Palvella Therapeutics Announces Abstract Highlighting the Estimated Diagnosed Prevalence and Annual Incidence of Lymphatic Malformations in the U.S. Accepted for Poster Presentation at the 82nd Annual Meeting of the Society for Investigative Dermatology

April 23, 2025

Quantitative analysis of medical claims indicates an estimated 44,553 to 92,967 diagnosed U.S. patients with lymphatic malformations (LMs) with cutaneous involvement

Estimates of annual incidence of LMs with cutaneous involvement to be highlighted in the poster presentation

Poster to be presented on Thursday, May 8, 2025, 4:30-6:00 pm PT

WAYNE, Pa., April 23, 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 genetic skin diseases for which there are no U.S. Food and Drug Administration (FDA)-approved therapies, today announced an abstract highlighting the estimated diagnosed prevalence and annual incidence of lymphatic malformation (LM) cases in the U.S. was accepted for poster presentation at the Society for Investigative Dermatology (SID) Annual Meeting, taking place May 7-10, 2025, in San Diego, California.

The abstract includes a medical claims-based analysis over a 7-year period conducted with data from Komodo Health. The analysis was aimed at determining the number of LM cases with cutaneous involvement in the U.S., with the following highlights:

- Claims from diagnostic codes that are recommended for use with LM patients by the Vascular Anomalies Special Interest Group of the American Society of Pediatric Hematologist Oncologists were rigorously analyzed by a multi-disciplinary research team, including Dr. Ionela Iacobas, M.D., Medical Director of the Vascular Anomalies Center at Texas Children's Hospital, and David Lapidus, Founder and President of LapidusData and a rare disease epidemiology expert.
- An estimated 44,553 high probability LM patients with cutaneous involvement are projected based on the methodology developed by the authors that includes having greater than or equal to two diagnosis claims related to LM.
- An additional cohort of an estimated 48,414 potential LM patients with cutaneous involvement are projected based on a methodology developed by the authors that includes having at least 1 diagnosis claim related to LM.
- In total, the two cohorts, which include high probability LM patients with cutaneous involvement and potential LM patients with cutaneous involvement, result in up to an estimated 92,967 LM patients with cutaneous involvement.
- Estimates of annual incidence of LMs with cutaneous involvement will also be highlighted in the poster presentation.

"This study is the first to quantify the U.S. LM population based on a rigorous, quantitative analysis of medical claims data. The findings reinforce what we are seeing in clinical practice, which is growing demand for multidisciplinary care of patients with complex vascular anomalies, including microcystic lymphatic malformations," said Dr. Ionela Iacobas, M.D., Medical Director of the Vascular Anomalies Center at Texas Children's Hospital. She continued, "Many of the historical estimates in the literature date from a period prior to advances in care; today we are able to offer patients more in terms of clinical trials and potential treatments, which has increased awareness of the disease and also referrals."

The full text of the abstract can be found here and will be published in the August 2025 supplement issue of the *Journal of Investigative Dermatology (JID)*. The details of the poster to be presented at SID are as follows:

Society for Investigative Dermatology Annual Meeting
Title: Incidence, prevalence, and care for patients with lymphatic malformations (LMs) in the U.S.: A claims-based analysis
Abstract ID: LB1123
Poster Session Category: Clinical Research: Epidemiology and Observational Research
Date & Time: Thursday May 8, 2025, 4:30-6:00 pm PT
Location: Indigo Ballroom/Foyer, Hilton San Diego Bayfront

About 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. The disease is 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. The natural history of microcystic LMs is persistent and progressive without spontaneous resolution, with symptoms generally worsening during life, including increases in the number and size of malformed vessels that lead to complications and lifetime morbidity. There are currently no FDA-approved treatments for the estimated more than 30,000 diagnosed patients with microcystic LMs in the United States.

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 genetic 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 genetic 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 evaluated in the Phase 3 SELVA clinical trial in microcystic lymphatic malformations and the Phase 2 TOIVA clinical trial in cutaneous venous malformations. For more information, please visit www.palvellatx.com or follow Palvella on LinkedIn or X (formerly known as Twitter).

QTORIN™ rapamycin is for investigational use only and has not 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 and financial resources and expected cash runway, and the potential of, and expectations regarding, Palvella's programs, including QTORIN™ rapamycin, 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; 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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