Palvella Therapeutics Provides Corporate Update and 2026 Outlook: Advancing a Late Clinical-Stage Pipeline and Platform to Address Multiple Serious, Rare Skin Diseases and Vascular Malformations with No FDA-Approved Therapies

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Phase 3 SELVA study evaluating QTORIN™ rapamycin 3.9% anhydrous gel (QTORIN™ rapamycin) for microcystic lymphatic malformations (microcystic LMs) remains on track, with topline results anticipated in March 2026; pending positive results, an NDA submission is planned for the second half of 2026

Accelerating U.S. launch readiness for QTORIN™ rapamycin for microcystic LMs which has the potential to become the first FDA-approved therapy and a first-line, standard-of-care treatment for this serious, lifelong disease affecting an estimated more than 30,000 diagnosed patients in the U.S.

Following positive Phase 2 results for QTORIN™ rapamycin for the treatment of cutaneous venous malformations announced in December 2025, requested a Preliminary Breakthrough Therapy Designation Advice meeting with the U.S. Food and Drug Administration, anticipated in the first quarter of 2026

Phase 2 clinical studies evaluating QTORIN™ pitavastatin in disseminated superficial actinic porokeratosis and QTORIN™ rapamycin in clinically significant angiokeratomas are expected to initiate in the second half of 2026

QTORIN pipeline expansion: anticipate announcing one new QTORIN™ product candidate and a fourth clinical indication for QTORIN™ rapamycin in the second half of 2026

WAYNE, Pa., Jan. 09, 2026 (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 and vascular malformations for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today provided a corporate update and 2026 strategic outlook on the Company's late clinical-stage pipeline and QTORIN™ platform for addressing multiple serious, rare skin diseases and vascular malformations with no FDA-approved therapies.

"As we continue to advance our leadership in addressing serious, rare skin diseases and vascular malformations, we look forward to reporting Phase 3 SELVA topline results later this quarter for microcystic lymphatic malformations where QTORIN™ rapamycin has the potential, if approved, to become the first FDA-approved therapy and a first-line, standard-of-care treatment," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella Therapeutics. "Palvella now has four programs with the potential to be first-in-disease therapies for serious, rare conditions with no FDA-approved treatments in commercially attractive markets, including microcystic lymphatic malformations, cutaneous venous malformations, clinically significant angiokeratomas, and disseminated superficial actinic porokeratosis. Over the last several months, we have significantly strengthened our leadership team and are pleased with the continued progress across our R&D efforts and our commercial preparations for a potential near-term U.S. launch."

Corporate Update and 2026 Outlook

QTORIN™ rapamycin for microcystic lymphatic malformations

- Microcystic LMs are a rare, chronically debilitating genetic disease caused by dysregulation of the phosphatidylinositol 3-kinase (PI3K)/mammalian target of rapamycin (mTOR) pathway and characterized by malformed lymphatic vessels that protrude through the skin and persistently leak lymph fluid (lymphorrhea) and bleed, often leading to recurrent serious infections and cellulitis that can cause hospitalization; no FDA-approved therapies currently exist for microcystic LMs.
- In May 2025, an abstract highlighting the estimated diagnosed prevalence and annual incidence of lymphatic malformation cases in the U.S. was presented at the 82nd Annual Meeting of the Society for Investigative Dermatology, indicating an estimated 44,553 to 92,967 diagnosed U.S. patients with lymphatic malformations with cutaneous involvement.
- In June 2025, the Company surpassed its target enrollment in SELVA, a Phase 3, single-arm, baseline-controlled trial consisting of an 8-week observational baseline run-in period followed by 24 weeks of active treatment evaluating once-daily QTORIN™ rapamycin in individuals aged three years and older with microcystic LMs. The study enrolled 51 subjects, exceeding the original target of 40 subjects by over 25%. Patients who complete the 24-week efficacy evaluation

period have the opportunity to continue into a treatment extension period.
- The Phase 3 SELVA study remains on track, with topline results anticipated in March 2026.
- NDA planning is underway, with a potential submission in the second half of 2026, pending positive results from SELVA.
- FDA has previously granted Breakthrough Therapy Designation, Fast Track Designation, and Orphan Designation to QTORIN™ rapamycin for microcystic LMs; the program is also the recipient of an FDA Orphan Product Grant.

QTORIN™ rapamycin for cutaneous venous malformations (cutaneous VMs)

- 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 causing substantial morbidity and functional impairment, significantly impacting quality of life, and are associated with severe bleeding, thrombosis, pain, and other potential complications; cutaneous VMs are the most common vascular malformation, and no FDA-approved therapies currently exist for the disease.
- In October 2025, a nationally representative, retrospective, real-world, subject-blinded, physician-observational probability study published in *Orphanet Journal of Rare Diseases* indicated an estimated annual prevalence over 130,000 diagnosed U.S. patients with cutaneous only venous malformations.
- In December 2025, Palvella announced positive topline efficacy results from the Phase 2 TOIVA study, achieving statistical significance on multiple pre-specified clinician-reported and patient-reported efficacy endpoints, including dynamic change endpoints and static severity endpoints.
- In the Phase 2 TOIVA study, 73% of trial participants (11/15 participants) improved on the Overall Cutaneous Venous Malformations Investigator Global Assessment (Overall cVM-IGA) at Week 12, with 67% of trial participants (10/15 participants) rated as “Much Improved” (+2) or “Very Much Improved” (+3) on the Overall cVM-IGA at Week 12.
- In December 2025, Palvella requested a Preliminary Breakthrough Therapy Designation Advice meeting with the FDA which is anticipated for the first quarter of 2026.
- The Company plans to commence a Phase 3 pivotal study in the second half of 2026.
- FDA has previously granted Fast Track Designation to QTORIN™ rapamycin for the treatment of venous malformations.

QTORIN™ rapamycin for clinically significant angiokeratomas

- In September 2025, Palvella expanded the development of QTORIN™ rapamycin into clinically significant angiokeratomas, an isolated superficial lymphatic malformation which can cause bleeding, pain, functional impairment, and risk of infection, with no tendency for spontaneous regression; no FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed U.S. patients.
- In December 2025, the U.S. FDA granted Fast Track Designation to QTORIN™ rapamycin for angiokeratomas.
- Palvella plans to meet with the FDA in the first quarter 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.

QTORIN™ pitavastatin for disseminated superficial actinic porokeratosis (DSAP)

- In November 2025, Palvella announced a new QTORIN™ product candidate, QTORIN™ pitavastatin, for the treatment o disseminated superficial actinic porokeratosis, a premalignant genetic skin disease that presents as persistent, often extensive lesions that enlarge and increase in size, number, and extent over time, causing chronic loss of skin integrity which can severely impact quality-of-life; no FDA-approved therapies currently exist for the estimated more than 50,000 diagnosed patients in the U.S.
- QTORIN™ pitavastatin leverages Palvella’s proprietary QTORIN™ platform and is designed to be the first pathogenesis-directed therapy for DSAP by directly inhibiting the causal mevalonate pathway.
- QTORIN™ pitavastatin formulation development remains on track, with key formulation innovations implemented and the program advancing toward near-term clinical study initiation.
- Palvella plans to meet with the FDA in the first half of 2026 to discuss the proposed design of a Phase 2 clinical trial evaluating QTORIN™ pitavastatin in subjects with disseminated superficial actinic porokeratosis, with study initiation expected in the second half of 2026.

QTORIN™ rapamycin and QTORIN™ platform expansior

- Palvella plans to announce the fourth target clinical indication for QTORIN™ rapamycin in the second half of 2026. The expansion of QTORIN™ rapamycin into additional indications is supported by comprehensive publications which highlight the broad potential of rapamycin in several difficult-to-treat, mTOR-driven skin diseases while advocating for targeted, topical approaches suited to improve tolerability and safety.
- Palvella plans to announce the third product candidate from the QTORIN™ platform in the second half of 2026.

Intellectual Property

- Palvella employs a multi-layered exclusivity strategy by combining robust patent protection, covering compositions,

formulations, and methods of use, stringent trade secret programs for proprietary know-how generated during formulation development and manufacturing, and regulatory exclusivity mechanisms such as orphan drug and data exclusivity.
- In 2025, Palvella expanded its intellectual property portfolio with two U.S. patents for QTORIN™ rapamycin covering anhydrous compositions, 0.1–20% mTOR inhibitor formulations, and methods of use for rare skin diseases, strengthening the asset's multi-layered protection through 2038, and the Company filed a patent for its QTORIN™ pitavastatin program, establishing the foundation for potential exclusivity through 2046.

Leadership Team

- Key leadership hires have strengthened Palvella's commercial, research and development, medical affairs, and technical operations capabilities in support of QTORIN™ programs, including the appointments of:
  - Ashley Kline, Chief Commercial Officer, formerly U.S. General Manager at Dompé where she led the U.S. launch of OXERVATE®, a first-in-disease orphan therapy, while growing sales from initial launch to more than $500 million annually while achieving profitability in the first year.
  - David Osborne, Ph.D., Chief Innovation Officer, formerly Co-Founder and Chief Technical Officer at Arcutis Biotherapeutics, to lead product development from the QTORIN™ platform and over the course of his career has been involved in the development of more than three dozen approved topical drugs.
  - Vimal Patel, PharmD, Senior Vice President of Medical Affairs, to lead scientific engagement, KOL collaboration, disease state awareness, and medical education; Dr. Patel previously served as Head of Dermatology Medical Affairs at Incyte.
  - Jason Burdette, Senior Vice President of Technical Operations, formerly Senior Vice President of Technical Operations at Venatorx Pharmaceuticals and Aimmune Therapeutics, to oversee chemistry, manufacturing, and controls and technical operations supporting late-stage development and commercial manufacturing.
  - Peter Finlayson, Vice President of Marketing, to oversee U.S. launch readiness, bringing orphan disease commercial experience and having previously worked with Ms. Kline at Genentech.
  - Shama Munim, Senior Director of Regulatory Affairs, formerly with Korro Bio and Centessa Pharmaceuticals, to guide regulatory strategy and submissions.
- Palvella is actively strengthening medical affairs and commercial leadership, particularly in Market Access and Sales Leadership, in anticipation of a potential U.S. launch of QTORIN™ rapamycin for microcystic lymphatic malformations, if approved.

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 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, 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 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 ongoing clinical trials, including the TOIVA study, 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; 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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