September 19, 2017

Acceleron to Develop Sotatercept in Pulmonary Arterial Hypertension

- Acceleron gains rights to fund, develop, and lead global commercialization of sotatercept in pulmonary arterial hypertension -

- Acceleron expects to initiate a Phase 2 trial of sotatercept in pulmonary arterial hypertension in 1H 2018 -

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

- Sotatercept is a well-defined TGF-beta superfamily ligand trap with a widely established preclinical and clinical profile -

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 it has amended the sotatercept development and commercialization collaboration agreement with Celgene Corporation, originally executed on February 20, 2008. The amended agreement provides Acceleron with global access to sotatercept for development and commercialization in pulmonary arterial hypertension (PAH).

"We are very pleased to announce the execution of this amended agreement alongside our long-time collaboration partner, Celgene. Sotatercept shows tremendous potential to be a groundbreaking therapy for pulmonary arterial hypertension, and we expect sotatercept, as a Phase 2-ready compound, to be the lead molecule in our new pulmonary disease franchise," said Habib Dable, Chief Executive Officer for Acceleron. "Our mission with sotatercept, as we move to initiate a Phase 2 trial in the first half of 2018, is to improve the lives of those living with pulmonary arterial hypertension."

"While many therapies are approved for the treatment of pulmonary arterial hypertension, these therapies all focus on a mechanism of vasodilation and the prognosis for patients remains poor," said Eric Austin, M.D., Director, Vanderbilt Pediatric Pulmonary Hypertension Program. "Sotatercept's mechanism is intended to rectify the deficits in molecular signaling that underlie both the familial and idiopathic forms of this disease. The preclinical data with sotatercept is very encouraging, and I look forward to seeing data from clinical trials."

"This amended agreement unites Acceleron's scientific leadership in the TGF-beta superfamily with Celgene's commitment to advance novel molecules, such as sotatercept, for patients in therapeutic areas of high unmet medical need," said Scott Smith, Chief Operating Officer for Celgene.

Under the amended and restated collaboration agreement, Acceleron has the right to fund and conduct all research and development activities for sotatercept in the pulmonary hypertension field. Should sotatercept be approved for an indication in the pulmonary hypertension field, Acceleron will be responsible for global commercialization and Celgene will be eligible to receive royalties on global net sales in that field. The original collaboration deal terms will remain in place with respect to development and commercialization outside of the pulmonary hypertension field.

Acceleron also updated its cash guidance, announcing that its existing cash, cash equivalents and investments will be sufficient to fund projected operating requirements into mid-2019, versus its earlier guidance of the second half of 2019.

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

Acceleron R&D Day Conference Call and Webcast Information

The live webcast will be accessible under "Events & Presentations" in the Investors/Media page of the Company's website at www.acceleronpharma.com.

Individuals can participate in the conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and refer to "Acceleron 2017 R&D Day."

The archived webcast will be available for replay on the Acceleron website approximately two hours after the event.

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 (TGF-β) protein 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 transporting high pressure blood from the heart to the lungs. The high or increased blood pressure causes 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%. There are currently four classes of therapies approved for treatment of PAH: endothelin receptor antagonists (ERAs), phosphodiesterase-5 (PDE-5) inhibitors, prostacyclin analogues and soluble guanylate cyclase (sGC) stimulators. The available therapies all act by promoting the dilation of pulmonary vessels, which have positive effects for patients, but do not impact 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-β signaling as a primary driver of PAH in familial, idiopathic and acquired forms of the disease.

About Acceleron

Acceleron is a biopharmaceutical leader in TGF-beta science, focused on the discovery and development of innovative therapeutics to treat patients with serious and rare diseases. Its pioneering research and protein engineering platform engages the target-rich TGF-beta superfamily and its remarkable ability to regulate cellular growth and repair.

Under a global partnership with Celgene, Acceleron is in Phase 3 development with luspatercept, a potential first-in-class chronic anemia therapy for the treatment of rare blood diseases. The Company is also advancing a 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. Acceleron has ongoing preclinical research efforts targeting additional indications in these three disease areas where there is significant unmet medical need.

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," "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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