December 26, 2014

# Ligand Partner Spectrum Pharmaceuticals Announces Submission of New Drug Application for Captisol-Enabled™ Melphalan

SAN DIEGO-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** announces that its partner Spectrum Pharmaceuticals (NASDAQ: SPPI) has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for Captisol-enabled™ Melphalan (CE-Melphalan) HCl for injection (propylene glycol-free) for use as a high-dose conditioning treatment prior to hematopoietic progenitor (stem) cell transplantation in patients with multiple myeloma. Spectrum is also seeking approval for the palliative treatment of patients with multiple myeloma for whom oral therapy is not appropriate.

According to Spectrum Pharmaceuticals, the Captisol-enabled Melphalan NDA was filed under a 505(b)(2) application and Spectrum expects FDA review to take approximately 10 months. Spectrum plans to launch this drug with its existing hematology/oncology sales force next year pending approval.

“We congratulate the team at Spectrum Pharmaceuticals on their development progress,” said John Higgins, Ligand’s President and Chief Executive Officer. “With a specialty sales force already in place, we look forward to Spectrum’s potential launch and commercial success with this valuable asset.”

Ligand licensed global development and commercialization rights to CE-Melphalan to Spectrum in 2013. Spectrum assumed the responsibility for a pivotal clinical trial and was responsible for filing the NDA. Under the license agreement, Ligand received a license fee and is eligible to receive revenue from Captisol material sales, milestone payments, as well as royalties on net sales following potential commercialization.

According to previous announcements by Spectrum, the Phase 2 pivotal trial evaluating CE-Melphalan was a multicenter trial evaluating safety and efficacy. The primary objective of the study was to determine the overall safety and toxicity profile in multiple myeloma patients receiving 200 mg/m$^2$ of CE-Melphalan as myeloablative therapy prior to autologous stem cell transplantation (ASCT). The secondary objectives evaluated the efficacy of CE-Melphalan in this patient population as measured by Multiple Myeloma Response Rate (according to International Myeloma Working Group [IMWG] criteria), and the rates of myeloablation and engraftment. The primary as well as secondary endpoints of this Phase 2 trial were met.

In a previous clinical study, CE- Melphalan met the requirements for establishment of bioequivalence to the currently approved commercial intravenous formulation of melphalan. This demonstrated bioequivalence of CE-Melphalan may facilitate the use of this new more

stable, propylene glycol-free Captisol formulation of melphalan following the potential approval of the NDA.

**About Captisol-Enabled Melphalan**

Captisol-Enabled, Propylene Glycol-free Melphalan is a novel intravenous formulation of melphalan being investigated for the multiple myeloma transplant setting, for which it has been granted an Orphan Drug Designation by the FDA. This formulation eliminates the use of propylene glycol, which has been reported to cause renal and cardiac side effects that limit the ability to deliver higher doses of therapeutic compounds. The use of the Captisol® technology to reformulate melphalan also improves its stability and is anticipated to allow for slower infusion rates and longer administration durations, potentially enabling clinicians to safely achieve a higher dose intensity for pre-transplant chemotherapy.

**About Captisol®**

Captisol is a patent-protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. Captisol was invented and initially developed by scientists in the laboratories of Dr. Valentino Stella at the University of Kansas' Higuchi Biosciences Center for specific use in drug development and formulation. This unique technology has enabled six FDA-approved products, including Onyx Pharmaceuticals' Kyprolis®, Baxter International's Nexterone® and Merck's NOXAFIL IV. There are more than 30 Captisol-enabled products currently in clinical development.

**About Ligand Pharmaceuticals**

Ligand is a biopharmaceutical company with a business model that is based upon the concept of developing or acquiring royalty revenue generating assets and coupling them to a lean corporate cost structure. Ligand's goal is to produce a bottom line that supports a sustainably profitable business. By diversifying the portfolio of assets across numerous technology types, therapeutic areas, drug targets and industry partners, we offer investors an opportunity to invest in the increasingly complicated and unpredictable pharmaceutical industry. In comparison to its peers, we believe Ligand has assembled one of the largest and most diversified asset portfolios in the industry with the potential to generate revenue in the future. These therapies seek to address the unmet medical needs of patients for a broad spectrum of diseases including diabetes, hepatitis, muscle wasting, Alzheimer's disease, dyslipidemia, anemia, asthma and osteoporosis. Ligand's Captisol platform technology is a patent-protected, chemically modified cyclodextrin with a structure designed to optimize the solubility and stability of drugs. Ligand has established multiple alliances with the world's leading pharmaceutical companies including GlaxoSmithKline, Onyx Pharmaceuticals (a subsidiary of Amgen Inc.), Merck, Pfizer, Baxter International, Lundbeck Inc., Eli Lilly & Co. and Spectrum Pharmaceuticals. Please visit www.captisol.com for more information on Captisol and www.ligand.com for more information on Ligand.

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**Forward-Looking Statements**

This news release contains certain forward-looking statements by Ligand that involve risks and uncertainties and reflect Ligand's judgment as of the date of this release. These

statements include those related to potential future regulatory approval and launch of products and product candidates, including Spectrum Pharmaceuticals' CE Melphalan, and future financial performance, market potential and other developments regarding Ligand and Spectrum Pharmaceuticals' CE Melphalan. Actual events or results may differ from our expectations. There can be no assurance that Spectrum Pharmaceuticals' CE Melphalan will achieve commercial success, that any of our partners will continue clinical development of any compound(s); that clinical development will be successful; that future clinical trial data will be favorable or that such trials will confirm any improvements over other products or lack negative impacts; that drugs will receive required regulatory approvals or that they will be commercially successful, that any future milestone or royalty payments will be received, or that if any future milestones or royalties are received that they will not be subject to sharing obligations with any third party. Our stock price could be harmed if any of these events or trends fails to occur, is delayed or otherwise differs from expectations. Additional information concerning these and other risk factors affecting Ligand's business can be found on the company's prior press releases as well as in public periodic filings with the Securities and Exchange Commission, available via www.ligand.com. Ligand disclaims any intent or obligation to update these forward-looking statements beyond the date of this release. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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