Palvella Therapeutics Completes Enrollment in Phase 3 SELVA Trial of QTORIN™ Rapamycin for Microcystic Lymphatic Malformations, Exceeding Enrollment Target by Over 25%

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Enrollment of 51 subjects highlights significant unmet need in this serious, lifelong genetic disease which currently has no FDA-approved therapies

Top-line data expected in the first quarter of 2026

WAYNE, Pa., June 23, 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 genetic skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced the successful completion of SELVA, a Phase 3 trial of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs). The Phase 3 trial enrolled 51 subjects, exceeding the original target of 40 subjects by over 25%.

"The strong demand and over-enrollment in SELVA underscore the high interest within the scientific and clinical communities in establishing a new paradigm of localized, pathogenesis-directed therapy," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "With SELVA now fully enrolled, we remain on track to deliver top-line Phase 3 data in the first quarter of 2026 to support a planned NDA submission for QTORIN™ rapamycin as the first targeted therapy for this chronically debilitating disease."

SELVA is a 24-week, Phase 3, single-arm, baseline-controlled trial evaluating once-daily QTORIN™ rapamycin in individuals aged three years and older with microcystic LMs. Initially designed to enroll 40 subjects, the trial surpassed its enrollment target, enrolling 51 subjects at leading U.S. vascular anomaly centers. Following an 8-week baseline period and 24-week evaluation, eligible participants may continue treatment in an open-label extension study. The open-label extension study also remains open to enrolling new subjects aged three to five years who meet the study's inclusion criteria.

QTORIN™ rapamycin has been granted Breakthrough Therapy, Orphan Drug, and Fast Track designations by the FDA for the treatment of microcystic LMs. If approved, QTORIN™ rapamycin is expected to qualify for seven years of orphan drug market exclusivity in the U.S. Palvella has also been named an awardee of an FDA Orphan Products Grant from the FDA's Office of Orphan Products Development and is eligible to receive up to $2.6 million over the life of the grant to support the ongoing SELVA Phase 3 trial of QTORIN™ rapamycin.

Top-line results from SELVA are expected in the first quarter of 2026, with a New Drug Application submission planned for the second half of 2026.

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 disease is characterized by malformed lymphatic vessels that 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 during life, 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 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, 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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