NEWS RELEASE

Acceleron Receives Orphan Designation from the European Commission (EC) for Sotatercept in Pulmonary Arterial Hypertension (PAH)

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– Sotatercept received Orphan Drug designation from the U.S. Food and Drug Administration (FDA) in PAH in 2019 –

– In the spring of 2020, sotatercept received Breakthrough Therapy designation from the FDA and Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), both in PAH –

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (Nasdaq: XLRN), a biopharmaceutical company dedicated to the discovery, development, and commercialization of TGF-beta superfamily therapeutics to treat serious and rare diseases, today announced that the European Commission (EC) has granted orphan designation to sotatercept for the treatment of patients with pulmonary arterial hypertension (PAH).

“We’re thrilled at the European Commission’s decision to grant orphan designation to sotatercept in PAH,” said Habib Dable, President and Chief Executive Officer of Acceleron. “We fully intend to take advantage of the benefits that this and other special statuses—including Orphan Drug and Breakthrough Therapy designations in the United States and PRIME designation in Europe—provide to drug developers as we work with health authorities to deliver this potential new backbone therapy in PAH to patients in need as quickly as possible.”

The EC grants orphan designation to medicines intended to treat, prevent or diagnose a disease of low prevalence (fewer than 5 individuals per 10,000 population) that is life-threatening or chronically debilitating. To encourage the development of such medicines, the designation carries with it certain incentives, including scientific advice and assistance with clinical trial protocols, and the potential for a 10-year period of market exclusivity. For additional information about orphan designation, visit the **EMA website**.

Acceleron is currently advancing a Phase 3 development plan for sotatercept, beginning with the registrational trial known as STELLAR expected to initiate by the end of this year.

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 expected to be initiated before the end of 2020. Acceleron is planning two additional Phase 3 studies in patients with PAH: the HYPERION trial, exploring early intervention with sotatercept, and the ZENITH trial assessing later-stage intervention.

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/2 trial in systemic sclerosis-associated interstitial lung disease (SSc-ILD) next 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, Quarterly Report on Form 10-Q, 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.

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