

Ligand Partner GSK Announces Results of Phase 3 PETIT2 Study of Eltrombopag (Promacta™/Revolade™) in Pediatric Patients with Chronic Immune Thrombocytopenia

SAN DIEGO-- **Ligand Pharmaceuticals Incorporated (NASDAQ: LGND)** announces that its partner, GlaxoSmithKline (GSK) plc, presented the results from the Phase 3 PETIT2 study evaluating the efficacy of eltrombopag vs. placebo in pediatric patients with chronic immune (idiopathic) thrombocytopenic purpura (cITP). Eltrombopag - marketed as Promacta™ in the U.S. and as Revolade™ in Europe and other countries across the world - met its primary endpoint, achieving a statistically significant improvement in platelet counts with almost 40 percent of patients treated with eltrombopag attaining a consistent platelet response for 6 of 8 weeks compared to placebo (39.7 percent vs. 3.4 percent, respectively, $p < 0.001$).

The PETIT2 study results were highlighted today as part of a Press Briefing and Oral Presentation at the European Hematology Association Annual Congress in Milan, Italy.

GSK also announced that it is moving forward with planned regulatory submissions for a pediatric indication in cITP later this year.

Efficacy results for PETIT2 were consistent across age cohorts. The safety profile was consistent with the established profile for eltrombopag and no new safety concerns were observed. The most common adverse events (AEs) occurring most frequently in the eltrombopag arm included nasopharyngitis, rhinitis, cough and respiratory tract infection. Grade 3/4 AEs occurred in 12.7 percent of patients treated with eltrombopag and 10.3 percent of patients in the placebo group. Serious AEs were reported in 8 percent of eltrombopag-treated patients vs. 14 percent in the placebo arm.

Additional results from an early phase study of eltrombopag in previously treated pediatric patients with cITP were also presented at the European Hematology Association Annual Congress.

About cITP

Immune (idiopathic) thrombocytopenic purpura (ITP) - characterized by a low platelet count - affects as many as 5 in 100,000 children each year.¹ While many children with ITP do not require treatment and/or their disease resolves, up to 30 percent of patients experience persistent disease at 12 months and are diagnosed with cITP.^{2, 3, 4} Patients with pediatric

cITP are at a high risk of severe bleeding.

About PETIT2

PETIT2 was 2-part, double-blind, randomized placebo-controlled and open-label study to investigate the efficacy, safety and tolerability of eltrombopag in pediatric patients with previously treated cITP. The multi-center study enrolled 93 subjects at 38 centers in 14 countries.

The primary objective of the study was to assess the efficacy of eltrombopag, relative to placebo, in achieving platelet counts of ≥ 50 Gi/L among pediatric patients with previously treated cITP for at least 12 months. The initial phase of the study compared eltrombopag to placebo for 13 weeks. All study participants were then treated with eltrombopag in the second phase of the study (through to week 24).

About Eltrombopag (Promacta™/Revolade™)

Eltrombopag is not approved or licensed anywhere in the world for use in chronic immune (idiopathic) thrombocytopenic purpura in the pediatric setting.

For full US 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.

Important Safety Information for Eltrombopag

WARNING: RISK FOR HEPATIC DECOMPENSATION IN PATIENTS WITH CHRONIC HEPATITIS C

In patients with chronic hepatitis C, eltrombopag in combination with interferon and ribavirin may increase the risk of hepatic decompensation.

Hepatotoxicity:

Eltrombopag can cause liver enzyme elevations. Measure serum ALT, AST, and bilirubin prior to initiation of eltrombopag, every 2 weeks during the dose adjustment phase, and monthly following establishment of a stable dose. Eltrombopag inhibits UGT1A1 and OATP1B1, which may lead to indirect hyperbilirubinemia. If bilirubin is elevated, perform fractionation. Evaluate abnormal serum liver tests with repeat testing within 3 to 5 days. If the abnormalities are confirmed, monitor serum liver tests weekly until resolved or stabilized.

Discontinue eltrombopag if ALT levels increase to $\geq 3X$ upper limit of normal (ULN) in patients with normal liver function or $\geq 3X$ baseline in patients with pre-treatment elevations in transaminases and are: progressively increasing; or persistent for ≥ 4 weeks; or accompanied by increased direct bilirubin; or accompanied by clinical symptoms of liver injury or evidence for hepatic decompensation.

If the potential benefit for reinitiating treatment with eltrombopag is considered to outweigh

the risk for hepatotoxicity, then consider cautiously reintroducing eltrombopag and measure serum liver tests weekly during the dose adjustment phase. Hepatotoxicity may reoccur if eltrombopag is reinitiated. If liver tests abnormalities persist, worsen or recur, then permanently discontinue eltrombopag.

Thrombotic/Thromboembolic Complications:

Thrombotic/thromboembolic complications may result from increases in platelet counts with eltrombopag. Reported thrombotic/thromboembolic complications included both venous and arterial events and were observed at low and at normal platelet counts. Consider the potential for an increased risk of thromboembolism when administering eltrombopag to patients with known risk factors for thromboembolism. To minimize the risk for thrombotic/thromboembolic complications, do not use eltrombopag in an attempt to normalize platelet counts. Follow the dose adjustment guidelines to achieve and maintain target platelet counts.

Cataracts:

In the 3 controlled clinical trials in chronic ITP, cataracts developed or worsened in 15 (7%) patients who received 50 mg eltrombopag daily and 8 (7%) placebo-group patients. In the extension trial, cataracts developed or worsened in 4% of patients who underwent ocular examination prior to therapy with eltrombopag.

Cataracts were observed in toxicology studies of eltrombopag in rodents. Perform a baseline ocular examination prior to administration of eltrombopag and, during therapy with eltrombopag, regularly monitor patients for signs and symptoms of cataracts.

Laboratory Monitoring:

In patients with chronic ITP, monitor serum liver tests (see Hepatotoxicity section). During therapy with eltrombopag, assess complete blood counts (CBCs) with differentials, including platelet counts, weekly until a stable platelet count has been achieved. Monitor platelet counts monthly thereafter. Obtain CBCs with differentials, including platelet counts, weekly for at least 4 weeks following discontinuation of eltrombopag.

Drug Interactions:

Eltrombopag must not be taken within 4 hours of any medications or products containing polyvalent cations such as antacids, dairy products, and mineral supplements.

Adverse Reactions:

The most common adverse reactions in 3 placebo-controlled clinical trials in chronic ITP patients ($\geq 3\%$ and greater than placebo) for eltrombopag versus placebo were: nausea (9% vs. 3%), diarrhea (9% vs. 7%), upper respiratory tract infection (7% vs. 6%), vomiting (6% vs. $<1\%$), increased ALT (5% vs. 3%), myalgia (5% vs. 2%), urinary tract infection (5% vs. 3%), oropharyngeal pain (4% vs. 3%), increased AST (4% vs. 2%), pharyngitis (4% vs. 2%), back pain (3% vs. 2%), influenza (3% vs. 2%), paresthesia (3% vs. 2%), and rash (3% vs. 2%).

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 Terrell DR, Beebe LA, Vesely SK, Neas BR, Segal JB, George JN. Am J Hematol. 2010 Mar;85(3):174-80. doi: 10.1002/ajh.21616.

² El-Bostany E, El-Ghoroury E, and El-Ghafar E. Anti-Beta 2 Glycoprotein I in childhood immune thrombocytopenic purpura. Blood Coagulation and Fibrinolysis. 2008;19(1):26-31.

³ BCSH, (British Committee for Standards in Haematology). Guidelines for the investigation and management of idiopathic thrombocytopenic purpura in adults, children and in pregnancy. Br J Haematol. 2003;120:574-596.

⁴ Walker R.W., Walker W. Idiopathic thrombocytopenia, initial illness and long term follow up. Archives of Disease in Childhood. 1984;59:316-322.

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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