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Palvella Therapeutics Announces U.S. FDA Breakthrough Therapy Designation Granted to QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Microcystic Lymphatic Malformations

QTORIN™ rapamycin has potential to be first approved therapy and standard of care in the U.S. for Microcystic Lymphatic Malformations

Microcystic Lymphatic Malformations is a chronically debilitating and lifelong genetic disease affecting an estimated more than 30,000 patients in the U.S.

WAYNE, Pa., Nov. 16, 2023 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a clinical-stage biopharmaceutical company 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 that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to QTORIN rapamycin™ for the treatment of Microcystic Lymphatic Malformations (Microcystic LMs). QTORIN rapamycin is a novel, 3.9% rapamycin anhydrous gel currently under development by Palvella for the treatment of Microcystic LMs and other serious, functionally debilitating skin diseases driven by the overactivation of the mammalian target of rapamycin (mTOR) pathway.

Microcystic LMs is a rare, chronically debilitating genetic disease caused by dysregulation of the PI3K/mTOR pathway. The disease is characterized by localized masses of malformed lymphatic vessels that protrude through the skin barrier and persistently leak lymph fluid (lymphorrhea) and bleed, often leading to recurrent serious infections and cellulitis. The natural history of Microcystic LMs is progressive, with symptoms generally worsening during life, including increases in the number and size of cysts that lead to complications and lifetime morbidity. There are currently no FDA-approved treatments for the estimated more than 30,000 patients with Microcystic LMs in the U.S.

“We are very encouraged that the FDA has granted Breakthrough Therapy Designation to QTORIN rapamycin, recognizing the strength of the clinical results from our Phase 2 study and the potential for QTORIN rapamycin as a novel, targeted therapy for the treatment of Microcystic LMs,” said Wes Kaupinen, Founder and Chief Executive Officer of Palvella Therapeutics. “We believe QTORIN rapamycin can fulfill an urgent unmet need for patients suffering from Microcystic LMs who from a very young age endure significant morbidity as a result of this lifelong disease.”

The Breakthrough Therapy Designation for QTORIN rapamycin is based on positive Phase 2 results from a multi-center, open-label trial of once-daily QTORIN rapamycin in Microcystic LMs. In the Phase 2 study, 100% of participants (n=12) were either “Much Improved” or “Very Much Improved” as rated by the Clinician Global Impression of Change after 12 weeks of QTORIN rapamycin treatment. Based on the positive Phase 2 results and a productive end-of-Phase 2 meeting with FDA, Palvella previously announced plans to conduct a pivotal Phase 3 study in approximately 50 adult and pediatric patients.

Breakthrough Therapy Designation is intended to expedite the development and review of therapies for serious or life-threatening conditions and whose preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. Under the designation, the FDA provides intensive guidance, organizational commitment involving senior managers, and eligibility for rolling and priority reviews.

In addition to Breakthrough Therapy Designation, the FDA previously granted Fast Track Designation and Orphan Drug Designation to QTORIN rapamycin for the treatment of Microcystic LMs. The European Medicines Agency has also granted Orphan Drug Designation to QTORIN rapamycin for the treatment of Microcystic LMs.

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.

About Palvella Therapeutics

Founded and led by rare disease 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. We are developing a broad pipeline of product candidates based on our patented QTORIN™ platform, with an initial focus on serious, rare genetic skin diseases, many of which are lifelong in nature. Our lead product candidate, QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) is currently in late-stage clinical development for Microcystic Lymphatic Malformations (Microcystic LMs) and Basal Cell Carcinomas (BCCs) in Gorlin Syndrome (GS). QTORIN rapamycin has received FDA Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation for Microcystic LMs, and Fast Track Designation for the prevention of BCCs in GS.

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

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. 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.

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