Acceleron Receives FDA Orphan Drug Designation for ACE-083 in Facioscapulohumeral Muscular Dystrophy

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 12, 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 orphan drug designation for ACE-083, the Company's locally acting "Myostatin+" muscle agent, for the treatment of patients with facioscapulohumeral muscular dystrophy (FSHD).

"We are pleased to receive orphan drug designation for ACE-083, which has shown the potential to address an area of high unmet medical need," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "We believe that ACE-083 could become an important new treatment for patients with FSHD whose muscle weakness negatively affects their functional abilities. We presented positive preliminary data from Part 1 of our Phase 2 trial in patients with FSHD earlier this year and look forward to sharing Part 2 results later next year."

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.

In May of 2018, FDA granted ACE-083 Fast Track designation for FSHD, which could facilitate its development and potentially expedite its review. ACE-083 is currently being evaluated in two Phase 2 trials: one in FSHD and one in Charcot-Marie-Tooth (CMT) disease. The final Part 1 results from both Phase 2 trials are expected in the second of half of 2018. Preliminary results from Part 2 of the trial in patients with FSHD are expected in the second half of 2019.

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 disorders such as Charcot-Marie-Tooth (CMT) disease and facioscapulohumeral muscular dystrophy (FSHD), in which improved muscle strength in target muscles may provide a clinical benefit and enhance quality of life. For more information, please visit www.clinicaltrials.gov.

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 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 factors, 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 results of any clinical trial may not be predictive of the results or success of other clinical trials of the same product candidate, 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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