



## **Celgene and Acceleron Announce Luspatercept Achieved Primary and All Key Secondary Endpoints in Phase III 'BELIEVE' Study in Adults with Transfusion-Dependent Beta-Thalassemia**

July 9, 2018

*Results showed a significant reduction in transfusion burden compared to placebo*

*Safety profile generally consistent with previously reported data*

*Regulatory submissions planned in the United States and Europe in the first half of 2019*

SUMMIT, N.J. & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 9, 2018-- Celgene Corporation (NASDAQ: CELG) and Acceleron Pharma Inc. (NASDAQ: XLRN) today announced results from a phase III, randomized, double-blind, multi-center clinical study (BELIEVE). Luspatercept achieved a highly statistically significant improvement in the primary endpoint of erythroid response, which was defined as at least a 33 percent reduction from baseline in red blood cell (RBC) transfusion burden with a reduction of at least 2 units during the protocol-defined period of 12 consecutive weeks, from week 13 to week 24, compared to placebo.

BELIEVE evaluated the efficacy and safety of luspatercept plus best supportive care versus placebo plus best supportive care in adults with transfusion-dependent beta-thalassemia.

In addition to achieving the primary endpoint of the study, luspatercept also met all key secondary endpoints of demonstrating statistically significant improvements in RBC transfusion burden from baseline of at least a 33 percent reduction during the period from week 37 to week 48, at least a 50 percent reduction during the period from week 13 to week 24, at least a 50 percent reduction during the period from week 37 to week 48, and a mean change in transfusion burden from week 13 to week 24.

Adverse events observed in the study were generally consistent with previously reported data.

"For decades, the management of beta-thalassemia in adults has been limited to transfusions and iron chelation. Reduction of transfusion burden represents an important step forward for patients with this rare and debilitating blood disease," said Jay Backstrom, M.D., Chief Medical Officer for Celgene. "We thank the patients, as well as their families and physicians, for their participation in the BELIEVE study."

"The BELIEVE study marks the second positive phase III study for luspatercept and underscores the potential of this erythroid maturation agent to impact a range of diseases associated with chronic anemia," said Habib Dable, President and Chief Executive Officer of Acceleron. "We continue to explore luspatercept across our broader development programs, including non-transfusion dependent beta-thalassemia in the ongoing BEYOND study."

The companies also recently announced that luspatercept met the primary and key secondary endpoints in the MEDALIST study, a phase III, randomized, double-blind, multi-center clinical trial evaluating the efficacy and safety of luspatercept versus placebo in patients with IPSS-R very low, low or intermediate risk myelodysplastic syndromes (MDS) with chronic anemia and refractory to, intolerant of, or ineligible for treatment with an erythropoietin-stimulating agent (ESA), ring sideroblast-positive and require frequent RBC transfusions.

Data from BELIEVE and MEDALIST will be submitted to a future medical meeting in 2018. The companies plan to submit regulatory applications for luspatercept in the United States and Europe in the first half of 2019.

Luspatercept is not approved for any indication in any geography.

### **About Luspatercept**

Luspatercept is a first-in-class erythroid maturation agent (EMA) that is believed to regulate late-stage red blood cell maturation. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Phase III clinical trials continue to evaluate the safety and efficacy of luspatercept in patients with MDS (the MEDALIST trial) and in patients with beta-thalassemia (the BELIEVE trial). A Phase III trial is being planned in first-line, lower-risk, MDS patients (the COMMANDS trial). The BEYOND Phase II trial in non-transfusion-dependent beta-thalassemia and a Phase II trial in myelofibrosis are ongoing. For more information, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### **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](http://www.celgene.com). Follow Celgene on Social Media: [@Celgene](#), [Pinterest](#), [LinkedIn](#), [Facebook](#) and [YouTube](#).

### **About Acceleron**

Acceleron is a Cambridge-based, 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 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 franchise with two distinct Myostatin+ agents, ACE-083 and ACE-2494, and a pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit [www.acceleronpharma.com](http://www.acceleronpharma.com). Follow Acceleron on Social Media: [@AcceleronPharma](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleron-pharma).

## 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," "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. 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.*

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