Celgene Corporation and Acceleron Pharma Announce U.S. FDA Accepts Luspatercept Biologics License Application in Myelodysplastic Syndromes and Beta-Thalassemia

June 4, 2019

U.S. Food and Drug Administration grants priority review for beta-thalassemia indication and sets target action date of December 4, 2019

U.S. Food and Drug Administration sets target action date of April 4, 2020 for myelodysplastic syndromes indication

Luspatercept EU Marketing Authorization Application also validated

SUMMIT, N.J. & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 4, 2019-- Celgene Corporation (NASDAQ: CELG) and Acceleron Pharma Inc. (NASDAQ: XLRN) today announced that the U.S. Food and Drug Administration (FDA) has accepted Celgene's Biologics License Application (BLA) for luspatercept, an investigational erythroid maturation agent, for the treatment of adult patients with very low to intermediate-risk myelodysplastic syndromes (MDS)-associated anemia who have ring sideroblasts and require red blood cell (RBC) transfusions, and for the treatment of adult patients with beta-thalassemia-associated anemia who require RBC transfusions. The FDA has granted Priority Review to this BLA for the evaluation of the beta-thalassemia indication and set a Prescription Drug User Fee Act (PDUFA), or target action, date of December 4, 2019. The FDA has also set a PDUFA date of April 4, 2020 for the evaluation of the MDS indication.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20190604005548/en/

"The acceptance of the luspatercept filings and granting of the U.S. priority review for beta-thalassemia represent another important step in delivering this novel therapy to patients in need," said Jay Backstrom, M.D., Chief Medical Officer for Celgene. "We believe that luspatercept can play a critical role in treating the anemia associated with these serious blood diseases, and with these milestones achieved we look forward to working closely with the agency to move this therapy toward approval."

The safety and efficacy results provided in the application are from the pivotal phase 3 studies MEDALIST and BELIEVE, which evaluated the ability of luspatercept to effectively treat the anemia associated with MDS and beta-thalassemia, respectively. MEDALIST and BELIEVE were both presented at the 2018 American Society of Hematology annual meeting, where MEDALIST was included in the plenary session.

The companies also announced that Celgene's Marketing Authorization Application in the EU has been successfully validated and the review is now underway.

"The ongoing U.S. and European regulatory reviews of the luspatercept filings in MDS and beta-thalassemia strongly support our primary goal, which has always been to bring a potentially transformative new treatment to these patients with unmet clinical need as quickly as possible," said Habib Dable, President and Chief Executive Officer of Acceleron. "At the same time, we continue to explore the ability of luspatercept to address anemia in additional settings, including patients with treatment-naïve MDS, non-transfusion-dependent beta-thalassemia, and myelofibrosis."

Luspatercept is an investigational therapy that is not approved for any use in any country.

About Luspatercept

Luspatercept is a first-in-class erythroid maturation agent (EMA) that regulates late-stage red blood cell maturation. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. A phase 3 trial (COMMANDS) in ESA-naïve, lower-risk MDS patients, the BEYOND phase 2 trial in non-transfusion-dependent beta-thalassemia, and a phase 2 trial in myelofibrosis are ongoing. For more information, please visit www.clinicaltrials.gov.

About MEDALIST

MEDALIST is a phase 3, randomized, double blind, placebo-controlled, multi-center study evaluating the safety and efficacy of luspatercept in adults with very low-, low-, or intermediate-risk myelodysplastic syndromes (MDS). All patients were RBC transfusion dependent and were either refractory or intolerant to prior erythropoiesis-stimulating agent (ESA) therapy or were ESA naïve with endogenous serum erythropoietin ≥ 200 U/L and had no prior treatment with disease modifying agents. The median age of the patients enrolled in the trial was 71 years in the luspatercept treatment group and 72 years in the placebo group. Median transfusion burden in both treatment arms was 5 RBC units/8 weeks. 229 patients were randomized to receive either luspatercept 1.0 mg/kg (153 patients) or placebo (76 patients) by subcutaneous injection once every 21 days. The study was conducted at 65 sites in 11 countries.

About BELIEVE

BELIEVE is a phase 3, randomized, double blind, placebo-controlled multicenter study comparing luspatercept + best supportive care (BSC) versus placebo + BSC in adults with beta-thalassemia patients who require regular RBC transfusions. The median age of the patients was 30 years in both treatment arms. 336 patients were randomized to receive either luspatercept 1.0 mg/kg (224 patients) or placebo (112 patients) by subcutaneous injection every 21 days for up to 48 weeks. Crossover to the luspatercept treatment groups was allowed after unblinding based on the recommendation of an independent Data Safety Monitoring Committee; patients treated with luspatercept will be followed for up to 3 years. The study was conducted at 65 sites in 15 countries.

About Celgene

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery,

development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit www.celgene.com.

Follow Celgene on Social Media: Twitter, Pinterest, LinkedIn, Facebook and YouTube.

About Acceleron

Acceleron is a clinical-stage biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. The Company's leadership in the understanding of TGF-beta superfamily biology and protein engineering generates innovative compounds that engage the body's ability to regulate cellular growth and repair.

Acceleron focuses its research and development efforts in hematologic, neuromuscular, and pulmonary diseases. In hematology, the Company and its global collaboration partner, Celgene, are developing luspatercept for the treatment of chronic anemia in myelodysplastic syndromes, beta-thalassemia, and myelofibrosis. Acceleron is also advancing its neuromuscular program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease and is conducting a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit www.acceleronpharma.com. Follow Acceleron on Social Media: @AcceleronPharma and LinkedIn.

FORWARD-LOOKING STATEMENTS

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of, and plans relating to the collaboration between Acceleron and Celgene; the potential of luspatercept as a therapeutic drug; and the benefit of each company's strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "will," "would," "could," "potential," "possible," "hope" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs. For example, there can be no guarantee that luspatercept will be successfully developed or complete necessary clinical phases. Forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including: results of clinical trials, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; the ability to obtain and maintain requisite regulatory approvals and to enroll patients in planned clinical trials; the ability to obtain, maintain and enforce patent and other intellectual property protection for luspatercept; the ability to maintain key collaborations; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in each company's public filings with the Securities and Exchange Commission and with respect to Celgene includes risk factors related to the proposed transaction between Bristol-Myers Squibb and Celgene, such as, but not limited to, the risks that: management's time and attention is diverted on transaction related issues; disruption from the transaction makes it more difficult to maintain business, contractual and operational relationships; and Bristol-Myers Squibb, Celgene or the combined company is unable to retain key personnel. Any forward-looking statements contained in this press release speak only as of the date hereof, and neither company has any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

Hyperlinks are provided as a convenience and for informational purposes only. Neither Celgene nor Acceleron bears responsibility for the security or content of external websites or websites outside of their respective control.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190604005548/en/

Source: Celgene Corporation

Celgene Corporation
Investors:
+1-908-673-9628
ir@celgene.com
or
Media:
+1-908-673-2275
media@celgene.com

Acceleron Pharma Inc.
Investors:
Todd James, IRC, (617) 649-9393
Vice President, Investor Relations and Corporate Communications
or
Media:
Matt Fearer, (617) 301-9557
Director, Corporate Communications