Palvella Therapeutics Announces First Patients Dosed in Phase 2 LOTU Trial of Fast Track-Designated QTORIN™ Rapamycin for Clinically Significant Angiokeratomas

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Clinically significant angiokeratomas represent a rare, chronic and debilitating lymphatic malformation with no FDA-approved therapies and an estimated more than 50,000 diagnosed patients in the U.S.

Phase 2 single-arm, baseline-controlled trial expected to enroll up to 15 subjects at leading vascular anomaly centers and high-volume dermatology centers in the U.S.

Topline results from the Phase 2 trial are expected in the second half of 2027

WAYNE, Pa., May 04, 2026 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc. (Palvella or "the Company") (Nasdaq: PVLA), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat patients suffering from serious, rare skin diseases and vascular malformations for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced that the first patients have been dosed in LOTU, 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 clinically significant angiokeratomas.

"Clinically significant angiokeratomas represent a chronic, debilitating condition characterized by hyperkeratotic vascular lesions that can bleed with minor trauma, are susceptible to infection, and significantly impair patients' quality of life," said Maria Buethe, MD, PhD, Division Chief of Dermatology at Rady Children's Hospital of Orange County and Director of Pediatric Dermatology at the University of California, Irvine, and principal investigator of the LOTU study. "These lesions are especially burdensome when large or located in difficult to treat areas or when causing functional impairments. Current management relies on invasive, often destructive procedures, including laser therapy and electrocautery, which can be associated with pain, scarring, recurrence, and incomplete disease control. Angiokeratomas do not spontaneously regress and can increase in size, number, and severity over time, underscoring the need for a targeted therapeutic option."

In 2025, the International Society for the Study of Vascular Anomalies (ISSVA) classified angiokeratomas as isolated lymphatic malformations, reflecting advances in the understanding of angiokeratomas as a lymphatic disease. This classification places angiokeratomas within the same ISSVA-recognized isolated lymphatic malformation category as microcystic lymphatic malformations, the lead indication for QTORIN™ rapamycin, strengthening the scientific rationale for extending QTORIN™ rapamycin into clinically significant angiokeratomas based on shared lymphatic disease biology. Emerging evidence implicating dysregulated mammalian target of rapamycin (mTOR) and vascular endothelial growth factor (VEGF) signaling pathways in lymphatic endothelial proliferation and abnormal vascular morphology further supports the mechanistic rationale for evaluating mTOR inhibition in angiokeratomas. Published case studies and real-world evidence suggest potential benefit from off-label mTOR inhibition; however, there are currently no FDA-approved therapies for angiokeratomas.

"We continue to see broad potential for QTORIN™ rapamycin across serious, rare diseases characterized by dysregulated mTOR signaling, significant patient burden, and no FDA-approved therapies," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella Therapeutics. "Clinically significant angiokeratomas represent a meaningful rare disease opportunity, with an estimated more than 50,000 diagnosed patients in the U.S. and no FDA-approved therapies. We are advancing this program in close collaboration with our scientific and medical collaborators, informed by significant unmet need, recent advances in the understanding of angiokeratomas as a lymphatic disease, and the central role of mTOR/VEGF signaling in lymphatic vascular biology. We look forward to reporting topline results from the Phase 2 study in the second half of 2027."

The Phase 2 LOTU study is a single-arm, baseline-controlled clinical trial evaluating QTORIN™ rapamycin administered topically once daily for the treatment of clinically significant angiokeratomas. 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 clinical manifestations that contribute to disease burden. The Phase 2 LOTU study is expected to enroll up to 15 subjects, ages six and older, at leading vascular anomaly centers and high-volume dermatology centers in the U.S.

QTORIN™ rapamycin is a novel, patented 3.9% rapamycin anhydrous gel designed to harness the potential therapeutic benefits of rapamycin, an mTOR inhibitor, in affected skin, including dermal tissue where the vascular component of angiokeratomas resides, while minimizing systemic exposure and potential adverse reactions associated with systemic mTOR inhibition.

QTORIN™ rapamycin was previously granted Fast Track Designation by FDA for the treatment of angiokeratomas, reflecting its potential to address an unmet medical need in a serious disease with no FDA-approved therapies.

About Palvella Therapeutics

Founded and led by rare disease biotech 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 skin diseases and vascular malformations 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 skin diseases and vascular malformations, many of which are lifelong in nature. Palvella's lead product candidate, QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin), is currently being developed for the treatment of microcystic lymphatic malformations, cutaneous venous malformations, and clinically significant angiokeratomas. Palvella's second product candidate, QTORIN™ pitavastatin, is currently being developed for the treatment of disseminated superficial actinic porokeratosis. For more information, please visit www.palvellatx.com or follow Palvella on LinkedIn or X (formerly known as Twitter).

QTORIN™ rapamycin and QTORIN™ pitavastatin are for investigational use only and neither has been approved by the FDA o 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 clinical trials, Palvella's clinical development plans and related anticipated development milestones, Palvella's plans to pursue Breakthrough Therapy Designation, Palvella's plans to meet with regulatory authorities, Palvella's cash, financial resources and expected runway, Palvella's expectations regarding its programs, including QTORIN™ rapamycin and QTORIN™ pitavastatin, 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 and QTORIN™ pitavastatin; 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; Palvella's limited experience in commercial manufacturing; 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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