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Ligand Acquires Milestone and Royalty Rights to PTX-022 from Palvella Therapeutics

PTX-022 is a late-stage drug candidate targeting pachyonychia congenita

SAN DIEGO--(BUSINESS WIRE)-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** announces the acquisition of economic rights to PTX-022 from Palvella Therapeutics. PTX-022 is a novel, topical formulation of rapamycin currently in Phase 2/3 development for the treatment of pachyonychia congenita (PC), a rare skin disorder for which no FDA-approved treatment exists. Ligand will pay $10 million to Palvella Therapeutics and in return will receive a tiered royalty on net sales in the mid-to-upper single digits, as well as regulatory and financing milestones. Ligand will not incur any expenses to develop or commercialize PTX-022.

PC is a serious, chronically debilitating genetic disorder that results in malformation of the skin and severely limits the mobility and quality-of-life of those affected. PTX-022 has received Fast Track and Orphan Drug designations from the U.S. Food and Drug Administration (FDA).

“This transaction provides Palvella Therapeutics with capital to complete its planned clinical trials that could be the basis for registration and approval of a novel and important new medicine,” said John Higgins, Chief Executive Officer of Ligand. “The PTX-022 program is being managed by a team of distinguished and capable scientists who have identified a novel way to treat PC. This deal has the potential to provide lucrative economics to Ligand for a drug that could launch in 2022, should its development be successful and if it is approved by the FDA. Ligand is assembling a large collection of significant royalty-bearing assets and partnerships for drugs, many of which could launch over the next several years. Ligand expects to continue entering into license agreements from its existing technologies and will continue to explore ways to acquire new revenue streams through product investments, such as this deal with Palvella, or through company acquisitions.”

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 pachyonychia congenita.

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 that often necessitates the use of aids to assist movement or alternative forms of mobility such as crawling on ones hands and knees. PC affects up to 10,000 people in the U.S. and no FDA-approved therapies exist to treat the disorder.

About Palvella Therapeutics

Palvella Therapeutics is a rare disease biopharmaceutical company based in Wayne, Pennsylvania focused on developing and commercializing pathogenetically targeted therapies for debilitating, rare genetic diseases with no approved treatments.

Refer to www.palvellatx.com for additional detail.

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 Captisol® platform technology is a patent-protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. OmniAb® is a patent-protected transgenic animal platform used in the discovery of fully human mono- and bispecific therapeutic antibodies. 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.

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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 report. These forward-looking statements include comments regarding Palvella's planned clinical development program for PTX-022; the potential for future regulatory and financing milestones as well as royalties from net sales of PTX-022, if approved; Ligand's expectations that it will not incur additional expenses in connection with the development or commercialization of PTX-022 and the expectation that Palvella will have sufficient capital to complete its planned clinical trials for PTX-022; the possibility that a Phase 2/3 clinical trial could be the basis for registration, which means it would be sufficient to submit a new drug application (NDA) to the FDA for PTX-022; the possibility that PTX-022 will show clinical benefit to treat patients with PC; the size of the PC patient population; Ligand's expectations that a number of royalty-bearing assets could launch in the early 2020's timeframe; Ligand's expectations that it will enter into new license agreements using its existing technologies and potential to acquire new revenue streams through product investments or company acquisition; and Palvella's expectations regarding the length and scope of patents covering PTX-022. Actual events or results may differ from Ligand's expectations. For example, the development of PTX-022 is entirely dependent on Palvella's success and Ligand will have no ability to direct the development program; Palvella may abandon the development of PTX-022 if commercially reasonable; there can be no assurance that Palvella will be able to successfully develop PTX-022, including initiation of a Phase 2/3 clinical trial or filing an NDA to the FDA; the FDA could require additional clinical trials than the planned clinical trials and the Phase 2/3 clinical trial may not be able to serve as a sufficient basis for an NDA filing with the FDA; Palvella's planned Phase 2/3 clinical trial could fail to reach its primary endpoints or show sufficient safety or efficacy to continue development or submit an NDA to the FDA; 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; other products that Ligand expects will be launched by partners may fail their respective clinical development programs or may fail to launch successfully; 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. Many of these risks also apply to the other programs which comprise Ligand's shots-on-goal portfolio. 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 (including Ligand's current reliance on revenues based on sales of Promacta® and Kyprolis®, and various risks to which Ligand's Captisol® cyclodextrin operations are subject) can be found in Ligand's prior periodic filings with the Securities and Exchange Commission (including its Form 10-K filed on March 1, 2018), available at www.sec.gov, as updated by future period reports filed with the Securities and Exchange Commission. Ligand disclaims any intent or obligation to update these forward-looking statements beyond the date of this report. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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