Acceleron Announces Publication of Luspatercept Phase 2 Beta-Thalassemia Study Results in Blood

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 10, 2019-- 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 that the journal *Blood* has published results from the Phase 2 study of luspatercept in patients with red blood cell (RBC) transfusion-dependent and non-transfusion-dependent beta-thalassemia. Luspatercept is an investigational therapy that is part of a global collaboration between Acceleron and Celgene.

In this open-label, Phase 2 study (a three-month initial stage followed by a five-year extension stage), patients were treated with luspatercept subcutaneously once every three weeks. The primary outcome measures were a reduction in RBC transfusion burden—compared with pretreatment—in transfusion-dependent patients, and changes in hemoglobin levels from baseline in non-transfusion-dependent patients. The extension study is ongoing and evaluates the long-term safety and tolerability of luspatercept for up to 5 years.

“Current treatment options for patients with beta-thalassemia are essentially limited to supportive therapy, including red blood cell transfusions, which leads to iron overload,” said Antonio Piga, M.D., Professor, Department of Clinical and Biological Sciences at Turin University and lead author of the newly published study. “Luspatercept has demonstrated an ability to improve hemoglobin levels and reduce transfusion burden through apparent restoration of the late-stage red blood cell maturation process, which is known to be inhibited in these patients. Based on these results and those of subsequent studies, luspatercept has the potential to address significant unmet medical needs for patients suffering from beta-thalassemia.”

The article, entitled “Luspatercept improves hemoglobin levels and blood transfusion requirements in a study of patients with beta-thalassemia,” is now available online and will be published in a future print edition of *Blood*.

“We’re pleased that these important results are now fully available to the greater hematology community,” said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. “This study provided key information on the efficacy and safety profile of luspatercept and informed the design of the Phase 3 trial, known as BELIEVE, for which positive results in transfusion-dependent patients with beta-thalassemia were presented last month at the American Society of Hematology (ASH) Annual Meeting and were included in the ‘Best of ASH’ session at that meeting.”

Luspatercept is being evaluated in ongoing studies in patients with non-transfusion-dependent beta-thalassemia (the Phase 2 BEYOND trial) as well as in patients with lower-risk myelodysplastic syndromes (MDS) (the Phase 2 PACE-MDS trial and the Phase 3 MEDALIST and COMMANDS trials) and in a Phase 2 trial in patients with myelofibrosis.

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

About Luspatercept

Luspatercept is a first-in-class erythroid maturation agent that is believed to regulate late-stage red blood cell maturation. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Phase 3 clinical trials continue 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 COMMANDS Phase 3 trial in first-line, lower-risk, MDS patients, 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 phase 2 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 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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