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Palvella Therapeutics Completes Enrollment in Phase 2/3 Pivotal Study of PTX-022 for Treatment of Pachyonychia Congenita

Top-Line Data Expected in Q4 2020 for Fast Track-Designated Program

Wayne, PA , March 06, 2020 (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, today announced the completion of enrollment for its seamless Phase 2/3 VALO Study of PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel) for the treatment of adults with pachyonychia congenita (PC). PC is a rare, chronically debilitating and lifelong genetic disease in which affected individuals experience extreme pain and difficulty with ambulation, frequently necessitating the use of either ambulatory aids or alternative forms of mobility such as crawling on hands and knees. There are currently no FDA-approved therapies for the over 9,000 individuals estimated to be living with PC in the U.S.[1]

“Our team at Palvella is passionately committed to enhancing the lives of individuals with pachyonychia congenita through the accelerated development of novel therapies which address the root genetic cause of this chronically debilitating disease,” stated Wes Kaupinen, President and Chief Executive Officer of Palvella. “The unwavering commitment of the clinical investigators, study coordinators and our partners at PC Project to achieve full enrollment of the VALO study has brought us all one step closer towards achieving this objective. We look forward to reporting top-line study results in the fourth quarter of 2020.”

“Our International Pachyonychia Congenita Research Registry, comprised of an enthusiastic PC patient population interested in helping themselves and younger and future generations of PC patients, along with the constant collaboration and feedback between Palvella and PC Project, all contributed to fast and successful enrollment of this historic study,” said Janice Schwartz, Executive Director of PC Project. “PC Project is profoundly grateful to the professionals at Palvella who have demonstrated their concern and compassion for PC patients in all their efforts.”

VALO is a multi-center, four-part, Phase 2/3 study evaluating the safety and effectiveness of PTX-022 in adults with PC. In November 2019, Palvella began treating participants in the Phase 3 double-blind, placebo-controlled, randomized withdrawal portion of the study (announced on November 13, 2019) where those who met the pre-specified clinical response criteria during the Phase 2 portion would be assigned to one of three arms: placebo, twice-daily (BID) PTX-022, or once-daily (QD) PTX-022. Palvella also intends to initiate an open-label extension program where patients will have the option to continue to receive study drug.

The potential for rapamycin as a targeted PC therapy was originally discovered in 2006 by leading PC scientists who elucidated a potential direct mechanism of action of rapamycin on the mutant keratin genes which are the root cause in PC. PTX-022 is a novel formulation of rapamycin which leverages Palvella's QTORIN™ 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, including Gorlin Syndrome.

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.

[1] *Gallagher et al. J Dermatol Dis 2019, 6:1*

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 genetic 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 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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