Palvella Therapeutics Announces Completion of FDA Pre-NDA Meeting for QTORIN™ Rapamycin in Microcystic Lymphatic Malformations

June 3, 2026

Palvella remains on track to submit an NDA in the second half of 2026

Microcystic lymphatic malformations are serious, chronic, lifelong vascular malformations that can cause persistent leaking, bleeding, infection, functional impairment, and significant daily burden for patients and families

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 03, 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 completion of its pre-New Drug Application (NDA) meeting with FDA for QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) in microcystic lymphatic malformations (microcystic LMs). The pre-NDA meeting addressed nonclinical, clinical pharmacology, and clinical information for the planned NDA and included an in-person discussion with FDA and receipt of official meeting minutes.

"We are pleased with the outcome of our pre-NDA meeting with FDA and encouraged as we continue advancing toward submission of an NDA for QTORIN™ rapamycin in microcystic lymphatic malformations in the second half of 2026, in line with our previous guidance," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "At the pre-NDA meeting, investigators from our Phase 3 SELVA study with expertise in vascular malformations, including a pediatric hematologist-oncologist and pediatric dermatologist, provided perspectives on the magnitude and clinical meaningfulness of the treatment effect observed in SELVA and reviewed representative before-and-after photographs from the study. Overall, we believe the results observed with QTORIN™ rapamycin in our Phase 3 SELVA and Phase 2 studies demonstrate its potential as the first targeted therapy for microcystic lymphatic malformations, a serious, lifelong disease for which patients today have no FDA-approved therapies and rely on interventional procedures and off-label treatments that do not adequately address the chronic burden of disease."

Following the in-person pre-NDA meeting with FDA and receipt of official meeting minutes, Palvella remains on track to submit its NDA for QTORIN™ rapamycin in microcystic LMs in the second half of 2026. Palvella and FDA discussed the planned NDA evidence package, which is expected to include efficacy and safety data from Palvella's Phase 3 SELVA study, including blinded independent review data and before-and-after photographs from all patients enrolled in the study, as well as supportive clinical data from the Phase 2 study that served as the basis for FDA's Breakthrough Therapy designation for QTORIN™ rapamycin in microcystic LMs. In addition, consistent with the goals of FDA's May 2026 drug repurposing initiative to help address unmet medical needs, particularly for chronic or rare diseases, the evidence package is expected to include real-world evidence from published literature and clinical experience with off-label rapamycin in microcystic LMs. Based on the official meeting minutes and the planned NDA evidence package discussed with FDA, Palvella plans to proceed with the NDA submission, supported by the existing evidence package, with no additional efficacy study planned.

QTORIN™ rapamycin has received Breakthrough Therapy, Orphan Drug, and Fast Track designations from FDA for the treatment of microcystic LMs, as well as an FDA Orphan Products Development grant.

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 and anticipated timing of regulatory submissions, 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.

Contact Information

Investors

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

Media
Marcy Nanus
Managing Partner, Trilon Advisors LLC
mnanus@trilonadvisors.com