Palvella Therapeutics Announces Expansion of QTORIN™ Rapamycin's Development into Clinically Significant Angiokeratomas, a Rare, Chronically Debilitating Lymphatic Disease with No FDA-approved Therapies

September 24, 2025

Clinically significant angiokeratomas are characterized by lymphatic-derived skin lesions that can persistently bleed and significantly impact quality-of-life; no FDA-approved therapies exist for the estimated more than 50,000 diagnosed U.S. patients

Company plans to initiate a Phase 2 trial evaluating QTORIN™ rapamycin for clinically significant angiokeratomas in the second half of 2026

Company to host webcast conference call today, September 24, 2025 at 8:30am ET

WAYNE, Pa., Sept. 24, 2025 (GLOBE NEWSWIRE) -- (Nasdaq: PVLA) Palvella Therapeutics, Inc. (Palvella), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat patients suffering from serious, rare skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced the expansion of its QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) development program into Clinically Significan Angiokeratomas.

"Based on scientific discoveries further characterizing the biology of angiokeratomas as well as published case studies on the use of off-label rapamycin, we're excited to expand the development of QTORIN™ rapamycin into this rare, chronically debilitating disease, which currently has no FDA-approved therapies," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "Clinically significant angiokeratomas represent our third target clinical indication for QTORIN™ rapamycin and align with Palvella's strategy of selecting serious, rare skin diseases in which Palvella has the opportunity to introduce the first FDA-approved therapy."

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.

Palvella plans to meet with the FDA in the first half of 2026 to discuss 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 half of 2026. Additionally, QTORIN™ rapamycin is currently being evaluated for microcystic lymphatic malformations in the Phase 3 SELVA study and cutaneous venous malformations in the Phase 2 TOIVA study. SELVA top-line results are anticipated in the first quarter of 2026 and TOIVA top-line results are expected in mid-December 2025.

Webcast Conference Call Details

Palvella will host a conference call and live audiovisual webcast to discuss the expansion of QTORIN™ 3.9% rapamycin anhydrous gel development program to include clinically significant angiokeratomas at 8:30 a.m. ET today. The conference call format will be presentation only. To access the live webcast of the call with slides, please click here or visit the "Events & Presentations" section of Palvella's website. 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 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 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, 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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