November 14, 2017

# Acceleron Announces Preclinical Results in Pulmonary Arterial Hypertension at the American Heart Association 2017 Scientific Sessions

*- Preclinical results show potential first-in-class disease-modifying properties of sotatercept in pulmonary arterial hypertension -*

*- Company expects to initiate a Phase 2 trial in 1H 2018 -*

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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 new preclinical results with sotatercept in pulmonary arterial hypertension (PAH) during an oral presentation at the American Heart Association (AHA) 2017 Scientific Sessions in Anaheim, California.

"We are extremely encouraged by these positive preclinical results, which support our mechanistic approach for sotatercept and its potential to be a first-in-class disease-modifying therapy for patients with pulmonary arterial hypertension. We look forward to initiating a Phase 2 trial with sotatercept in PAH during the first half of 2018," said Ravi Kumar, Chief Scientific Officer for Acceleron.

The oral presentation by Paul Yu, M.D., Ph.D., Associate Professor of Medicine, Brigham and Women's Hospital, entitled "ACTRIIA-Fc rebalances BMP and activin/TGFβ signaling to attenuate experimental pulmonary hypertension," provided data from three animal models of PAH demonstrating potent effects of sotatercept on many aspects of vascular and cardiac disease.

- In animals treated early in disease progression, sotatercept blocked the development of pulmonary vascular remodeling and right heart failure, and prevented the increase in pulmonary arterial pressure.
- For animals treated after established disease, sotatercept achieved a marked reduction in intimal-medial thickness of pulmonary arterioles relative to untreated animals, decreased vessel muscularization, improved pulmonary arterial pressures and decreased indicators of right heart failure.

"The sotatercept preclinical results show remarkable biological activity, particularly on the pulmonary vasculature, across multiple, well-established animal models of PAH," said Dr. Yu. "These results are very exciting, and further establish the importance of signaling by the TGF-beta superfamily as a target for therapies to treat PAH."

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

The AHA preclinical presentation is available under the Science page of the Company's website at www.acceleronpharma.com.

**About Sotatercept**

Sotatercept is an activin receptor type IIA fusion protein that acts as a ligand trap for members in the transforming growth factor-beta superfamily involved in remodeling and regeneration of a variety of different tissues, including the vasculature and fibrosis.

**About Pulmonary Arterial Hypertension**

Pulmonary Arterial Hypertension (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 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 a Phase 2 trial of sotatercept planned 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.

View source version on businesswire.com: http://www.businesswire.com/news/home/20171114006527/en/

Acceleron Pharma Inc.
Todd James, IRC, 617-649-9393
Vice President, Investor Relations and Corporate Communications
Or
Candice Ellis, 617-649-9226
Manager, Investor Relations and Corporate Communications
Or
Media:
BMC Communications
Brad Miles, 646-513-3125

Source: Acceleron Pharma