Acceleron Receives FDA Orphan Drug Designation for Sotatercept in Pulmonary Arterial Hypertension

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 9, 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, today announced that the United States Food and Drug Administration (FDA) has granted Orphan Drug designation to sotatercept for the treatment of patients with pulmonary arterial hypertension (PAH).

“We’re pleased that the FDA has granted this designation for sotatercept,” said Janethe de Oliveira Pena, MD, PhD, Vice President, Pulmonary Medical Research at Acceleron. “In preclinical studies, sotatercept has demonstrated an ability to target the underlying mechanisms of PAH, which is a rare disease of high unmet medical need. We believe that if similar effects are seen in a clinical setting, sotatercept has the potential to become an important addition to the standard of care in PAH.”

Orphan designation is granted by the FDA Office of Orphan Products Development to advance the evaluation and development of safe and effective therapies for the treatment of rare diseases or conditions affecting fewer than 200,000 people in the U.S. Under the Orphan Drug Act, the FDA may provide grant funding toward clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States following marketing approval by the FDA. The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. For more information about orphan designation, please visit the FDA website at www.fda.gov.

Sotatercept is being evaluated in two Phase 2 trials in patients with PAH: the PULSAR trial, which completed its target enrollment in June of this year and the SPECTRA exploratory trial, which is currently enrolling. The Company expects to report top-line results from the PULSAR trial during the first quarter of 2020.

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

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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