Palvella Therapeutics Awarded Up to $2.6 million Grant from the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development to Support Phase 3 Single-Arm, Baseline-Controlled Trial in Microcystic Lymphatic Malformations

October 3, 2024

FDA Orphan Products Grants are based on scientific and technical merit as determined by rare disease and regulatory experts

Ongoing Phase 3 trial evaluating QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment o microcystic lymphatic malformations, a program with FDA's Breakthrough Therapy Designation and Fast Track Designation

WAYNE, Pa., Oct. 03, 2024 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc. (Palvella), 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, today announced the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development has awarded the company a grant of up to $2.6 million to support the ongoing Phase 3 SELVA trial of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of microcystic lymphatic malformations (microcystic LMs).

"We are pleased to receive this grant from the FDA which will support the advancement of SELVA, our ongoing single arm, baseline-controlled Phase 3 trial of QTORIN rapamycin for the treatment of microcystic LMs," said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. "We believe this grant underscores the high unmet medical need in this serious, rare and chronically debilitating genetic disease, the scientific rationale for targeted inhibition of the causative PI3K/mTOR pathway, and QTORIN rapamycin's potential to be the first approved therapy and standard of care in the U.S. for microcystic LMs."

Out of 51 grant applications received by the FDA Orphan Products Grants Program in fiscal year 2024, Palvella's Phase 3 clinical trial was one of seven new clinical trials that was awarded a grant. The FDA Orphan Products Grants Program awards grants annually to support the development of safe and effective medical products to address unmet medical needs for patients with rare diseases or conditions. Grant applications are individually reviewed and scored for scientific and technical merit by an independent ad hoc panel of rare disease and regulatory experts and may involve consultation with the relevant FDA review division to help determine whether the proposed study will provide acceptable data that could contribute to product approval. Since inception, the FDA Orphan Products Grants Program has funded clinical trials that have facilitated the approval of more than 85 products.

About Microcystic Lymphatic Malformations
Microcystic LMs is a rare, chronically debilitating genetic disease caused by dysregulation of the phosphatidylinositol 3-kinase (PI3K)/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 are 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 QTORIN™ Platform and QTORIN™ rapamycin
Palvella's research team developed QTORIN, a patented and versatile platform designed to generate novel topical therapies that penetrate the deep layers of the skin to locally treat a broad spectrum of serious, rare genetic skin diseases. Well-accepted mechanisms of action of rapamycin and other therapeutic agents represent potential therapies for rare genetic skin diseases. However, the adverse event profile of those agents through systemic exposure poses significant barriers to patient adoption. Palvella's QTORIN product candidates are designed for targeted, localized delivery of therapeutic agents to pathogenic tissue of interest while minimizing systemic absorption and thereby reducing the risk of unwanted adverse events associated with systemic therapy.

QTORIN rapamycin is the lead product candidate from Palvella's QTORIN platform. QTORIN rapamycin is a novel, patented 3.9% rapamycin anhydrous gel, which aims to harness the potential therapeutic benefits of rapamycin, a mammalian target of rapamycin (mTOR) inhibitor, while minimizing systemic exposure of rapamycin and potential adverse reactions associated with systemic therapy. QTORIN rapamycin is currently under development for the treatment of microcystic LMs, cutaneous venous malformations, and other serious, functionally debilitating skin diseases driven by the overactivation of the mTOR pathway.

QTORIN rapamycin has received FDA Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation for microcystic LMs, and Fast Track Designation for venous malformations. QTORIN rapamycin is protected by multiple issued composition patents in the U.S. and Japan and pending patent applications broadly covering anhydrous gel formulations of rapamycin in the U.S., Europe, and Japan.

In the third quarter of 2024, Palvella initiated SELVA, a 24-week, Phase 3, single-arm, baseline-controlled clinical trial of QTORIN rapamycin for the treatment of microcystic LMs. The study's primary and key secondary endpoints are clinician-reported outcomes. The study is expected to enroll 40 subjects at leading vascular anomaly centers across the U.S.

QTORIN rapamycin is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency.

About Palvella Therapeutics
Founded and led by rare drug disease drug development veterans, Palvella Therapeutics 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 in clinical development for microcystic lymphatic malformations (microcystic LMs) and cutaneous venous malformations.

In July 2023, Palvella and Pieris Pharmaceuticals, Inc. (Nasdaq: PIRS) announced they have entered into a definitive merger agreement to combine the companies in an all-stock transaction.

Forward-Looking Statements
This press release contains forward-looking statements concerning the development and commercialization of Palvella's products, the potential benefits and attributes of such products, and the company's expectations regarding its prospects, including the potential merger with Pieris Pharmaceuticals. Forward-looking statements are subject to risks, assumptions and uncertainties that could cause actual future events or results to differ materially from such statements. These statements are made as of the date of this press release. Actual results may vary. Palvella undertakes no obligation to update any forward-looking statements for any reason.

No Offer or Solicitation
This press release is not intended to and does not constitute an offer to sell or the solicitation of an offer to subscribe for or buy or an invitation to purchase or subscribe for any securities or the solicitation of any vote in any jurisdiction pursuant to the proposed transaction or otherwise, nor shall there be any sale, issuance or transfer of securities in any jurisdiction in contravention of applicable law. No offer of securities shall be made except by means of a prospectus meeting the requirements of the Securities Act. Subject to certain exceptions to be approved by the relevant regulators or certain facts to be ascertained, the public offer will not be made directly or indirectly, in or into any jurisdiction where to do so would constitute a violation of the laws of such jurisdiction, or by use of the mails or by any means or instrumentality (including without limitation, telephone and the internet) of interstate or foreign commerce, or any facility of a national securities exchange, of any such jurisdiction.

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Important Additional Information About the Proposed Transactions Will be Filed with the SEC
In connection with the proposed transaction between Pieris and Palvella, Pieris intends to file relevant materials with the SEC, including a registration statement on Form S-4 that will contain a proxy statement and prospectus of Pieris and an information statement of Palvella. PIERIS URGES INVESTORS AND STOCKHOLDERS TO READ THESE MATERIALS CAREFULLY AND IN THEIR ENTIRETY WHEN THEY BECOME AVAILABLE, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THESE MATERIALS, BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION ABOUT PIERIS, PALVELLA, THE PROPOSED TRANSACTION AND RELATED MATTERS. Investors and stockholders will be able to obtain free copies of the proxy statement/prospectus/information statement and other documents filed by Pieris with the SEC (when they become available) through the website maintained by the SEC at www.sec.gov. In addition, investors and stockholders will be able to obtain free copies of the proxy statement/prospectus/information statement and other documents filed by Pieris with the SEC free of charge on Pieris' website at www.pieris.com, or by contacting Investor Relations by email at info@pieris.com. Investors and stockholders are urged to read the proxy statement/prospectus/information statement and the other relevant materials when they become available before making any voting or investment decision with respect to the proposed transaction.

Participants in the Solicitation
Palvella, Pieris and their respective directors and executive officers may be considered participants in the solicitation of proxies in connection with the proposed transaction. Information about Pieris' directors and executive officers is included in Pieris' most recent Annual Report on Form 10-K, as amended, including any information incorporated therein by reference, as filed with the SEC on March 29, 2024, and amended on April 29, 2024. Additional information regarding the persons who may be deemed participants in the solicitation of proxies will be included in the proxy statement/prospectus/information statement relating to the proposed transaction when it is filed with the SEC. These documents can be obtained free of charge from the sources indicated above.

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