Source: *Palvella Therapeutics*

December 01, 2023 08:20 ET

Palvella Therapeutics and Ligand Pharmaceuticals Expand Strategic Partnership to Accelerate Phase 3 Development of QTORIN™ rapamycin for Microcystic Lymphatic Malformations and Additional High Unmet Need Clinical Indications

Palvella Received $5 Million Upfront Payment

QTORIN™ rapamycin has Potential to be First FDA Approved Therapy and Standard of Care in the U.S. for an Estimated more than 30,000 Diagnosed Patients with Microcystic Lymphatic Malformations

U.S. FDA Breakthrough Therapy Designation Granted to QTORIN™ rapamycin for the Treatment of Microcystic Lymphatic Malformations in November 2023

WAYNE, Pa., Dec. 01, 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, and Ligand Pharmaceuticals Incorporated (Nasdaq: LGND), a biopharmaceutical company enabling scientific advancement through supporting the clinical development of high-value medicines, today announced the expansion of their strategic partnership to accelerate Phase 3 development of QTORIN™ rapamycin for the treatment of Microcystic Lymphatic Malformations (Microcystic LMs).

“We are pleased to expand our strategic partnership with Ligand Pharmaceuticals, a recognized leader with an established track record in partnering with rare disease companies to accelerate development of high-value therapies,” said Wes Kaupinen, Founder and Chief Executive Officer of Palvella. “With an estimated more than 30,000 diagnosed patients in the U.S. suffering from Microcystic Lymphatic Malformations, we see a very significant and attractive commercial opportunity for QTORIN rapamycin as the first potential FDA-approved therapy for this lifelong disease which causes significant patient morbidity from a very young age.”

According to the terms of the amended agreement, Palvella received an upfront payment of $5 million from Ligand. In return, Ligand’s existing tiered royalty on worldwide commercial sales of QTORIN rapamycin increased to 8.0–9.8%. Additionally, Ligand was granted an option to acquire a single-digit royalty on each novel topical product candidate generated from Palvella’s QTORIN™ platform which can be exercised at a future date.

“Based on the positive Phase 2 results announced earlier this year and FDA’s recent granting of Breakthrough Therapy Designation, we are encouraged by the significant commercial potential of QTORIN rapamycin to be the first FDA approved therapy for Microcystic Lymphatic Malformations in the U.S.,” said Matt Korenberg, President and Chief Operating Officer of Ligand.

QTORIN rapamycin, the lead product candidate from Palvella’s QTORIN platform, is a novel, 3.9% rapamycin anhydrous gel currently under development 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. There are currently no FDA-approved treatments for the estimated more than 30,000 diagnosed patients with Microcystic LMs in the U.S.

Palvella previously announced 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 once-daily QTORIN rapamycin treatment. The U.S. FDA granted Breakthrough Therapy Designation to QTORIN rapamycin for the treatment of Microcystic Lymphatic Malformations in November 2023. Based on the positive Phase 2 results and a productive end-of-Phase 2 meeting with FDA, Palvella plans to conduct a pivotal Phase 3 study in approximately 50 adult and pediatric patients. 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.

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.

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