Palvella Therapeutics Appoints Rare Disease Senior Executive Elaine J. Heron, PhD to Board of Directors

- Dr. Heron Adds Deep Expertise in Orphan Drug Development and Commercialization -

Wayne, PA – September 5, 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 rare disease expert, Elaine J. Heron, PhD, has joined the company's Board of Directors. Dr. Heron brings over 25 years of rare disease and life sciences drug development experience to Palvella's Board. She has served as a member of the Board of Directors of BioMarin (NASDAQ: BMRN) since 2002 where she continues to serve on the Board and as chair of the Corporate Governance and Nominating Committee. Most recently, Dr. Heron was chief executive officer of Amplyx Pharmaceuticals where she remains on the Board and was previously the chief executive officer of Labcyte Inc.

"Pachyonychia congenita (PC) is a life-altering genetic disease which dramatically limits patients' ability to ambulate and perform everyday functional activities," stated Dr. Heron. "Palvella's lead program PTX-022 (novel, high-strength rapamycin topical formulation, optimized for dermal targeting) has significant potential to address this serious unmet need that currently has no approved treatments. I look forward to applying my background in rare diseases in supporting Palvella's efforts to advance PTX-022 through late stage clinical development and commercialization."

Dr. Heron joins a Board of orphan drug and life sciences industry veterans, including:

- George M. Jenkins (Chairman): Previously Chief Operating Officer and General Partner of Apax Partners;
- Wesley H. Kaupinen (President and CEO): Previously a Senior Executive at Insmed and Venture Capitalist at Apax Partners and Quaker Partners;
- Timothy J. Henkel, MD, PhD: Chief Medical Officer of VenatoRx and Previously Chief Medical Officer of Ceptaris (Acquired by Actelion);
- David S. Tierney, MD: Previously President and CEO of Valera Pharmaceuticals (Acquired by Indevus Pharmaceuticals) and Current Board Director of Catalyst Pharmaceuticals;
- Todd C. Davis: Founding Managing Director at HealthCare Royalty Partners and Current Board Director of Ligand Pharmaceuticals; and
- Michael S. Christopher: Senior Executive of ARM Energy and Private Investor

In partnership with Pachyonychia Congenita Project, Palvella is currently entering a Phase 2/3 clinical study of PTX-022 for the treatment of PC, 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. The potential for rapamycin in PC was originally discovered in 2006 by leading scientists in the field of pachyonychia congenita who elucidated a potential direct, disease-modifying mechanism of action of rapamycin on the mutant keratin genes driving the

pachyonychia congenita disease process. Rapamycin has since achieved proof of concept in pilot human clinical studies that have demonstrated promising efficacy results in PC patients, including rapid improvement of ability to ambulate and improved quality of life. PTX-022 is a novel topical 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. 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. 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

Founded and led by life sciences and orphan drug veterans, 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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