# Palvella Therapeutics to Present Update on PTX-022 at Pachyonychia Congenita Project Patient Support Meeting in London, UK

Wayne, PA, Oct. 19, 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, announced that they will be presenting an update on PTX-022 (novel, high-strength rapamycin topical formulation, optimized for dermal targeting) at Pachyonychia Congenita Project's 2018 London England Patient Support Meeting. In partnership with Pachyonychia Congenita Project, Palvella will be initiating a Phase 2/3 clinical study of PTX-022 for the treatment of pachyonychia congenita, a rare, chronically debilitating, and lifelong genetic disease in which mutated genes responsible for keratin production lead to extreme cell fragility. This causes impaired ambulation which frequently necessitates the use of either ambulatory aids or alternative forms of mobility such as hands and knee crawling.

"PC Project is privileged to have approximately 140 people attend this year's PC Patient Support Meeting, including 67 PC patients, plus caregivers, physicians, scientists and its industry partner, Palvella," commented Janice Schwartz, chair of the PC Project Board of Trustees. "These meetings are not only educational for patients and their caregivers, but emotional and even life-changing for patients who have often never met another person with PC. Furthermore, PC Patient Support Meetings serve as an opportunity for clinicians, researchers and drug developers to observe and learn directly from PC patients in their efforts to find effective treatments for PC."

Mrs. Schwartz continued, "The hope with every patient meeting is that instead of feeling embarrassed and alone, PC patients will leave encouraged, edified and more able to cope with this debilitating disease. PC Project is appreciative of its partnership with Palvella and its sponsorship of this valuable patient meeting. To work with an industry partner like Palvella is nothing short of a dream come true for our rare disease group. Palvella consists of an energetic team of individuals deeply committed to developing a treatment that could make a real difference for PC patients."

PTX-022 leverages Palvella's proprietary and patent-pending QTORIN™ formulation and delivery technology. QTORIN™ employs a highly specific composition of excipients to enable 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.

“Strengthening our ongoing collaboration with PC Project and supporting the pachyonychia congenita patient community is core to Palvella’s mission of serving patients with rare, serious diseases,” said Wes Kaupinen, president and CEO of Palvella Therapeutics. “We share PC Project’s passion for accelerating the development of potentially effective therapies for this serious and chronically debilitating genetic disease that dramatically limits patients’ ability to ambulate and perform everyday functional activities.”

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. In addition to PC, Palvella is actively evaluating the potential to apply QTORIN™ and its related technologies in other rare, serious genodermatoses.

**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 genetic disease in which mutated genes responsible for keratin production lead to extreme cell fragility. This causes impaired ambulation which frequently necessitates the use of either ambulatory aids or alternative forms of mobility such as hands and knee crawling. 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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