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Ligand Earns $3 Million Milestone Payment from Palvella Therapeutics

SAN DIEGO--(BUSINESS WIRE)-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** announces it has earned a $3 million milestone payment from Palvella Therapeutics as a result of Palvella raising $45 million in an oversubscribed Series C financing to leading biotechnology crossover and venture investors. The milestone payment will consist of $2 million in cash and $1 million in Palvella Series C Preferred Stock.

Palvella is a privately-held rare disease biopharmaceutical company focused on developing and commercializing pathogenetically targeted therapies for serious, rare genetic diseases with no approved treatments. In December 2018 Ligand entered into a development funding and royalties agreement with Palvella to advance their lead product candidate PTX-022 (QTORIN™ 3.9% rapamycin anhydrous gel) for the treatment of pachyonychia congenita (PC), a rare, chronically debilitating and lifelong genetic disease estimated to affect more than 9,000 individuals in the U.S. In addition, Palvella's second product candidate, PTX-367, will enter into a late-stage clinical study for individuals with Gorlin syndrome. Ligand paid $10 million to Palvella and in return will receive a tiered royalty on net sales of certain Palvella products (including PTX-022 and PTX-367) in the mid-to-upper single digits, as well as regulatory and financing milestones.

"We are proud of our history of partnering with innovative biopharmaceutical companies to develop important medicines, including our relationship with Palvella. The PTX-022 program is currently being assessed in a Phase 2/3 pivotal study and is led by a team of distinguished and capable scientists who have identified a novel way to treat PC," said John Higgins, Chief Executive Officer of Ligand Pharmaceuticals. "Palvella intends to deploy the Series C proceeds to support the advancement of PTX-022 for the treatment of adults with PC and ultimately prepare for commercialization. We are also pleased to see the progress Palvella has made advancing PTX-367 for people afflicted with Gorlin syndrome, a disease where patients can develop hundreds of basal cell carcinomas, oftentimes beginning in adolescence."

In March 2020 Palvella announced completion of enrollment in its Phase 2/3 pivotal VALO Study of PTX-022 in adults with PC. Palvella expects topline results from this trial to be available in the fourth quarter of 2020.

VALO is a multicenter, four-part, Phase 2/3 study evaluating the safety and effectiveness of PTX-022 in adults with PC. In November 2019 Palvella began treating participants in the Phase 3 double-blind, placebo-controlled, randomized withdrawal portion of the study where those who met the pre-specified clinical response criteria during the Phase 2 portion were assigned to one of three arms: placebo, twice-daily PTX-022 or once-daily PTX-022. Following completion of Phase 3, Palvella intends to initiate an open-label extension program where patients will have the option to continue to receive study drug.

About PTX-022

PTX-022 is a novel, topical formulation of the mTOR inhibitor rapamycin that leverages Palvella's proprietary and patent-pending QTORIN™ formulation and delivery technology. The potential for rapamycin to treat patients with PC was discovered by leading scientists in the field who elucidated a direct mechanism of action of rapamycin on the mutant keratin genes, which are believed to be the root cause of PC. QTORIN™ employs a highly-specific composition of excipients that enables distribution of mTOR inhibitors into the basal keratinocytes, which harbor the mutant keratin genes. PTX-022 is supported by multiple issued method-of-use patents in the U.S. that broadly cover the use of mTOR inhibitors in PC through 2032. PTX-022 has received FDA Fast Track Designation and Orphan Drug Designation for the treatment of PC.

About Pachyonychia Congenita

Pachyonychia congenita is a rare inherited, severe and chronically debilitating skin disorder caused by mutations in certain keratin genes. The disorder is manifested by the overproduction of keratin, which are proteins that give shape and strength to skin cells. In patients with PC, the keratin structure does not form properly, leading to painful conditions including blisters and calluses on the feet that impact mobility, as well as thickened nails, cysts and sores. It is a lifelong disorder that significantly impacts quality-of-life and often necessitates the use of aids to assist movement or alternative forms of mobility such as crawling on ones hands and knees. PC affects more than 9,000 people in the U.S. and no FDA-approved therapies exist to treat the disorder.

About Ligand Pharmaceuticals

Ligand is a biopharmaceutical company focused on developing or acquiring technologies that help pharmaceutical companies discover and develop medicines. Our 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. Our 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. Our business model is based on doing what we do best: drug discovery, early-stage drug development, product reformulation and partnering. We partner with other pharmaceutical companies to leverage what they do best (late-stage development, regulatory management and commercialization) to ultimately generate our revenue. Ligand's OmniAb technology platform is a patent-protected transgenic animal platform used in the discovery of fully human mono- and bispecific therapeutic antibodies. The Captisol platform technology is a patent-protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. The Vernalis Design Platform (VDP) integrates protein structure determination and engineering, fragment screening and molecular modeling, with medicinal chemistry, to help enable success in novel drug discovery programs against highly-challenging targets. Ab Initio™ technology and services for the design and preparation of customized antigens enable the successful discovery of therapeutic antibodies against difficult-to-access cellular targets. Ligand has established multiple alliances, licenses and other business relationships with the world's leading pharmaceutical companies including Amgen, Merck, Pfizer, Sanofi, Janssen, Takeda, Gilead Sciences and Baxter International.

For more information, please visit www.ligand.com. Follow Ligand on Twitter @Ligand_LGND.

Forward-Looking Statements

This news release contains forward-looking statements by Ligand that involve risks and uncertainties and reflect Ligand's judgment as of the date of this release. These include statements regarding: the potential for future regulatory and financing milestones as well as royalties from net sales of PTX-022 if approved; Palvella's plans to initiate an open-label extension program; Palvella's expectation of topline results in the fourth quarter of 2020; the size of the PC patient population; and Palvella's expectations regarding the length and scope of patents covering PTX-022. Actual events or results may differ from Ligand's expectations due to risks and uncertainties, including; VALO could fail to reach its primary endpoints or show sufficient safety or efficacy to continue development or submit a new drug application (NDA) to the FDA; the FDA could require additional clinical trials in addition to the VALO study; the FDA could rescind Fast Track or Orphan Drug designations previously granted to PTX-022; even if approved, Palvella may not successfully launch PTX-022; and patents covering PTX-022 could be challenged or may not provide the expected scope of coverage to exclude other products used to treat PC. The failure to meet expectations with respect to any of the foregoing matters may reduce Ligand's stock price. Additional information concerning these and other important risk factors affecting Ligand can be found in Ligand's prior press releases as well as in Ligand's public periodic filings with the Securities and Exchange Commission. Ligand disclaims any intent or obligation to update these forward-looking statements beyond the date of this press release, except as required by law. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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