Palvella Therapeutics Reports Topline Results from Pivotal Phase 3 VAPAUS Study of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Pachyonychia Congenita

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WAYNE, Pa., July 20, 2023 (GLOBE NEWSWIRE) -- Palvella Therapeutics, Inc., a clinical-stage biopharmaceutical company developing and commercializing novel therapies to treat patients suffering from serious, rare genetic skin diseases for which there are no FDA-approved therapies, today announced topline results from VAPAUS, a 24-week, randomized, double-blind, placebo-controlled pivotal Phase 3 study of QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) for the treatment of Pachyonychia Congenita (PC).

In the Intent-to-Treat population (n=87), QTORIN rapamycin did not show a treatment effect on the Patient Global Assessment of Activities Difficulty primary endpoint, a daily patient-reported outcome measure which assessed the difficulty of patients carrying out activities on their feet, when compared to placebo. QTORIN rapamycin was well-tolerated in the study and no participants withdrew due to drug-related adverse events. No drug-related serious adverse events were reported, and all other adverse events were deemed mild or moderate in nature with the most common treatment emergent adverse events reported in the category of infections and infestations (nasopharyngitis, COVID-19, and upper respiratory tract infections).

“We would like to sincerely thank PC patients, the leadership of PC Project with whom we have an incredible partnership, the VAPAUS study investigators in the U.S. and U.K., the Palvella team, and our investors who together supported this study,” said Wes Kaupinen, Founder and Chief Executive Officer. “While we are disappointed that the VAPAUS study did not meet its primary endpoint, we hope that Palvella’s efforts to raise awareness of PC and encourage the advancement of novel therapies for this devastating disease will be enduring contributions that one day result in a better quality of life for this deserving patient community.”

The company plans to further analyze and share the Phase 3 VAPAUS study results with key stakeholders, including PC Project, physicians, and scientists. Based on these top-line results, Palvella does not plan to invest in additional clinical studies or commercial preparation activities for QTORIN rapamycin for the treatment of PC.

Mr. Kaupinen continued, “Our vision at Palvella remains focused on becoming the leading rare disease company developing and commercializing novel therapies to treat patients suffering from serious, rare genetic skin diseases in indications for which there are no FDA-approved therapies.”

About Palvella Therapeutics

Founded and led by rare disease veterans, Palvella Therapeutics is a clinical-stage biopharmaceutical company focused on developing and commercializing novel therapies to treat patients suffering from serious, rare genetic skin diseases for which there are no FDA-approved therapies. We are developing a broad pipeline of product candidates based on our patented QTORIN™ platform, with an initial focus on serious, rare genetic skin diseases, many of which are lifelong in nature. Our lead product candidate, QTORIN™ 3.9% rapamycin anhydrous gel (QTORIN™ rapamycin) is currently in late-stage clinical development for Microcystic Lymphatic Malformations (Microcystic LMs) and Basal Cell Carcinomas (BCCs) in Gorlin Syndrome (GS). QTORIN rapamycin has received FDA Fast Track Designation for Microcystic LMs and prevention of BCCs in GS.

QTORIN rapamycin is for investigational use only and has not been approved or cleared by the FDA or by any other regulatory agency. The safety or efficacy has not been established for any use.

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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