Palvella Therapeutics Announces QTORIN™ Rapamycin 3.9% Anhydrous Gel for the Treatment of Microcystic Lymphatic Malformations Featured in Oral Presentation 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

April 11, 2025

Presentation highlighted the recent expansion of the Phase 3 SELVA trial to include children 3 to 5 years old

Presentation reviewed clinically and statistically significant Phase 2 results and the design of the ongoing Phase 3 SELVA trial

Top-line results from SELVA remain on track for the first quarter of 2026

QTORIN™ 3.9% rapamycin anhydrous gel has the potential to be the first approved therapy and standard of care for microcystic lymphatic malformations in the U.S.

WAYNE, Pa., April 11, 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, today announced QTORIN™ rapamycin 3.9% anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs) was featured by Dr. Amy Paller in an oral presentation at the 15th World Congress of Pediatric Dermatology in Buenos Aires, Argentina. Dr. Amy Paller is the Walter J. Hamlin Professor and Chair of Dermatology, Professor of Pediatrics, and Principal Investigator of the NIH-funded Skin Biology and Diseases Resource-based Center at Northwestern University's Feinberg School of Medicine and has served as President of the Society for Investigative Dermatology (SID), the Society for Pediatric Dermatology (SPD), the International Eczema Council (IEC), the Pediatric Dermatology Research Alliance (PeDRA), and the Women's Dermatological Society (WDS).

"There is an urgent need for a safe and effective targeted topical therapy for mosaic genetic skin disorders including microcystic lymphatic malformation," said Amy Paller, M.S., M.D., Chair of Dermatology, Northwestern University's Feinberg School of Medicine. "Many patients have considerable complications associated with this disease, and I am looking forward to the Phase 3 results early next year."

The oral presentation titled, "SELVA: A Phase 3 study with a fit-for-purpose primary endpoint evaluating QTORIN™ 3.9% rapamycin anhydrous gel in the treatment of microcystic lymphatic malformations in patients 3 years of age and older," highlighted:

- Microcystic LMs are congenital mosaic lesions that gradually increase in size with risk of complications and are best managed aggressively during childhood
- Microcystic LMs are proliferative with no spontaneous regression
- QTORIN™ rapamycin, an investigational therapy designed to selectively inhibit the mammalian target of rapamycin (mTOR) in the skin, potentially reduces endothelial cell hyper-proliferation and vascular endothelial growth factor signaling, both of which are the result of overactive mTOR signaling in microcystic LMs
- A multicenter, open-label, 12-week, Phase 2 study evaluating the safety and efficacy of QTORIN™ rapamycin for microcystic LMs demonstrated:
    - 100% of participants were either "Very Much Improved" (41.7%) or "Much Improved" (58.3%) as rated by the Clinician Global Impression of Change (CGI-C), a 7-point change scale conducted by live clinician assessment
    - 83% of participants were either "Very Much Improved" (25%) or "Much Improved" (58.3%) as rated by the Patient Global Impression of Change, a 7-point change scale reported by patients
    - QTORIN™ rapamycin was generally well-tolerated; all treatment related adverse events were moderate or mild and there were no discontinuations due to adverse events.
- SELVA, a 24-week, Phase 3, single-arm, baseline-controlled clinical trial of QTORIN™ rapamycin for the treatment of microcystic LMs, mimics the Phase 2 study, with key study elements including the following:
    - Based on data from Phase 2 and clinician interviews, the primary endpoint is the fit-for-purpose Microcystic Lymphatic Malformations Investigator's Global Assessment (mLM-IGA), a 7-point change scale conducted by live clinician assessment with similarities to the CGI-C
    - Patient population enriched to include patients with moderate to severe disease
    - Target sample size of 40 subjects

- Treatment duration extended to 24-weeks
- Enrollment criteria expanded to include patients 3 years and older

"Microcystic LMs are a serious, rare, and chronically debilitating genetic disease with a pediatric onset and lifelong course. Early intervention is essential to minimizing disease burden for this patient population who currently have no FDA-approved therapies. Palvella is pleased to include patients younger than 6 years old in the ongoing Phase 3 SELVA study," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella.

SELVA is currently enrolling patients at 13 centers in the United States. Top-line data is anticipated in the first quarter of 2026. The U.S. 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 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.

Contact Information

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

Media
Marcy Nanus
Managing Partner, Trilon Advisors LLC
mnanus@trilonadvisors.com