Palvella Therapeutics Commences Phase 3 Portion of Phase 2/3 Pivotal Study of PTX-022 in Pachyonychia Congenita

Fast Track-designated program aims to be first targeted therapy addressing root cause of chronically debilitating rare genetic disease

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Wayne, PA, Nov. 13, 2019 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a rare-disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for serious, rare genodermatoses with no approved treatments, announced that the Phase 3 pivotal portion of the seamless Phase 2/3 VALO Study of PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel) for the treatment of patients with pachyonychia congenita (PC) has commenced.

PC is a rare, chronically debilitating and lifelong monogenic disease in which mutations of genes responsible for keratin production lead to dysregulated keratinocyte proliferation, increased skin fragility and impaired skin barrier function on the plantar aspects of the feet. As a result, affected individuals experience pain and difficulty with ambulation, which frequently necessitates the use of either ambulatory aids or alternative forms of mobility such as crawling on hands and knees. There are currently no approved therapies for PC.

The Phase 2/3 VALO study is the largest clinical study ever conducted of a therapy for PC and is currently enrolling patients at nine sites across the US. In the Phase 3 double-blind, placebo-controlled, randomized withdrawal portion of the study, enrolled patients who met the pre-specified clinical response criteria during the Phase 2 portion will be assigned to one of three arms: placebo, twice-daily (BID) PTX-022, or once-daily (QD) PTX-022. Following completion of Phase 3, Palvella intends to initiate an open-label extension program where patients will have the option to continue to receive study drug.

“Commencing the Phase 3 portion of this study represents a significant milestone for all who have been involved in accelerating the development of PTX-022 for pachyonychia congenita,” noted Palvella president and CEO, Wes Kaupinen. “We’re grateful for the efforts of the patients, their families, the VALO clinical investigators and study coordinators, and our partners at PC Project. We look forward to reporting the study results in the second half of 2020.”

PTX-022 is a novel formulation of rapamycin which was discovered by leading scientists in the field of PC research to have a potential direct mechanism of action on the mutant keratin genes which are the root cause of PC. PTX-022 leverages Palvella's QTORIN™ formulation and delivery technology. QTORIN™ is a proprietary and patent-pending technology that employs a specific composition of excipients that enable distribution of rapamycin into the basal keratinocytes which harbor the mutant keratin genes that are the primary defect in pachyonychia congenita. In addition to PC, QTORIN™ and its related technologies are being investigated in other serious, rare genodermatoses.

PTX-022 is supported by multiple issued method of use patents in the U.S. broadly covering the use of mTOR inhibitors in pachyonychia congenita. PTX-022 has received FDA Fast Track Designation, FDA Orphan Drug Designation, and EMA Orphan Drug Designation.

About Palvella Therapeutics

Palvella Therapeutics is a rare disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for serious, rare genodermatoses with no approved treatments. Palvella's lead program, PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel), is in Phase 2/3 development for pachyonychia congenita, a rare, chronically debilitating and lifelong monogenic disease. In PC, mutations of genes responsible for keratin production lead to dysregulated keratinocyte proliferation, increased skin fragility and impaired skin barrier function on the plantar aspects of the feet. As a result, affected individuals experience pain and difficulty with ambulation, which frequently necessitates the use of either ambulatory aids or alternative forms of mobility such as crawling on hands and knees. More information 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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