Acceleron Receives FDA Fast Track Designation for ACE-083 in Charcot-Marie-Tooth Disease

November 28, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 28, 2018-- 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 that the United States Food and Drug Administration (FDA) has granted Fast Track designation to ACE-083, the Company's locally-acting "Myostatin+" muscle agent, for the treatment of patients with Charcot-Marie-Tooth disease (CMT).

"We're pleased that the FDA has granted this designation for ACE-083," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "Patients with CMT currently have no approved therapies. To date, results from our Phase 2 trials have shown that patients treated with ACE-083 experience robust increases in muscle volume. If our ongoing clinical studies show that ACE-083 also improves functional outcomes and confirm the favorable safety profile observed thus far, the Fast Track process could help us work with the FDA to deliver it to patients as quickly as possible."

The FDA's Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

ACE-083 is currently being evaluated in two Phase 2 trials: one in CMT and one in facioscapulohumeral muscular dystrophy (FSHD). Preliminary results from Part 2 of the trials are expected by year end 2019 for CMT and in the second half of 2019 for FSHD. Earlier this year, the FDA granted Fast Track and Orphan Drug designations for ACE-083 for the treatment of patients with FSHD.

About ACE-083

ACE-083, a locally-acting therapeutic candidate based on the naturally-occurring protein follistatin, utilizes the Myostatin+ approach to inhibit multiple TGF-beta superfamily ligands. It is designed to have a concentrated effect along targeted muscles to maximize growth and strength selectively in the muscles into which the drug is administered. Acceleron is developing ACE-083 for disorders such as CMT and FSHD, in which improved muscle strength in target muscles may provide a clinical benefit and enhance quality of life.

About CMT

CMT is the most common inherited neurologic disease, estimated to affect more than 125,000 people in the United States. The primary clinical manifestations of CMT include muscle weakness in the lower legs and hands. The lower leg muscle weakness can result in foot drop leading to frequent trips and falls. The disease is typically diagnosed by a characteristic pattern of muscle weakness and sensory deficits, family history, nerve conduction studies, and genetic testing. There are no FDA-approved therapies for CMT.

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