Acceleron Announces Updated Results from Ongoing Phase 2 Trials of Luspatercept in Beta-Thalassemia at the 23rd Congress of the European Hematology Association

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- Clinically meaningful increases in hemoglobin and reductions in red blood cell transfusion burden observed through 36 months –

- Top-line results from the BELIEVE Phase 3 trial are on track for the middle of 2018 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 15, 2018-- Acceleron Pharma Inc. (Nasdaq:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta therapeutics to treat serious and rare diseases, today announced updated results from the ongoing Phase 2 trials of luspatercept in patients with beta-thalassemia during an oral presentation at the 23rdCongress of the European Hematology Association (EHA) in Stockholm, Sweden. Luspatercept is being developed as part of a global collaboration between Acceleron and Celgene.

“These results reinforce our enthusiasm for luspatercept’s potential as a safe, efficacious therapy for beta-thalassemia patients over the long term,” said Habib Dable, President and Chief Executive Officer of Acceleron.

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 corresponding treatment for the iron overload that results.

“We now have patients with both non-transfusion- and transfusion-dependent beta-thalassemia continuing on treatment for three years,” Dable continued. “We look forward to sharing top-line results from the BELIEVE Phase 3 trial over the next few months.”

Phase 2 Results

A total of 31 non-transfusion-dependent patients have been treated with luspatercept (dose levels ≥ 0.6 mg/kg) in the trials.

- 53% (16 of 30) achieved a ≥ 1 gram per deciliter increase in hemoglobin in the fixed 12-week intervals including weeks 13 to 24 and weeks 37 to 48 when compared to baseline.
- At week 48, the mean improvement in 6-minute walk distance was 18.6% compared to baseline (n=9).
- Multiple patients remain on treatment through 36 months, and continue to sustain clinically meaningful increases in hemoglobin.

A total of 32 transfusion-dependent patients have been treated with luspatercept (dose levels ≥ 0.6 mg/kg) in the trials.

- 41% (12 of 29) achieved a reduction in RBC transfusion burden of at least 33% in the fixed 12-week intervals including weeks 13 to 24 and weeks 37 to 48 when compared to baseline.

Phase 2 Safety Summary

The majority of adverse events (AEs) were Grade 1 or 2. Grade 3 AEs possibly or probably related to study drug were bone pain (n=3 patients), asthenia (n=2 patients), bone infarction (n=1 patient), headache (n=1 patient), and presyncope (n=1 patient). One serious AE of biliary colic was reported as possibly related to study drug.

The EHA beta-thalassemia presentation will be available immediately following the presentation on Saturday, June 16th at the conference under the Science page of the Company’s website at www.acceleronpharma.com/.

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

About the Phase 2 Trial

Data from two Phase 2 trials were presented at the 23rd Congress of the EHA: the base study in which beta-thalassemia patients received treatment with luspatercept for three months and the ongoing long-term safety extension study in which patients may receive treatment with luspatercept for up to an additional five years.

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. Phase 3 clinical trials are underway 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 3 trial is being planned in first-line, lower-risk, MDS patients (the COMMANDS trial). 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 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. Follow Acceleron on Social Media: @AcceleronPharma and LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements about the Company's strategy, future plans and prospects, including statements regarding the development of the Company's compounds, the timeline for clinical development and regulatory approval of the Company'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 the Company'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 the Company's compounds will take longer and/or cost more than planned, that the Company or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of the Company's compounds, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, and that the Company'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 the Company's most recent Annual Report on Form 10-K, and other filings that the Company 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 the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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