



## **Acceleron Announces that Phase 3 Results from the MEDALIST and BELIEVE Trials of Luspatercept will be Presented at the 60th American Society of Hematology Annual Meeting**

November 1, 2018

– Phase 3 MEDALIST trial results to be presented in Plenary Scientific Session –

– Phase 3 BELIEVE trial results to be shared in oral presentation –

– Acceleron to hold conference call and webcast live from ASH, December 3, at 9:00 a.m. EST –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 1, 2018-- Acceleron Pharma Inc. (Nasdaq:XLRN) today announced that results from the MEDALIST and BELIEVE Phase 3 trials of luspatercept in patients with low-to-intermediate risk myelodysplastic syndromes (MDS) and transfusion-dependent beta-thalassemia, respectively, will be presented at the 60<sup>th</sup> American Society of Hematology (ASH) Annual Meeting & Exposition in San Diego, California, on December 1-4, 2018. Luspatercept is being developed as part of a global collaboration between Acceleron and Celgene.

The results from the MEDALIST trial will be presented on Sunday, December 2<sup>nd</sup> during the Plenary Scientific Session, which honors the top six research papers submitted for presentation at the meeting. The ASH Program Committee selected the MEDALIST abstract for inclusion in the Plenary Session from more than 6,500 abstracts submitted this year. The BELIEVE results will be communicated in an oral presentation on Saturday, December 1<sup>st</sup>.

"There is no better forum in which to present results than at ASH, the world's largest hematology meeting. The acceptance of our abstracts for presentation in plenary and oral sessions validates our belief that luspatercept could become a truly transformative therapy for patients with MDS and beta-thalassemia," said Habib Dable, President and Chief Executive Officer of Acceleron. "We also have multiple ongoing trials in other MDS and beta-thalassemia populations as well as in myelofibrosis—reflecting our confidence in the potential for luspatercept to become a platform medicine for the management of a range of chronic anemias."

"We're thrilled by the prospect of altering the treatment landscapes of MDS and beta-thalassemia with the first novel anemia agent in more than two decades," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "The positive results in pivotal trials for two distinct diseases speak to the promise of luspatercept's unique mechanism of action as an erythroid maturation agent and the potential for its broader applicability to anemias with high unmet medical need. We continue to work diligently with our global collaboration partner, Celgene, to submit global regulatory applications beginning in the first half of 2019."

'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**

Session: Plenary Scientific Session

Date: Sunday, December 2<sup>nd</sup>

Time: 2:00 - 4:00 p.m. PST (San Diego Convention Center, Hall AB)

'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  $\beta$ -Thalassemia (Abstract # 163)**

Session: Thalassemia Globin Gene Regulation: Clinical

Date: Saturday, December 1<sup>st</sup>

Time: 2:00 - 3:30 p.m. PST (San Diego Convention Center, Room 30D)

The MEDALIST and BELIEVE Phase 3 trial abstracts include primary and secondary endpoint response rates and select baseline patient characteristics. A complete listing of abstracts can be found on the ASH Annual Meeting website (<http://www.hematology.org/Annual-Meeting/Abstracts/>). The luspatercept ASH presentations, which will include additional information beyond the abstracts, will be available in the "Science" section on Acceleron's website, [www.acceleronpharma.com](http://www.acceleronpharma.com), immediately following their presentations at the conference.

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

#### **Conference Call and Webcast**

Acceleron will host a webcast and conference call to discuss highlights from its presentations at ASH on December 3, 2018, at 9:00 a.m. EST.

The webcast will be accessible under "Events & Presentations" in the Investors/Media page of the Company's website at [www.acceleronpharma.com](http://www.acceleronpharma.com). Individuals can participate in the conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and referring to the "Acceleron ASH 2018 Conference Call."

The archived webcast will be available for replay on the Acceleron website approximately two hours after the event.

#### **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](http://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. Because lower-risk MDS-related chronic anemia often fails to respond to unapproved therapies which include erythropoiesis-stimulating agents, many patients 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](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 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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