



Acceleron Announces Luspatercept Phase 3 MEDALIST and BELIEVE Presentations Selected for “Best of ASH” by the American Society of Hematology

December 5, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 5, 2018-- Acceleron Pharma Inc. (Nasdaq:XLRN) today announced that the presentations of the MEDALIST and BELIEVE Phase 3 trial results of luspatercept in patients with lower-risk myelodysplastic syndromes (MDS) and beta-thalassemia associated anemias, respectively, were selected for “Best of ASH” by the American Society of Hematology at its 60th Annual Meeting & Exposition in San Diego. Luspatercept is an investigational therapy that is part of a global collaboration between Acceleron and Celgene.

The Society describes its “Best of ASH” selections, chosen from among the thousands of meeting abstracts, as “the biggest breakthroughs from the meeting’s scientific presentations.”

“Having both the MEDALIST and BELIEVE trials included in the Best of ASH session at the close of the annual meeting is extraordinarily gratifying,” said Habib Dable, President and Chief Executive Officer of Acceleron. “Throughout its development, luspatercept has demonstrated promising results in patients with these two distinct diseases associated with anemia. Yesterday’s event is a clear indication that the global hematology community recognizes the potential clinical impact of luspatercept.”

The full titles of the trials highlighted during the Best of ASH session are as follows:

‘MEDALIST’ trial presentation

Title: The MEDALIST Trial: Results of a Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Luspatercept to Treat Anemia in Patients with Very Low-, Low-, or Intermediate-Risk Myelodysplastic Syndromes (MDS) with Ring Sideroblasts (RS) who Require Red Blood Cell (RBC) Transfusions (Plenary Scientific Session, Abstract #1)

‘BELIEVE’ trial presentation

Title: The BELIEVE Trial: Results of a Phase 3, Randomized, Double-Blind, Placebo-Controlled, Study of Luspatercept in Adults Who Require Regular Red Blood Cell (RBC) Transfusions Due to β -Thalassemia (Abstract #163)

Luspatercept is an investigational therapy that is not approved for any use in any country. Celgene and Acceleron are planning regulatory application submissions of luspatercept in the United States and Europe in the first half of 2019.

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. In addition to the Phase 3 MEDALIST and BELIEVE studies reported at ASH, luspatercept is being evaluated in multiple other clinical trials. The Phase 3 COMMANDS trial was recently initiated in first-line, lower-risk, MDS patient population. 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 MDS

Patients with lower-risk MDS suffer from insufficient production of red blood cells, resulting in chronic anemia that can lead to debilitating fatigue, diminished quality of life and increased mortality. Many patients with lower-risk MDS-related chronic anemia require frequent red blood cell transfusions.

About Beta-Thalassemia

Beta-thalassemia is caused by a genetic defect in the production of hemoglobin, a protein that carries oxygen to red blood cells throughout the body. Patients suffer from severe, chronic anemia and often experience fatigue, organ enlargement, and bone complications. Patients require lifelong therapy that includes frequent red blood cell transfusions and treatment of the consequent iron overload.

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 Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit 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 about Acceleron's strategy, future plans and prospects, including statements regarding the development of Acceleron's compounds, the timeline for clinical development and regulatory approval of Acceleron's compounds and the expected timing for reporting of data from ongoing clinical trials. The words "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "may," "plan," "potential," "project," "should," "target," "will," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Actual results could differ materially from those included in the forward-looking statements due to various risks and uncertainties, including, but not limited to, that preclinical testing of Acceleron's compounds and data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, that the development of Acceleron's compounds will take longer and/or cost more than planned, that Acceleron or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of Acceleron's compounds, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, and that Acceleron's compounds will not receive regulatory approval or become commercially successful products. These and other risks and uncertainties are identified under the heading "Risk Factors" included in Acceleron's most recent Annual Report on Form 10-K, and other filings that Acceleron has made and may make with the SEC in the future.

The forward-looking statements contained in this press release are based on management's current views, plans, estimates, assumptions and projections with respect to future events, and Acceleron does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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