FDA Grants Rolling Review of Palvella's QTORIN™ Rapamycin NDA for Microcystic Lymphatic Malformations

June 22, 2026

FDA's rolling review process is intended to facilitate expedited review, enabling FDA to begin evaluating completed sections of the NDA before the full application is submitted

Palvella remains on track to complete the NDA submission in the second half of 2026

QTORIN™ rapamycin has the potential to become the first FDA-approved therapy and standard of care for the estimated more than 30,000 individuals with microcystic lymphatic malformations in the U.S.

WAYNE, Pa., June 22, 2026 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc. (Palvella or the "Company") (Nasdaq: PVLA), a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies for serious, rare skin diseases and vascular malformations for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, announced today that FDA has granted rolling review for the Company's New Drug Application (NDA) for QTORIN™ rapamycin for the treatment of microcystic lymphatic malformations (microcystic LMs).

"We remain on track to complete submission of the QTORIN™ rapamycin NDA in the second half of 2026 and intend to utilize the benefits of rolling review, Breakthrough Therapy and Fast Track designations to support an efficient path toward potential approval," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella Therapeutics. "Following the positive Phase 3 SELVA results, our focus is clear: move with urgency to advance QTORIN™ rapamycin as the potential first FDA-approved therapy for patients and families affected by microcystic LMs, a serious, lifelong rare disease with no approved treatment options."

Microcystic LMs are a rare, chronically debilitating genetic disease driven by dysregulation of the PI3K/mTOR pathway. Malformed lymphatic vessels can protrude through the skin, persistently leak and bleed, and cause recurrent infections, cellulitis and hospitalization. Published natural history data demonstrate that microcystic LMs are persistent and progressive and do not spontaneously regress. Surgery, sclerotherapy and laser can be limited by recurrence and repeated procedures, while systemic PI3K/mTOR inhibitors may be constrained by chronic safety and tolerability considerations, particularly in children, and by challenges in achieving sufficient exposure in affected skin. Advances in molecular genetics have established dysregulated PI3K/mTOR signaling as a central disease driver, supporting precision, mechanism-based treatment. QTORIN™ rapamycin is designed to deliver rapamycin directly to pathogenic skin tissue to achieve local, on-target inhibition of disease-driving mTOR signaling while minimizing systemic exposure. There are no FDA-approved treatments for the estimated 30,000 or more people diagnosed with microcystic LMs in the United States.

Rolling review is an FDA regulatory feature available to programs with Fast Track or Breakthrough Therapy designation and is intended to facilitate expedited FDA review of applications for therapies addressing serious conditions with unmet medical need. Rolling review allows Palvella to submit completed sections of the QTORIN™ rapamycin NDA as they become available, enabling FDA review to proceed while the Company completes the remainder of the application.

QTORIN™ rapamycin has received Breakthrough Therapy, Orphan Drug and Fast Track designations from FDA for the treatment of microcystic LMs.

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 living with 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 and anticipated timing of regulatory submissions, Palvella's plans with respect to the timing of, and anticipated FDA review process for, the NDA for QTORIN™ rapamycin, Palvella's plans to pursue Breakthrough Therapy Designation, Palvella's plans to meet with regulatory authorities, Palvella's expectations regarding the benefits of orphan drug designation and potential benefit of orphan drug exclusivity for QTORIN™ rapamycin for the treatment of microcystic lymphatic malformations, 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 make regulatory submissions on anticipated timelines; 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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