Palvella Therapeutics Announces $10 Million in Funding from Ligand Pharmaceuticals to Accelerate PTX-022 through Phase 2/3 Clinical Study in Pachyonychia Congenita

- Fast Track-Designated Program Commencing Phase 2/3 Study in Q1 2019 –

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Wayne, PA – December 18, 2018 – 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 company has entered into a development funding and royalties agreement with Ligand Pharmaceuticals, Inc. (NASDAQ: LGND) to advance Palvella's lead product candidate PTX-022 (QTORIN™ rapamycin formulation) for the treatment of pachyonychia congenita (PC). Under the terms of the agreement, Palvella will receive $10 million from Ligand in exchange for milestones and a tiered single-digit royalty on future sales of PTX-022. As part of the agreement, Palvella will continue to have responsibility for all clinical development, regulatory, manufacturing, marketing and other commercialization activities on a worldwide basis.

"Ligand has a long and successful track record in partnering with leading biopharmaceutical companies to develop some of the world's most important medicines," stated Wes Kaupinen, president and chief executive officer of Palvella. "This agreement with Ligand represents another milestone for Palvella in our ambition to improve the lives of PC patients who currently have no FDA-approved treatments. Furthermore, this funding brings Palvella one step closer to recognizing the significant commercial potential of PTX-022."

In partnership with Pachyonychia Congenita Project, Palvella is entering a Phase 2/3 clinical study of PTX-022 for the treatment of PC, 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 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 potential for rapamycin in PC was discovered by leading scientists in the field of PC research who elucidated a direct mechanism of action of mTOR inhibitors on the mutant keratin genes, which are the root cause of pachyonychia congenita. PTX-022 is a novel formulation that has been developed using a scientifically rigorous process in partnership with MedPharm Ltd. 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.

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 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 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.

About Ligand Pharmaceuticals

Ligand is a biopharmaceutical company focused on developing or acquiring technologies that help pharmaceutical companies discover and develop medicines. Ligand's business model creates value for stockholders by providing a diversified portfolio of biotech and pharmaceutical product revenue streams that are supported by an efficient and low corporate cost structure. Ligand's goal is to offer investors an opportunity to participate in the promise of the biotech industry in a profitable, diversified and lower-risk business than a typical biotech company. Ligand has established multiple alliances, licenses and other business relationships with the world's leading pharmaceutical companies including Novartis, Amgen, Merck, Pfizer, Celgene, Gilead, Janssen, Baxter International and Eli Lilly.

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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