Palvella Therapeutics to Expand Phase 3 SELVA Clinical Trial of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Microcystic Lymphatic Malformations to Include the Younger Pediatric Population, Children 3 to 5 Years Old

February 10, 2025

Expansion reflects Palvella's commitment to serving all patients affected by microcystic lymphatic malformations (microcystic LMs)

Company remains on track to report top line results from SELVA, a Phase 3 single-arm, baseline-controlled trial evaluating QTORIN™ rapamycin for the treatment of microcystic LMs, in Q1 2026

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

WAYNE, Pa., Feb. 10, 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 FDA-approved therapies, today announced that it will expand SELVA, the Company's Phase 3 clinical trial of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment o microcystic lymphatic malformations (microcystic LMs), to include patients ages 3 to 5 years old. Previously, trial participants were required to be at least 6 years old. This decision follows communication with the U.S. Food and Drug Administration (FDA) in which the agency deemed the Company's proposed expansion acceptable.

“Microcystic LMs is a debilitating disease that is chronic, progressive, and usually present at birth or shortly after. Patients with microcystic LMs often have lymphorrhea, bleeding, infection, pain, and disfigurement which may lead to difficulty with physical activities as well as more significant complications like cellulitis and hospitalization,” said Joyce Teng MD, PhD, Professor of Dermatology and Pediatrics at Stanford University and Principal Investigator of the SELVA Study. “Early intervention is essential to minimize disease burden during children’s development which is why I am so excited by the opportunity QTORIN rapamycin presents to the younger pediatric population.”

Palvella is currently enrolling approximately 40 patients in SELVA, a 24-week, Phase 3, single-arm, baseline-controlled trial of QTORIN™ rapamycin for the treatment of microcystic LMs. The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation to QTORIN™ rapamycin for the treatment of microcystic LMs. Additionally, the SELVA study is supported by an Orphan Products Grant of up to $2.6 million from FDA’s Office of Orphan Products Development.

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 drug disease drug development veterans, Palvella Therapeutics (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.

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 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 "Risk Factors" section of Palvella's registration statement on Form S-1 filed with the Securities and Exchange Commission on December 31, 2024 and other documents filed by Palvella from time to time with the Securities Exchange Commission. 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.

Contact Information

Investors
Wesley H. Kaupinen
Founder and CEO, Palvella Therapeutics
wes.kaupinen@palvellatx.com

Media
Stephanie Jacobson
Managing Director, Argot Partners