

Ligand Partner GSK Announces the Start of a Phase 3 Study with Eltrombopag in Patients with Myelodysplastic Syndromes

SAN DIEGO-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** announces that its partner GlaxoSmithKline (GSK) plc has started a Phase 3 study to evaluate the platelet supportive care effects of eltrombopag (Promacta™/Revolade™) in combination with azacitidine (the current standard of care) versus placebo in combination with azacitidine in intermediate-1, intermediate-2 or high-risk patients with myelodysplastic syndromes (MDS). The global SUPPORT (TRC112121) study will assess the proportion of patients who are platelet transfusion free during the first four cycles of treatment.

MDS is a type of cancer in which the bone marrow does not make enough healthy blood cells and there are abnormal (blast) cells in the blood and/or bone marrow¹. The disease usually manifests itself with one or more cytopenias, or reductions in the number of blood cells, and patients typically present with complications related to anemia (fatigue), neutropenia (infections) or thrombocytopenia (bleeding)². MDS may evolve into acute myeloid leukemia (AML) in up to 45% of patients.³

“With the start of the SUPPORT trial GSK continues to advance eltrombopag in multiple indications to boost platelets to treat thrombocytopenia, including clinical programs and regulatory approvals in oncology and infectious disease indications,” said John Higgins, President and CEO of Ligand Pharmaceuticals. “GSK has maintained a comprehensive global view of brand management and a deep commitment to maximizing the reach of Promacta/Revolade to patients with significant unmet needs. The SUPPORT trial is one of more than 25 active clinical trials underway with eltrombopag.”

About the SUPPORT study

SUPPORT (**StUdy of eltromboPag in myelodysPlastic SyndrOmes Receiving azaciTidine**) is a Phase 3, randomized, double-blind, placebo-controlled, multicenter study of eltrombopag or placebo in combination with azacitidine in subjects with MDS. It is estimated that 350 patients across 30 countries and 156 study sites with a baseline platelet count of $< 75 \text{ Gi/L}$, and intermediate-1, intermediate-2 or high-risk MDS (by International Prognostic Scoring System) will be enrolled in the study. Eligible patients will be randomized to receive either eltrombopag (200 mg once daily [100 mg for East Asians]) plus azacitidine (75 mg/m^2 subcutaneously once daily for 7 days) every 28 days, for at least 6 cycles, or placebo plus azacitidine. Dose modifications of eltrombopag or placebo will be permitted to ensure that patient platelet counts remain at a safe and effective level.

The primary efficacy endpoint will determine the platelet supportive care effects of eltrombopag in combination with azacitidine versus placebo in combination with azacitidine

by comparing the proportion of subjects receiving eltrombopag plus azacitidine who are platelet transfusion free during the first 4 cycles of azacitidine, versus those treated with placebo plus azacitidine. Secondary objectives will compare the following between treatment arms: overall survival, disease response, hematologic improvement, platelet and red blood cell transfusions, adverse events ($\geq$ Grade 3), safety and tolerability, health-related quality of life and medical resource utilization.

About Eltrombopag (Promacta™/Revolade™)

Eltrombopag – marketed as Promacta™ in the U.S. and as Revolade™ in Europe and other countries around the world – works by interacting with the TPO receptor leading to increased platelet production. Eltrombopag is not approved or licensed anywhere in the world for use in patients with myelodysplastic syndromes.

For full U.S. Prescribing Information for Promacta® (eltrombopag), including Boxed Warning, please visit: <https://www.gsksource.com/gskprm/htdocs/documents/PROMACTA-PI-MG-COMBINED.PDF>. For the European Union (EU) Summary of Product Characteristics (SPC) for Revolade® (eltrombopag) in approved indications, please visit <http://health.gsk.com/>.

Promacta™ and Revolade™ are trademarks of the GSK group of companies.

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 our 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 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, Eli Lilly & Co. and Spectrum Pharmaceuticals. Please visit www.captisol.com for more information on Captisol. For more information on Ligand, please visit www.ligand.com.

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References

¹ National Cancer Institute. Myelodysplastic Syndromes Treatment (PDQ®). Available at: <http://www.cancer.gov/cancertopics/pdq/treatment/myelodysplastic/Patient/page1>. Accessed June 12, 2014.

² Faderl S, Kantarjian H. Myelodysplastic Syndromes. In: Devita V, Hellman S, Rosenberg

S, editor. Cancer: Principles & Practice of Oncology 7th Edition. Philadelphia, PA:Lippincott, Williams & Wilkins, 2005:2144-2154.

³ Greenberg P, Cox C, LeBeau MM, Fenaux P, Morel P, Sanz G, Sanz M, Vallespi T, Hamblin T, Oscier D, Ohyashiki K, Toyama K, Aul C, Mufti G, Bennett J. International scoring system for evaluating prognosis in myelodysplastic syndromes. Blood. 1997; 89: 2079-2088.

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 forward-looking statements include comments regarding eltrombopag, data analysis and evaluation of eltrombopag, utility or potential benefits to patients, the potential commercial market for eltrombopag and plans for continued development and further studies of eltrombopag. Actual events or results may differ from Ligand's expectations. For example, there can be no assurance that other trials or evaluations of eltrombopag will be favorable or that they will confirm results of previous studies, that data evaluation will be completed or demonstrate any hypothesis or endpoint, that eltrombopag will provide utility or benefits to certain patients, that any presentations will be favorably received, that eltrombopag will be useful, that marketing applications will be filed or, if filed, approved, or that clinical or commercial development of eltrombopag will be initiated, completed or successful or that our rights to eltrombopag will not be successfully challenged. 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 risk factors affecting Ligand can be found in prior press releases available at www.ligand.com as well as in public periodic filings with the Securities and Exchange Commission, available at www.sec.gov. Ligand disclaims any intent or obligation to update these forward-looking statements beyond the date of this press release. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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