Acceleron Announces Updated Results from Ongoing Phase 2 Trials of Luspatercept in Myelodysplastic Syndromes at the ASCO 2018 Annual Meeting

June 4, 2018

– Multiple patients continue on treatment with clinically meaningful increases in hemoglobin and reduction in red blood cell transfusions surpassing 3 years –

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

– COMMANDS Phase 3 trial in first-line, lower-risk MDS to be initiated in Q3 2018 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 4, 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 Phase 2 trials of luspatercept in patients with lower-risk myelodysplastic syndromes (MDS) at the American Society of Clinical Oncology (ASCO) 2018 Annual Meeting in Chicago. Luspatercept is being developed as part of a global collaboration between Acceleron and Celgene.

“The ongoing Phase 2 trials continue to provide important insights into luspatercept’s potential to deliver long-term benefit to thousands of patients with lower-risk MDS,” said Habib Dable, President and Chief Executive Officer of Acceleron. “With multiple patients on treatment for more than three years, we are increasingly confident in luspatercept’s novel mechanism as an erythroid maturation agent to address a significant unmet medical need in lower-risk MDS. We look forward to sharing top-line results from the MEDALIST Phase 3 trial over the next few months.”

Patients with 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 MDS-related chronic anemia often fails to respond to unapproved therapies which include erythropoiesis-stimulating agents, many patients require frequent red blood cell transfusions.

Phase 2 Results

A total of 101 patients with lower-risk MDS have been treated with luspatercept (dose levels ? 0.75 mg/kg) in the Phase 2 trials.

- 55% (55 of 101 patients) achieved a clinically meaningful erythroid improvement (IWG HI-E criteria).
- 44% (30 of 68 patients) with a red blood cell (RBC) transfusion burden at baseline achieved RBC transfusion independence (RBC-TI) for at least 8 weeks.
    - The mean duration of treatment for RBC-TI responders was 18.3 months (n=30, ongoing).
- Multiple patients continue on treatment through 40 months, and continue to sustain a clinically meaningful increase in hemoglobin and reduction in transfusion burden.

Phase 2 Safety Summary

The majority of adverse events (AEs) were Grade 1 or 2. Grade 3 non-serious AEs possibly related to study drug were ascites, blood bilirubin increase, bone pain, hypertension, mucosal inflammation, platelet count increase, and transformation to AML (previously reported as a blast cell count increase). The Grade 3 non-serious AEs occurred in one patient each, with the exception of hypertension in 2 patients.

Serious AEs (SAEs) possibly related to study drug were general physical health deterioration, muscular weakness, musculoskeletal pain, and myalgia. The four SAEs occurred in three individual patients.

The ASCO MDS poster presentation is available 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 Ongoing MDS Phase 2 Trials

Data from two Phase 2 trials were presented at the 2018 ASCO Annual Meeting: the base study in which patients with lower-risk MDS received treatment with luspatercept for three months and the long-term extension study in which patients who completed the base study 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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