Acceleron Announces Initiation of PULSAR Phase 2 Trial of Sotatercept in Pulmonary Arterial Hypertension

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PULSAR trial expands Acceleron's pipeline into pulmonary disease

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 22, 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 the initiation of the PULSAR Phase 2 trial of sotatercept for the treatment of patients with pulmonary arterial hypertension (PAH), a chronic and life-changing disease that can lead to heart failure.

"There is a real need for new treatment options that have the potential to improve survival for PAH patients," said David Badesch, M.D., the Director of the Pulmonary Hypertension Program at the University of Colorado and Chair of the PULSAR Trial Steering Committee. "Based on its novel mechanism and preclinical results, sotatercept, particularly in combination with standard-of-care therapies, could be an important advancement in the future treatment of PAH. I look forward to the results of the trial."

"The initiation of the PULSAR trial is an important milestone in our newly expanded research and development efforts in pulmonary disease," said Habib Dable, Chief Executive Officer of Acceleron. "We believe that treatment with sotatercept could restore a vital signaling pathway that is known to be deficient in PAH patients. This is an extremely important trial, as we hope to bring an innovative medicine to the thousands of patients who are suffering from PAH."

The Company plans to report preliminary results from the 6-month primary treatment period of the PULSAR Phase 2 trial in the first half of 2020.

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 90 patients will be randomized 1:1:1 into three treatment arms with standard-of-care vasodilator therapies in combination with sotatercept or placebo. Following the 6-month double-blind treatment period, participants in the trial will be eligible to enroll into the 18-month extension period.

For additional information about this clinical trial, please visit clinicaltrials.gov, identifier NCT03496207.

About Sotatercept

Sotatercept acts as a ligand trap for members of the transforming growth factor-beta superfamily, including those directly involved in the BMP pathway proven critical for maintaining healthy pulmonary vasculature. In multiple preclinical studies in PAH, sotatercept significantly decreased pulmonary vessel muscularization, improved pulmonary arterial pressures, and decreased indicators of right heart failure. Sotatercept is currently being evaluated in the PULSAR Phase 2 trial in PAH.

About Pulmonary Arterial Hypertension (PAH)

Pulmonary Arterial Hypertension (PAH) is a rare, chronic, and rapidly progressing disorder characterized by the narrowing of small pulmonary arteries and elevated blood pressure in the pulmonary circulation. PAH results in significant and progressive strain on the right side of 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 primarily 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 all forms of the disease.

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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