



Acceleron Receives FDA Orphan Drug Designation for ACE-083 in Charcot-Marie-Tooth Disease

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 5, 2019-- Acceleron Pharma Inc. (NASDAQ:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases, today announced that the United States Food and Drug Administration (FDA) has granted Orphan Drug designation to ACE-083, the Company's locally-acting "Myostatin+" muscle agent, for the treatment of patients with Charcot-Marie-Tooth disease (CMT).

"This formal designation aligns with our mission to develop therapies for patients with rare diseases of high unmet medical need," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "In clinical trials to date, treatment with ACE-083 has resulted in substantial increases in muscle volume in target muscles. We believe that if our ongoing studies demonstrate that such increases lead to improved functional outcomes, ACE-083 has the potential to become an important new therapy for patients with CMT."

Orphan designation is granted by the FDA Office of Orphan Products Development to advance the evaluation and development of safe and effective therapies for the treatment of rare diseases or conditions affecting fewer than 200,000 people in the U.S. Under the Orphan Drug Act, the FDA may provide grant funding toward clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States following marketing approval by the FDA. The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. For more information about orphan designation, please visit the FDA website at www.fda.gov.

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. During 2018, the FDA granted Fast Track designation for ACE-083 in CMT as well as 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.

ACE-083 is an investigational therapy that is not approved for any use in any country.

About CMT

CMT is one of the most common inherited neurologic diseases, 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 regulatory authority-approved therapies for CMT.

About Acceleron

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

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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