Palvella Therapeutics Announces First Patients Dosed in Phase 2 TOIVA Clinical Trial of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Cutaneous Venous Malformations

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Phase 2 single-arm, baseline-controlled trial evaluating QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of cutaneous venous malformations (cutaneous VMs) to enroll approximately 15 subjects at leading vascular anomaly centers across the U.S.

Cutaneous VMs are a serious and lifelong genetic disease that can result in substantial morbidity and functional impairment of the skin affecting an estimated more than 75,000 diagnosed patients in the U.S.

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

WAYNE, Pa., Jan. 08, 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 the first patients have recently been dosed in TOIVA, a multicenter, Phase 2 clinical trial designed to evaluate the safety and efficacy of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of cutaneous venous malformations (cutaneous VMs).

“Cutaneous VMs are a serious, lifelong disease which leads to significant disease burden for children and adults living with the disease, including risk of serious complications such as bleeding, ulceration, thrombosis, and pain leading to significant impact on quality of life and daily function,” said Megha M. Tollefson, M.D., Pediatric Dermatologist and Medical Director of Mayo Clinic Vascular Malformation Clinic. “We’re excited to have the first patients dosed in the landmark Phase 2 TOIVA study evaluating QTORIN rapamycin, a targeted topical therapy with potential to inhibit the mammalian target of rapamycin (mTOR) pathway which is a causative driver of this disease. A potential new treatment option would be transformative for children and adults living with this disease, as no FDA-approved therapies currently exist.”

Cutaneous VMs are a rare genetic disease caused by mutations in genes that cause overactivation of the PI3K/mTOR signaling pathway, leading to dysfunctional veins within the skin. These malformations can cause substantial morbidity and functional impairment, significantly impact quality of life, and are associated with severe bleeding, ulceration, thrombosis, and other potential complications. An urgent need exists for an FDA-approved, targeted, localized therapy to treat cutaneous VMs. While published case studies and real-world evidence have provided preliminary evidence of clinical benefit from the off-label use of systemic mTOR inhibitors for venous malformations, there are currently no FDA-approved therapies for the estimated more than 75,000 diagnosed patients with cutaneous VMs in the U.S.

The Phase 2 TOIVA study is a single-arm, open-label, baseline-controlled clinical trial of QTORIN™ rapamycin administered topically once daily for the treatment of cutaneous VMs. Safety and tolerability will be assessed based on the incidence and severity of adverse events. This proof-of-concept study includes multiple measures of efficacy, including change from baseline to week 12 in clinician and patient global impression assessments as well as assessments of specific individual clinical manifestations which contribute to disease burden. The Phase 2 study is expected to enroll approximately 15 participants, ages six and older, at leading vascular anomaly centers across the U.S.

QTORIN rapamycin is a novel, patented 3.9% rapamycin anhydrous gel which aims to harness the potential therapeutic benefits of rapamycin, an mTOR inhibitor, while minimizing systemic exposure of rapamycin and potential adverse reactions associated with systemic therapy. In April 2024, the FDA granted Fast Track Designation to QTORIN™ rapamycin for the treatment of venous malformations.

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.

Contact Information

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

Media
Stephanie Jacobson
Managing Director, Argot Partners
palvella@argotpartners.com