Palvella Therapeutics Announces Publication of Results from Phase 2 Clinical Trial of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) fo the Treatment of Microcystic Lymphatic Malformations in the Journal of Vascular Anomalies

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Publication reports 100% of participants were either "Much Improved" or "Very Much Improved" as rated by the Clinician Global Impression of Change following 12-weeks of QTORIN™ rapamycin

FDA previously granted Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation to QTORIN™ rapamycin for microcystic lymphatic malformations (microcystic LMs)

Ongoing Phase 3 single-arm, baseline-controlled trial evaluating QTORIN™ rapamycin for the treatment of microcystic LMs with topline data expected 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., Jan. 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 results from the Phase 2 study of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs) were published in the *Journal of Vascular Anomalies* (JoVA). JoVA, which is the official journal of the International Society for the Study of Vascular Anomalies (ISSVA), is an international peer reviewed journal dedicated to the discovery and report of the scientific investigation, diagnosis, and treatment of congenital and acquired human vascular lesions.

"The Phase 2 results highlight QTORIN™ rapamycin's potential to be the first targeted therapy for children and adults living with microcystic lymphatic malformations, a serious, rare genetic disease," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "We look forward to further evaluating the potential of QTORIN™ rapamycin in the ongoing Phase 3 SELVA trial and to expediting this potential first-in-disease therapy to patients."

As previously reported by Palvella, the publication presents results demonstrating nominal statistical significance across several of the efficacy endpoints assessing the change from pre-treatment baseline to end of treatment (Week 12) with once daily QTORIN™ rapamycin (n=12), including clinician and patient global impression assessments as well as assessments of individual clinical manifestations that are important disease burdens for individuals living with microcystic LMs. QTORIN™ rapamycin was generally well-tolerated with no participants experiencing drug related serious adverse events. The publication, titled "Phase 2 Study of the Safety and Efficacy of Topical QTORIN™ Rapamycin for the Treatment of Cutaneous Microcystic Lymphatic Malformations", can be accessed here.

Palvella is currently enrolling approximately 40 subjects 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 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, the sufficiency of Palvella's capital resources; Palvella's cash runway; 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; the expected timing of initiating, as well as the design of Palvella's Phase 2 clinical trial of QTORIN™ rapamycin in cutaneous venous malformations. 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 definitive proxy statement/information statement dated November 8, 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.

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