



Acceleron Announces Presentations on ACE-083 at the 23rd International Annual Congress of the World Muscle Society

September 27, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 27, 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 plans to deliver poster presentations on ACE-083 from Part 1 of each its ongoing Phase 2 trials in patients with facioscapulohumeral muscular dystrophy (FSHD) and Charcot-Marie-Tooth disease (CMT) at the 23rd International Annual Congress of the World Muscle Society to be held in Mendoza, Argentina, October 2-6, 2018.

Presentations

Title: Results for a dose-escalation phase 2 study to evaluate ACE-083, a local muscle therapeutic, in patients with facioscapulohumeral muscular dystrophy (Poster: P.365)

Session: Poster Session 4 - FSHD/OPMD/EDMD/DMI

Date: Friday, October 5, 2018

Time: 5:00 - 6:30 p.m. ART (Hotel Intercontinental Mendoza, Poster area)

Title: Preliminary phase 2 results for ACE-083, local muscle therapeutic, in patients with CMT1 and CMTX (Poster: P.339)

Session: Poster Session 4 - CMT and neurogenic disease

Date: Friday, October 5, 2018

Time: 5:00 - 6:30 p.m. ART (Hotel Intercontinental Mendoza, Poster area)

"As we've reported at neurology congresses throughout this year, patients with FSHD and CMT treated with ACE-083 in these studies have experienced robust mean increases exceeding 12% in total and contractile muscle volume," said Robert K. Zeldin, M.D., Chief Medical Officer of Acceleron. "We're now pleased to be able to share these results at this important global muscle meeting as we enroll patients in Part 2 of these Phase 2 trials to evaluate ACE-083's potential to improve functional outcomes in both disorders."

The ACE-083 clinical presentations will be available in the "Science" section on Acceleron's website, www.acceleronpharma.com, on the opening day of the conference.

ACE-083 is currently being evaluated in two Phase 2 trials: one in FSHD and one in CMT. Preliminary results from Part 2 of the trials are expected in the second half of 2019 for FSHD and by year end 2019 for CMT.

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 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 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 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 arms. The lower leg muscle weakness can result in foot drop leading to a high-stepped gait and frequent tripping or falls. The disease is typically diagnosed by the presence of a characteristic pattern of muscle weakness, nerve conduction studies, and genetic testing. There are no FDA approved drug 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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