***Source:*** *Palvella Therapeutics*

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# Palvella Therapeutics Completes $45 Million Series C Financing

*Investor Syndicate Comprised of Leading Biotech Investors*

*Proceeds to Accelerate Pipeline of Rare Disease Therapies, Including Late-Stage Programs in Pachyonychia Congenita and Gorlin Syndrome*

*Top-Line Data Expected in Q4 2020 from Phase 2/3 Pivotal Study for Fast Track-Designated Lead Program in Pachyonychia Congenita*

WAYNE, Pa., May 28, 2020 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a rare disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for serious genetic diseases with no approved treatments, today announced the closing of an oversubscribed $45 million Series C financing. Leading biotech investors participating in this round include CAM Capital, Samsara BioCapital, BVF Partners L.P., Adams Street Partners, Opaleye Management, Ligand Pharmaceuticals (Nasdaq: LGND), Agent Capital, BioAdvance and Nolan Capital (the investment fund of former AveXis CEO Sean Nolan).

Concurrent with the close of the Series C financing, Palvella announced that two of the lead investors, Scott Morenstein, Managing Director of CAM Capital, and Cory Freedland, Principal of Samsara BioCapital, have been nominated to join the Palvella Board of Directors.

“Palvella was founded on fundamental beliefs that every individual with a rare disease deserves a treatment and that significant value creation occurs upon the introduction of the first approved therapy for a serious rare genetic disease,” stated Wes Kaupinen, President and Chief Executive Officer of Palvella. “The capital invested from this highly regarded syndicate of public market and venture investors strengthens our team’s unwavering commitment to develop and commercialize targeted therapies to individuals suffering from serious, life-altering rare genetic diseases such as pachyonychia congenita (PC) and Gorlin syndrome.”

Proceeds from the Series C financing will support the advancement of PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel) for the treatment of adults with PC, a rare, chronically debilitating and lifelong genetic disease. Individuals with PC 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. (*Gallagher et al, 2019*).

In partnership with Pachyonychia Congenita Project, Palvella completed enrollment in March 2020 of the Phase 2/3 pivotal VALO Study for PTX-022. PTX-022 is a novel formulation of rapamycin, which was shown by geneticist Dr. Roger Kaspar to have a direct mechanism of action on mutant keratin genes which are the root cause in PC. PTX-022 leverages Palvella's QTORIN™ technology to enable localized distribution of rapamycin into the suprabasal keratinocytes which express the mutant keratin genes that are the primary defect in PC. The company expects top-line results from VALO to be available in the fourth quarter of 2020.

In partnership with the Gorlin Syndrome Alliance, Palvella's second candidate, PTX-367 (QTORIN™ rapamycin) will enter into a late-stage clinical study for individuals with Gorlin syndrome, a genetic disease caused by a mutation in PTCH1, a tumor suppressor gene. Individuals afflicted with Gorlin syndrome can develop hundreds of basal cell carcinomas (BCCs), oftentimes beginning in adolescence. BCCs are a malignant skin cancer requiring repeated and potentially disfiguring surgical removal for individuals with Gorlin syndrome. Palvella initiated internal research efforts on the potential for QTORIN™ rapamycin in Gorlin syndrome in 2017, and in 2018 the role of the mTOR pathway in BCC tumorigenesis in Gorlin syndrome was further elucidated (*Kim et al, 2018*). Gorlin syndrome affects an estimated 10,000 people in the U.S. and there are no FDA-approved therapies.

PTX-022 is protected by multiple issued method-of-use patents in the U.S. broadly covering the use of rapamycin and derivatives thereof in treating PC that expire as late as 2032 and an allowed patent application in the U.S. covering the use of anhydrous gel formulations of rapamycin for treating PC and Gorlin syndrome that will expire in 2038. PTX-022 has received FDA Fast Track Designation, FDA Orphan Drug Designation and EMA Orphan Drug Designation.

**About Palvella Therapeutics**
Founded and led by rare disease veterans, Palvella Therapeutics is a rare disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for serious genetic diseases with no approved treatments. Palvella's development model involves partnering with patient advocacy organizations and their patient registries to design fit-for-purpose, accelerated clinical development programs aimed at expediting the introduction of targeted therapies to patients who currently lack any approved treatment options. Palvella's lead program, PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel), is in a Phase 2/3 pivotal study for pachyonychia congenita (PC), a rare, chronically debilitating and lifelong genetic disease estimated to affect more than 9,000 individuals in the U.S.

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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