for Investigative Dermatology.
- The United States Patent and Trademark Office (USPTO) issued patent No. 12,268,673 with claims to QTORIN™ rapamycin for the treatment of microcystic LMs with anticipated patent life extending into 2038.
- Top-line results from SELVA are on track for the first quarter of 2026.

QTORIN™ rapamycin for the treatment of cutaneous venous malformations (cutaneous VMs)

- Continued execution of ongoing Phase 2 TOIVA trial, a single-arm, open-label, baseline-controlled clinical trial evaluating QTORIN™ rapamycin for the treatment of cutaneous VMs. Enrollment is ongoing, targeting approximately 15 subjects.
- The Company, in collaboration with key opinion leaders in treating venous malformations, completed a nationally representative, blinded, real-world observational study estimating the U.S. annual prevalence of cutaneous VMs, with findings submitted to a peer reviewed journal.
- Top-line results from TOIVA are on track for the fourth quarter of 2025.

QTORIN™ pipeline development

- The expansion of QTORIN™ rapamycin into a third serious, rare skin disease with no FDA-approved therapies is progressing, with clinical and regulatory planning ongoing, and announcement of the program is planned for the second half of 2025.
- The second product candidate from the QTORIN™ platform for a serious, rare skin disease with no FDA-approved therapies, has achieved key pre-clinical development milestones, and announcement of the program is planned for the second half of 2025.

Recent Corporate Highlights

- Wes Kaupinen, Palvella's Founder and CEO, participated in the FDA's CEO Forum with Commissioner Marty A. Makary, M.D., M.P.H, and other senior FDA officials on June 5th.
- Strengthened executive leadership team with the appointment of Ashley Kline, a rare disease commercial veteran, as Chief Commercial Officer. Ashley previously led the successful launch of Oxervate®, a first-in-disease topical therapy for neurotrophic keratitis, a rare, serious, and progressive eye disease that previously had no FDA-approved therapies.
- The USPTO issued patent No. 12,329,748 encompassing a wide range of composition and method-of-use claims for QTORIN™ rapamycin and other mTOR inhibitors formulated in 0.1–20% anhydrous compositions for a broad spectrum of rare and common skin diseases, with patent term expected through at least 2038.
- The Company was added to the Russell 3000® and Russell 2000® indexes on June 27, 2025.

Second Quarter 2025 Financial Results

- Cash and cash equivalents as of June 30, 2025 were $70.4 million. Palvella expects such resources will be sufficient to fund its operations into the second half of 2027, and sufficient to accomplish its current strategic agenda.
- Research and development expenses were $5.1 million for the three months ended June 30, 2025, as compared to $1.4 million for the three months ended June 30, 2024. The increase in research and development expenses was primarily due to increased spending on the clinical development of QTORIN™ rapamycin for the treatment of microcystic LMs and cutaneous VMs, including conducting the Phase 3 SELVA and Phase 2 TOIVA trials, which were initiated in 2H 2024.
- General and administrative expenses were $4.1 million for the three months ended June 30, 2025, as compared to $1.5 million for the three months ended June 30, 2024. The increase in general and administrative expenses was primarily driven by increased employee compensation expense due to headcount additions, as well as increases in expenses related to operating as a publicly-traded company.
- Net loss attributable to common stockholders was $9.5 million, or $0.86 per basic and diluted share, for the three months ended June 30, 2025, as compared to net loss attributable to common stockholders of $4.4 million, or $2.47 per basic and diluted share, for the three months ended June 30, 2024.
- Shares outstanding were 13,735,390 as of August 8, 2025, including 11,059,665 shares of common stock and 2,675,725 common share equivalents assuming conversion of outstanding preferred shares and prefunded warrants.

Conference Call Details

Palvella will host a conference call and live audiovisual webcast to discuss the Company's second quarter 2025 financial results and provide a corporate update at 8:30 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 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 patients suffering from serious, rare genetic skin diseases 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 genetic 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 evaluated in the Phase 3 SELVA clinical trial in microcystic lymphatic malformations and the Phase 2 TOIVA clinical trial in cutaneous venous malformations. For more information, please visit www.palvellatx.com or follow Palvella on LinkedIn or X (formerly known as Twitter).

QTORIN™ rapamycin is for investigational use only and has not been approved or cleared by the FDA or 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, Palvella's clinical development plans and related anticipated development milestones, Palvella's cash and financial resources and expected cash runway, and the potential of, and expectations regarding, Palvella's programs, including QTORIN™ rapamycin, and its research-stage opportunities and any additional indications or platform candidates, 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; 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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PALVELLA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				

Research and development	$ 5,118	$ 1,442	$ 9,192	$ 2,426
General and administrative	4,132	1,466	7,929	2,241
Total operating expenses	9,250	2,908	17,121	4,667
Loss from operations	(9,250)	(2,908)	(17,121)	(4,667)
Total other income (expense), net	(221)	(1,264)	(535)	(2,041)
Net loss	$ (9,471)	$ (4,172)	$ (17,656)	$ (6,708)
Less: Cumulative Series D preferred dividends	—	(194)	—	(388)
Net loss attributable to common stockholders	$ (9,471)	$ (4,366)	$ (17,656)	$ (7,096)
Net loss per share — basic and diluted	$ (0.86)	$ (2.47)	$ (1.60)	$ (4.01)
Weighted-average number of common shares used in computing net loss per share — basic and diluted	11,052,741	1,770,167	11,033,327	1,770,167

PALVELLA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEET INFORMATION
(in thousands)

	June 30, 2025	December 31, 2024
Assets		
Cash and cash equivalents	$ 70,433	$ 83,602
Other current assets	3,314	4,632
Total current assets	73,747	88,234
Total assets	$ 73,747	$ 88,234
Liabilities and Stockholders' Equity		
Current liabilities	$ 9,616	$ 12,038
Non-current liabilities	16,351	13,589
Total liabilities	25,967	25,627
Total stockholders' equity	47,780	62,607
Total liabilities and stockholders’ equity	$ 73,747	$ 88,234