Acceleron Announces Clinical Trial Updates, Preclinical Presentations on Sotatercept in Pulmonary Arterial Hypertension (PAH) at the American Thoracic Society (ATS) 2021 International Conference

4/5/2021

– Conference to include interim results from the open-label extension of the PULSAR Phase 2 trial showing consistent or improved responses in efficacy endpoints among sotatercept-treated patients –

– Interim results from the SPECTRA Phase 2 trial to show improvements in measures of exercise hemodynamics –

– Sotatercept was generally well tolerated in both trials, consistent with the previously reported safety profile in PAH and in other diseases –

– Acceleron to host investor and analyst conference call and webcast on Wednesday, May 19 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (Nasdaq: XLRN), a leading biopharmaceutical company in the discovery, development, and commercialization of TGF-beta superfamily therapeutics to treat serious and rare diseases, today announced that updates from the PULSAR and SPECTRA Phase 2 clinical trials of sotatercept in patients with pulmonary arterial hypertension (PAH) will be presented at the American Thoracic Society 2021 International Conference (ATS 2021), held virtually May 14-19. The Company will also present preclinical research on the effects of a murine version of sotatercept in animal models of PAH and pulmonary hypertension.

ATS 2021 will include interim results from the open-label extension of the PULSAR Phase 2 trial through 48 weeks. The presentation will highlight changes from baseline in a range of endpoints, including six-minute walk distance and World Health Organization (WHO) functional class. The conference will also feature interim data from the SPECTRA Phase 2 trial assessing the effect of sotatercept on peak oxygen uptake and exercise hemodynamics, as measured by invasive cardiopulmonary exercise testing.
“It’s quite gratifying to return to the ATS International Conference with new sotatercept data, having first presented topline results from the PULSAR trial during a special breaking news session at ATS 2020 Virtual,” said Habib Dable, President and Chief Executive Officer of Acceleron. “The results to be shared this year strengthen our confidence in sotatercept’s potential to become a backbone therapy for patients with PAH.”

Clinical Presentations

Session: Come Together – Clinical Advances in Pulmonary Hypertension: Lesson from Best Abstracts

Date: Wed, May 19, 8:00 - 9:30 a.m. EDT

Title: PULSAR Study Open-Label Extension: Interim Results from a Phase 2 Study of the Efficacy and Safety of Sotatercept When Added to Standard of Care for the Treatment of Pulmonary Arterial Hypertension (PAH)

Title: The SPECTRA Study: A Phase 2a Single-Arm, Open-Label, Multicenter Exploratory Study to Assess the Effects of Sotatercept for the Treatment of Pulmonary Arterial Hypertension (PAH)

The PULSAR and SPECTRA presentations will include additional data and analyses not disclosed in the abstracts.

Preclinical ePoster Presentations

Session: A Hard Day’s Night – Novel Molecular Mechanisms and Treatment Options in PAH and Beyond: From Pulmonary Vasculature to RV

Title: Sotatercept Analog RAP-011 Alleviates Cardiopulmonary Remodeling and Inflammation in a Model of Heritable PAH Arising from Bmpr2 Haploinsufficiency

Title: Sotatercept Analog RAP-011 Reduces Right Ventricular Hypertrophy and Alleviates Pulmonary Hypertension in A ZSF1 Rat Model of Heart Failure with Preserved Ejection Fraction

All of the abstracts above are currently viewable on the ATS website.

The preclinical presentations will be posted to the “Publications” page under the “Science & Pipeline” section on Acceleron’s website, www.acceleronpharma.com, beginning Friday, May 14, with the clinical presentations being added to the Acceleron website on Wednesday, May 19 at 9:30 a.m. EDT.

Webcast and Conference Call Information
The Company will host a webcast and conference call to review the presentations of sotatercept at ATS 2021 on Wednesday, May 19, 2021.

The webcast will be accessible under “Events & Presentations” on the Investors & Media page of the Company’s website at [www.acceleronpharma.com](http://www.acceleronpharma.com). Additional details, including specific timing and call-in information will be announced prior to the presentations at ATS 2021.

**About Sotatercept**

Sotatercept is an investigational reverse-remodeling agent designed to be a selective ligand trap for members of the TGF-beta superfamily to rebalance BMPR-II signaling, which is a key molecular driver of PAH. The PULSAR Phase 2 trial evaluating sotatercept in combination with approved PAH-specific medicines in patients with PAH achieved its primary endpoint of improvement in pulmonary vascular resistance and its key secondary endpoint of improvement in 6-minute walk distance. Sotatercept was generally well tolerated in the trial. Adverse events observed in the study were generally consistent with previously published data on sotatercept in other diseases. Following the PULSAR results, sotatercept was granted Breakthrough Therapy designation from the FDA and Priority Medicines designation from the EMA in PAH. Sotatercept is also being evaluated in the SPECTRA Phase 2 exploratory trial.

The Company recently presented details of its Phase 3 development plan, including the design for the registrational STELLAR trial, which is currently enrolling patients with PAH. Acceleron is planning two additional Phase 3 studies in patients with PAH: the HYPERION trial in newly diagnosed patients and the ZENITH trial assessing intervention in patients diagnosed with World Health Organization (WHO) functional class IV disease.

Sotatercept is an investigational therapy that is not approved for any use in any country. Sotatercept is part of a licensing agreement with Bristol Myers Squibb.

**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 biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases. Acceleron'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, development, and commercialization efforts in pulmonary and hematologic diseases. In pulmonary, Acceleron is developing sotatercept for the treatment of pulmonary arterial hypertension (PAH), having reported positive topline results of the PULSAR Phase 2 trial. The Company is currently planning multiple Phase 3 trials with the potential to support its long-term vision of establishing sotatercept as a backbone therapy for patients with PAH at all stages of the disease. Acceleron is also investigating the potential of its early-stage pulmonary candidate, ACE-1334, which it plans to advance into a Phase 1b/Phase 2 trial in systemic sclerosis-associated interstitial lung disease (SSc-ILD) this year.

In hematology, REBLOZYL® (luspatercept-aamt) is the first and only erythroid maturation agent approved in the United States, Europe, and Canada for the treatment of anemia in certain blood disorders. REBLOZYL is part of a global collaboration partnership with Bristol Myers Squibb. The Companies co-promote REBLOZYL in the United States and are also developing luspatercept for the treatment of anemia in patient populations of myelodysplastic syndromes, beta-thalassemia, and myelofibrosis.

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 sotatercept in PAH, the timeline for clinical development and regulatory approval of sotatercept in PAH, the expected timing for reporting of data from ongoing clinical trials, and the potential of Acceleron's compounds as therapeutic drugs. The words "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "may," "plan," "possible," "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 Acceleron's compounds and data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, that regulatory approval of Acceleron's compounds in one indication or country may not be predictive of approval in another indication or country, that the development of Acceleron's compounds will take longer and/or cost more than planned, that Acceleron will be unable to successfully complete the clinical development of Acceleron's compounds, that Acceleron may be delayed in initiating, enrolling or completing any clinical trials, that Acceleron's compounds...
will not receive regulatory approval or become commercially successful products, and that Breakthrough Therapy or PRIME designation may not expedite the development or review of sotatercept. 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210405005179/en/

Investors:
Jamie Bernard, IRC, 617-301-9650
Associate Director, Investor Relations

Media:
Matt Fearer, 617-301-9557
Senior Director, Corporate Communications

Source: Acceleron Pharma Inc.