



Acceleron Receives FDA Fast Track Designation for ACE-083 in Facioscapulohumeral Muscular Dystrophy (FSHD)

May 1, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 1, 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 U.S. Food and Drug Administration (FDA) has granted Fast Track designation to ACE-083, a locally-acting "Myostatin+" muscle agent, for the treatment of patients with facioscapulohumeral muscular dystrophy (FSHD).

"This is an important milestone in the development of ACE-083—our lead program within our neuromuscular franchise," said Matthew Sherman, Chief Medical Officer of Acceleron. "FSHD is a serious and rare neuromuscular disorder for which there are currently no approved therapies available. With this designation, we will be able to expedite the FDA review process of ACE-083, and if successful, deliver the first locally-acting, 'Myostatin+' muscle agent as a meaningful treatment option for the thousands of patients impacted by FSHD."

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. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions. 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 FSHD and one in Charcot-Marie-Tooth (CMT) disease.

About ACE-083

ACE-083 is a locally-acting therapeutic candidate, based on the naturally-occurring protein follistatin, which utilizes the "Myostatin+" approach to inhibit multiple TGF-beta 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 diseases such as facioscapulohumeral muscular dystrophy (FSHD) and Charcot-Marie-Tooth (CMT) disease, in which improved muscle strength in target muscles may provide a clinical benefit and enhance quality of life.

About Facioscapulohumeral Muscular Dystrophy(FSHD)

FSHD is a rare genetic muscle disorder affecting approximately 20,000 people in the United States for which there are currently no approved treatments. The primary clinical presentation of FSHD is debilitating skeletal muscle weakness and loss. The symptoms of FSHD develop in a descending pattern, beginning with the face and upper body and progressing to the lower body in a "muscle by muscle" fashion. The disease is typically diagnosed by a characteristic pattern of muscle weakness and other clinical symptoms, as well as through genetic testing.

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](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleron-pharma).

Cautionary Note on 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.

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