Palvella Therapeutics Granted FDA Fast Track Designation for QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Angiokeratomas

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Fast Track designation designed to facilitate the development, and expedite the review, of drugs to treat serious conditions and fill an unmet need

With Fast Track designation, QTORIN™ rapamycin for angiokeratomas may be eligible for Accelerated Approval and Priority Review in the future, if applicable criteria are met

Palvella plans to initiate a Phase 2 trial evaluating QTORIN™ rapamycin for clinically significant angiokeratomas in the second half of 2026

Angiokeratomas are characterized by lymphatic-derived skin lesions that can persistently bleed and significantly impact quality of life; no FDA-approved therapies exist for the estimated more than 50,000 diagnosed U.S. patients

WAYNE, Pa., Dec. 16, 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 skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced that the FDA has granted Fast Track Designation to QTORIN™ rapamycin for the treatment of angiokeratomas.

"We are thrilled that the FDA has granted Fast Track Designation for QTORIN™ rapamycin in angiokeratomas," said Wes Kaupinen, Founder and CEO of Palvella. "Angiokeratomas are chronic, often debilitating lesions with no FDA-approved therapies. This Fast Track designation underscores the potential of QTORIN™ rapamycin to address this significant unmet need and supports our opportunity to advance what could become the first FDA-approved therapy for these patients. We are committed to working closely with the FDA to advance development with urgency."

Clinically significant angiokeratomas are superficial vascular malformations of lymphatic origin that can cause bleeding, pain, functional impairment, and risk of infection, with no tendency for spontaneous regression. In 2025, the International Society for the Study of Vascular Anomalies reclassified angiokeratomas as an isolated lymphatic malformation, reflecting advancements in understanding of the disease's underlying biology. Current treatment options are limited to potentially destructive procedural interventions that carry meaningful risks of pain, scarring, and recurrence.

The FDA's Fast Track program is designed to facilitate the development, and expedite the review, of drugs to treat serious conditions and fill an unmet need. The purpose is to get important new drugs to the patient earlier. Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients. If relevant criteria are met, programs with Fast Track designation can become eligible for accelerated approval and priority review, both of which can reduce the timelines associated with regulatory review and action.

In September 2025, Palvella announced the expansion of its QTORIN™ rapamycin development program into clinically significant angiokeratomas. Palvella plans to meet with the FDA in the first half of 2026 to discuss the proposed design of a Phase 2 study of approximately 10–20 patients, with study initiation expected in the second half of 2026.

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 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 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 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 topical 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 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, 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; 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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