Palvella Therapeutics Announces FDA Fast Track Designation for PTX-022 for Treatment of Pachyonychia Congenita

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Wayne, PA, Nov. 12, 2018 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a rare disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for debilitating genetic diseases with no approved treatments, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for its lead product candidate **PTX-022**(novel, high-strength rapamycin topical formulation, optimized for dermal targeting) for the treatment of pachyonychia congenita (PC). PC is 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 difficulty with ambulation, which frequently necessitates the use of either ambulatory aids or alternative forms of mobility such as crawling on hands and knees.

“The recognition by the FDA of the potential of PTX-022 to treat pachyonychia congenita builds upon the compelling scientific rationale and the encouraging early human clinical results seen with rapamycin in the treatment of PC,” stated Joyce M. Teng, M.D., Ph.D., clinical professor of dermatology and pediatrics at Stanford University School of Medicine. “I look forward to working closely with the Palvella team as we move expeditiously to advance PTX-022 into a Phase 2/3 clinical study.”

Fast Track is a program designed to facilitate the development, and expedite the review, of drugs to treat serious conditions and fill an unmet need. The purpose is to ensure that

important new drugs reach patients quickly. If relevant criteria are met, programs with Fast Track designation can become eligible for priority review and rolling review, both of which can reduce the timelines associated with regulatory review and action. With Fast Track designation, the frequency of communication between the FDA and a drug company assures that questions are resolved quickly, often leading to earlier drug approval and access by patients suffering from serious diseases.

“We’ve heard from PC patients, who struggle to walk while experiencing extreme pain, that one step can truly make all of the difference in their lives,” stated **Wes Kaupinen**, president and chief executive officer of Palvella. “The FDA’s decision to grant Fast Track designation for PTX-022 is a positive step for our partners at PC Project and for Palvella in our shared ambition to accelerate the development of this important therapy.”

PTX-022 leverages Palvella’s proprietary and patent-pending **QTORIN™** formulation and delivery technology. QTORIN™ employs a highly specific composition of excipients that enables distribution of mTOR inhibitors into the basal keratinocytes, which harbor the mutant keratin genes that are the primary defect in pachyonychia congenita. QTORIN™ further enables drug penetration into the reticular dermis where neovascularization and inflammatory components of the pachyonychia congenita pathology manifest.

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 through 2032. PTX-022 has also received U.S. 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 debilitating, rare genetic diseases with no approved treatments. Palvella’s lead program, PTX-022, is entering 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 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.

Source: Palvella Therapeutics

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