

Ligand Partner GlaxoSmithKline Gains Priority Review Designation for Promacta™/Revolade™ for Severe Aplastic Anemia

SAN DIEGO-- Ligand Pharmaceuticals Incorporated (NASDAQ: LGND) today announced that the U.S. Food and Drug Administration (FDA) has granted its partner GlaxoSmithKline plc (LSE:GSK) Priority Review designation for Promacta™/Revolade™ (eltrombopag) for the treatment of cytopenias in patients with severe aplastic anemia (SAA) who have had insufficient response to immunosuppressive therapy.

SAA is a rare disorder in which the bone marrow fails to make enough new blood cells. There are no approved therapies available for SAA patients who are unresponsive to initial immunosuppressive therapy (IST). Of those patients unresponsive to initial IST, approximately 40% die from infection or bleeding within five years of their diagnosis.ⁱ

In February 2014, Ligand announced that GSK had gained Breakthrough Therapy designation for Promacta in Severe Aplastic Anemia. Also in February 2014, GSK announced the submission of a supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration (FDA) for Promacta for the treatment of cytopenias (a reduction in blood cells) in patients with SAA who have had an insufficient response to IST. The sNDA is based on the results from an open-label, Phase II National Institute of Health (NIH) study (09-H-0154) of eltrombopag in 43 heavily pre-treated SAA patients with an insufficient response to IST.

About Priority Review Designation

A Priority Review designation is assigned to applications for drugs that, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis or prevention of serious conditions and means FDA's goal is to take action on an application within 6 months, compared to 10 months under standard review.

Significant improvement may be demonstrated by the following examples:

- Evidence of increased effectiveness in treatment, prevention, or diagnosis of condition;
- Elimination or substantial reduction of a treatment-limiting drug reaction;
- Documented enhancement of patient compliance that is expected to lead to an improvement in serious outcomes; or
- Evidence of safety and effectiveness in a new subpopulation.ⁱⁱ

About Breakthrough Therapy Designation

The Breakthrough Therapy designation was enacted as part of the 2012 FDA Safety and Innovation Act (FDASIA) and is intended to expedite development and review of drugs to treat serious or life-threatening medical conditions when preliminary clinical evidence demonstrates that the drug may have substantial improvement on at least one clinically significant endpoint over available therapies. Breakthrough Therapy designation includes all the features of the Fast Track designation, as well as more intensive guidance from the FDA on a drug's clinical development program.ⁱⁱ

About Promacta

For full U.S. Prescribing Information and Medication Guide and EU Patient Information Leaflet or Summary of Product Characteristics (SPC) for eltrombopag please visit <http://www.gsk.com/products.html>.

Promacta is not approved or licensed anywhere in the world for use in SAA.

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.

Follow Ligand on Twitter @Ligand_LGND.

References

ⁱ Valdez JM, et al. Decreased Infection-Related Mortality and Improved Survival in Severe Aplastic Anemia in the Past Two Decades. *Clinical Infectious Diseases* 2011;52(6):726–735.

ⁱⁱ U.S. Food and Drug Administration. Fast Track, Breakthrough Therapy, Accelerated Approval and Priority Review. Available at: <http://www.fda.gov/forconsumers/byaudience/forpatientadvocates/speedingaccesstoimportant> Accessed April 28, 2014.

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 and other drug candidates, 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 or other product candidates 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 or other product candidates will provide utility or benefits to certain patients, that any presentations will be favorably received, that eltrombopag or other product candidates will be useful, that marketing applications will be filed or, if filed, approved, or that clinical or commercial development of these product candidates will be initiated, completed or successful or that our rights to eltrombopag and other related product candidates 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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