



June 1, 2017

Celgene and Acceleron Complete Target Enrollment in the MEDALIST and BELIEVE Phase 3 Studies of Luspatercept in Myelodysplastic Syndromes and Beta-Thalassemia

- Companies expect to report top-line results from the Phase 3 studies in mid-2018 -

SUMMIT, N.J. & CAMBRIDGE, Ma.--(BUSINESS WIRE)-- Celgene Corporation (NASDAQ: CELG) and Acceleron Pharma Inc. (NASDAQ: XLRN) today announced that they have completed target enrollment in the MEDALIST and BELIEVE Phase 3 studies of luspatercept in patients with myelodysplastic syndromes (MDS) and beta-thalassemia. The Companies expect to report top-line results from the clinical trials in the middle of 2018. Luspatercept is being developed to treat a range of hematologic diseases including MDS, beta-thalassemia, and myelofibrosis as part of a global collaboration between Acceleron and Celgene.

"We are excited to have completed target enrollment in our MEDALIST and BELIEVE Phase 3 studies of luspatercept, ahead of schedule, and look forward to reporting top-line results in the middle of next year," said Michael Pehl, President, Hematology and Oncology for Celgene. "Patients suffering from both diseases have limited treatment options to improve their underlying anemia. We believe that luspatercept may be a potentially paradigm-changing treatment option for patients and physicians alike."

"The rapid pace of patient recruitment in our global Phase 3 trials reflects the clear need for new MDS and beta-thalassemia therapies that can significantly reduce or eliminate dependence on red blood cell transfusions," said Habib Dable, President and Chief Executive Officer of Acceleron. "We are grateful for the support and dedication of the MEDALIST and BELIEVE study investigators, our patient advocacy partners, and most importantly the more than 500 patients and their families who are participating in our studies. I would also like to acknowledge the strong collaborative effort of the Celgene and Acceleron teams that led to this important achievement."

The MEDALIST Phase 3 trial has enrolled 210 patients with lower-risk MDS. The BELIEVE Phase 3 trial has enrolled 300 patients with transfusion dependent beta-thalassemia. Patients who are currently in screening remain eligible for randomization into both Phase 3 studies. The trials will remain blinded for both the primary and secondary endpoints until the end of the 48-week treatment period for all randomized patients.

About the MEDALIST Study

The MEDALIST Phase 3 trial is a randomized, double-blind, placebo-controlled, global study designed to evaluate the efficacy and safety of luspatercept in patients with ring sideroblasts (RS+), lower-risk MDS with a baseline RBC transfusion burden of at least 2 units per 8 weeks over the 16-week period prior to treatment. The primary endpoint of the study is the proportion of patients who are red blood cell (RBC) transfusion independent over any consecutive 8-week period through week 24. Secondary endpoints include duration of RBC transfusion independence and proportion of patients achieving a modified hematologic improvement - erythroid (HI-E) per the International Working Group over any consecutive 8-week period during treatment. Patients were randomized 2:1, luspatercept to placebo treatment, administered subcutaneously every 3 weeks for 48 weeks. The MEDALIST study is being conducted at 74 investigational sites in 11 countries.

About the BELIEVE Study

The BELIEVE Phase 3 trial is a randomized, double-blind, placebo-controlled, global study designed to evaluate the efficacy and safety of luspatercept in patients with transfusion dependent beta-thalassemia. The primary endpoint of the study is the proportion of patients achieving a $\geq 33\%$ reduction in red blood cell (RBC) transfusion burden from Week 13 to Week 24 compared to the baseline 12-week period prior to treatment. Secondary endpoints include reductions in RBC transfusion burden from Week 37 to Week 48 compared to baseline. Beta-thalassemia patients in the trial had a baseline RBC transfusion burden of 6 to 20 units over the 24-week period prior to treatment. Patients were randomized 2:1, luspatercept to placebo treatment, administered subcutaneously every 3 weeks for 48 weeks. The BELIEVE study is being conducted at 73 investigational sites in 15 countries.

About Luspatercept

Luspatercept is a modified activin receptor type IIB fusion protein that acts as a ligand trap for members in the transforming growth factor-beta superfamily involved in the late stages of erythropoiesis (red blood cell production). Luspatercept regulates late-stage erythrocyte (red blood cell) precursor cell differentiation and maturation. This mechanism of action is distinct from that of erythropoiesis-stimulating agents (ESAs), which stimulate the proliferation of early-stage erythrocyte progenitor cells. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Acceleron and Celgene are conducting two Phase 3 clinical trials that are designed to evaluate the safety and efficacy of luspatercept in patients with myelodysplastic syndromes (the "MEDALIST" study) and in patients with beta-thalassemia (the "BELIEVE" study). For more information, please visit www.clinicaltrials.gov.

Luspatercept is an investigational compound that is not approved or use in any country.

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: [@Celgene](#), [Pinterest](#), [LinkedIn](#), [FaceBook](#) and [YouTube](#).

About Acceleron

Acceleron is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases. Its pioneering research platform leverages the powerful biology behind the body's ability to rebuild and repair its own cells and tissues. This approach to drug discovery has generated four therapeutic candidates that are currently in clinical trials. Acceleron's lead therapeutic candidate, luspatercept, is being evaluated in Phase 3 studies for the treatment of the hematologic diseases myelodysplastic syndromes (MDS) and beta-thalassemia under a global partnership with Celgene Corporation. Acceleron is also advancing clinical programs in the fields of oncology and neuromuscular diseases and has a comprehensive preclinical research effort targeting fibrotic diseases and other serious conditions.

For more information, please visit www.acceleronpharma.com/. Follow Acceleron on Social Media: [@AcceleronPharma](#) and [LinkedIn](#).

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. Neither Celgene nor Acceleron undertake any obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond each company's control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, many of which are discussed in more detail in the Annual Report on Form 10-K and other reports of each company filed with the Securities and Exchange Commission.

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Source: Acceleron Pharma and Celgene Corporation

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