Acceleron Completes Target Enrollment in the PULSAR Phase 2 Trial of Sotatercept in Pulmonary Arterial Hypertension

June 26, 2019

- Company now expects to report top-line results in the first quarter of 2020 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 26, 2019-- Acceleron Pharma Inc. (Nasdaq:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases, announced today it has completed target enrollment of patients with pulmonary arterial hypertension (PAH) in the PULSAR Phase 2 trial of sotatercept. The Company now expects to report top-line results during the first quarter of 2020.

“We’re thrilled with PULSAR’s rapid enrollment over the past 12 months, which underscores the urgency for new therapeutic options for patients with PAH,” said Janethe de Oliveira Pena, MD, PhD, Vice President, Pulmonary Medical Research at Acceleron. “We believe that through its targeting of underlying disease mechanisms, sotatercept, when combined with standard-of-care therapies, has the potential to alter the PAH treatment landscape.”

The PULSAR trial has enrolled 100 patients with PAH. Additional patients who are currently in screening remain eligible for randomization into the trial over the next few weeks. Acceleron is also enrolling patients with PAH in the Phase 2 SPECTRA exploratory trial of sotatercept.

Sotatercept is an investigational therapy that is not approved for any use in any country.

About the PULSAR Trial

The PULSAR Phase 2 trial is a randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of sotatercept in PAH patients. The primary endpoint of the trial is the change from baseline in pulmonary vascular resistance (PVR) over a 24-week treatment period. The key secondary endpoint is change from baseline in six-minute walk distance (6MWD). A total of at least 100 patients will be randomized in a 3:3:4 ratio to receive placebo, sotatercept 0.3 mg/kg, or sotatercept 0.7 mg/kg subcutaneously every 21 days with standard-of-care therapies in combination. Following the 6-month double-blind treatment period, participants in the trial will be eligible to continue in the 18-month extension period.

About Sotatercept

Sotatercept is an investigational agent designed to be a selective ligand trap for members of the TGF-beta superfamily to rebalance BMPR2 signaling, which is a key molecular driver of PAH. In preclinical studies of PAH, sotatercept reversed pulmonary vessel muscularization and improved indicators of right heart failure. Sotatercept is currently being evaluated in the PULSAR and SPECTRA Phase 2 trials in PAH. For more information, please visit www.clinicaltrials.gov

About PAH

PAH is a rare and chronic, rapidly progressing disorder characterized by the constriction of small pulmonary arteries and elevated blood pressure in the pulmonary circulation. PAH results in significant strain on the heart, often leading to limited physical activity, heart failure, and reduced life expectancy. The 5-year survival rate for patients with PAH is approximately 57%. Available therapies generally act by promoting the dilation of pulmonary vessels without addressing the underlying cause of the disease. As a result, PAH often progresses rapidly for many patients despite standard of care treatment. A growing body of research has implicated imbalances in BMP and TGF-beta signaling as a primary driver of PAH in familial, idiopathic, and acquired forms of the disease.

About Acceleron

Acceleron is a 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 superfamily 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 program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease and is conducting 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 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 factors, 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 results of any clinical trial may not be predictive of the results or success of other clinical trials of the same product candidate, that the development of the Company's compounds will take longer and/or cost more than planned, that the Company will be unable to successfully complete the clinical development of the Company's compounds, that the Company 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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