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Palvella Therapeutics Reports Top-Line Results from Pivotal Phase 2/3 VALO Trial of QTORIN™ 3.9% Rapamycin Anhydrous Gel in Patients with Pachyonychia Congenita

~ VALO Study Misses Primary Endpoint in Phase 3 Randomized Withdrawal Portion ~

~ VALO Study Achieves Primary Endpoint in Phase 2 Open-Label Portion ~

~ Palvella Plans to Share Results with the U.S. Food and Drug Administration in the First Quarter of 2021 ~

WAYNE, Pa., Dec. 23, 2020 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a clinical-stage biopharmaceutical company committed to serving individuals suffering from serious, rare genetic diseases without approved therapies, today reported top-line results from the pivotal Phase 2/3 VALO study of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN rapamycin) for the treatment of pachyonychia congenita (PC).

The Phase 2/3 VALO study was a multi-center, two-stage, randomized withdrawal trial designed to evaluate the efficacy and safety of QTORIN rapamycin in adults with moderate to severe PC. Participants were followed for a four-week run-in (RI) period followed by a Phase 2 open-label (OL) treatment period in which they received QTORIN rapamycin once-daily for twelve weeks. At the end of Phase 2, participants who met a pre-specified clinical response threshold seamlessly progressed into a twelve-week, randomized, double-blind, vehicle-controlled Phase 3 study, and were randomized to receive either once-daily QTORIN rapamycin, twice-daily QTORIN rapamycin or vehicle gel (placebo). The primary endpoint was the Patient Global Assessment of Activities Difficulty (PGA-AD), a daily patient-reported outcome measure which assessed the difficulty of patients carrying out activities on their feet, comparing the last two weeks of each period to the last two weeks of the preceding period.

In the Phase 3 portion of the study, in the intent to treat (ITT) population (n=41), the pooled QTORIN rapamycin arms did not show a treatment effect on the PGA-AD primary endpoint when compared to vehicle gel (p-value=0.60). Similarly, the key secondary endpoint of Pain at Its Worst did not show a treatment effect when compared to vehicle gel.

In the Phase 2 portion of the study, in the ITT population (n=73), QTORIN rapamycin achieved a statistically significant improvement on the PGA-AD primary endpoint, which compared the last two weeks of the Phase 2 open-label treatment period to the last two weeks of the RI period (p-value <0.0001). Additionally, QTORIN rapamycin achieved statistically significant improvements on two of the three pre-specified key secondary efficacy endpoints in the Phase 2 portion of the study, including Pain at Its Worst (p-value <0.0001) and Clinician Global Impression of Change (p-value <0.0001); no improvement was seen in Number of Steps taken daily.

QTORIN rapamycin was well tolerated in both the Phase 2 and Phase 3 portions of the VALO study. No drug-related serious adverse events (SAE) were reported, and all other adverse events were deemed mild or moderate in nature. The most common treatment-emergent adverse events were nasopharyngitis and upper respiratory tract infections.

“While QTORIN rapamycin did not show a treatment effect when compared to vehicle gel on the primary endpoint in the Phase 3 randomized withdrawal portion, we are encouraged by the statistically and clinically significant results achieved in Phase 2,” said Wes Kaupinen, President and Chief Executive Officer. “We remain optimistic about the potential of QTORIN rapamycin as a targeted therapy for PC, a rare, chronically debilitating and lifelong genetic disease for which there are no FDA-approved therapies. We would like to sincerely thank the patients, families, clinical investigators and our partners at Pachyonychia Congenita Project involved in this important study.”

Palvella plans to incorporate these results into discussions with regulatory agencies to determine next steps for QTORIN rapamycin in PC. In parallel, the Company also plans to further analyze the Phase 2/3 VALO study design and results with key stakeholders in the PC community, including scientists, clinicians, and the leadership at Pachyonychia Congenita Project. QTORIN rapamycin has received Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration for the treatment of PC and Orphan Drug Designation from the European Medicines Agency.

About Palvella Therapeutics

Founded and led by rare disease veterans, Palvella Therapeutics is a clinical-stage biopharmaceutical company committed to serving individuals suffering from serious, rare genetic diseases without approved therapies through the development and commercialization of therapies that target the root causes of these diseases. Palvella’s development model involves partnering with patient advocacy organizations and their patient registries to design fit-for-purpose, accelerated clinical development programs aimed at expediting the introduction of targeted therapies to patients who currently lack any approved treatment options. Palvella’s lead program, QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN rapamycin), has completed a Phase 2/3 pivotal study for pachyonychia congenita (PC), a rare, chronically debilitating and lifelong genetic disease estimated to affect more than 9,000 individuals in the U.S. More information on the company and its pipeline may be found on the company’s website at www.palvellatx.com.

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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