Acceleron Announces Preliminary Results from Part 1 of the ACE-083 Phase 2 Trial in Patients with Facioscapulohumeral Dystrophy

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- Mean total muscle volume increases of over 12% in the tibialis anterior and biceps brachii muscle cohorts -

- Company plans to initiate Part 2 of the FSHD Phase 2 trial in Q2 2018 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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 positive preliminary results for the first two cohorts in Part 1 of the Phase 2 clinical trial with ACE-083 in patients with facioscapulohumeral dystrophy (FSHD), a rare genetic muscle disorder that results in progressive focal muscle loss and weakness. The Company plans to initiate Part 2 of the ACE-083 FSHD Phase 2 trial during the second quarter of 2018.

"Preliminary results of ACE-083 in FSHD patients demonstrated positive safety and tolerability along with unprecedented mean increases in total muscle volume of over 12% in the two distinct muscles evaluated," said Matthew Sherman, M.D., Chief Medical Officer of Acceleron. "These data support our decision to advance to Part 2 of the Phase 2 trial, which we expect to initiate in the second quarter of this year. We look forward to fully exploring functional outcomes in the larger, placebo-controlled Part 2 of the trial."

Part 1 of the Phase 2 trial is an open-label, dose-escalation study of ACE-083 designed to evaluate safety as well as changes in total muscle volume in up to 36 patients with FSHD. Preliminary results include data from 23 patients evaluable for magnetic resonance imaging (MRI) among two different cohorts (11 patients with tibialis anterior weakness and 12 patients with biceps brachii weakness). Each patient received ACE-083 (150 mg or 200 mg) as a unilateral intramuscular injection once every three weeks for 12 weeks. Total muscle volume changes were measured by MRI relative to baseline at 3 weeks after the last injection of ACE-083. Based on overlap in dosing on a milligram per gram muscle analysis, dose cohorts were pooled for the analyses of each muscle.

Tibialis Anterior Part 1 Cohorts (150 mg and 200 mg pooled) Preliminary Results (n=11):

The tibialis anterior (TA), which is located in the lower leg, is the main muscle responsible for ankle dorsiflexion, or the ability to lift the front of the foot when taking a step. Over 70% of FSHD patients experience tibialis anterior weakness over the course of their disease, which can lead to general decreased mobility and an increased frequency of falling.

- The TA cohorts generated a mean total muscle volume increase of 12.6%.
- The TA cohorts produced a mean decrease or improvement in muscle fat fraction of 5.3%

Biceps Brachii Part 1 Cohorts (150 mg and 200 mg pooled) Preliminary Results (n=12):

The biceps brachii (BB), which is located in the upper arm, is a major muscle responsible for elbow flexion, or the ability to lift the lower arm. A majority of FSHD patients experience biceps brachii weakness early in their disease, which leads to the inability to lift objects or perform other important daily activities without assistance.

- The BB cohorts generated a mean total muscle volume increase of 13.2%.
- The BB cohorts produced a mean decrease or improvement in muscle fat fraction of 0.6%.

In the BB cohorts, the majority of patients had less intramuscular fat at baseline relative to the patients in the TA cohorts. Patients with higher fat fraction in the BB cohorts at baseline demonstrated larger decreases in fat fraction with treatment.

"I am encouraged by the preliminary safety and tolerability results of ACE-083 in Part 1 of the trial. There are currently no FDA approved therapies for FSHD, therefore limiting our treatment of patients to supportive options such as physical therapy and bracing," said Dr. Jeffrey Statland, M.D., Associate Professor of Neurology at the University of Kansas Medical Center and the ACE-083 FSHD Phase 2 trial principal investigator. "ACE-083 is demonstrating encouraging activity in its ability to increase muscle volume, and I look forward to its advancement in Part 2 of the trial."

Strength and Function Tests:

Strength and function tests are being explored in Part 1 to assist with the design of the randomized, double-blind, placebo-controlled Part 2 of the study. Given the lack of placebo control and small sample size of patients in Part 1, no conclusions can be made on the strength and function assessments at this time. Effects of ACE-083 on strength and function versus a placebo-control will be evaluated in Part 2 of the study.

Part 1 Preliminary Safety Results (n=25):

There were no serious adverse events reported. The most common adverse events were injection site related and grades 1-2. One patient experienced a related grade 3 non-serious adverse event of lower leg intramuscular swelling. This patient fully recovered and was discontinued from the study.

Acceleron plans to present an in-depth review of Part 1 data at a medical conference in 2018.

For additional information on this clinical trial, please visit clinicaltrials.gov, identifier NCT02927080.

About ACE-083

ACE-083 is a 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 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 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 and LinkedIn.

Forward-Looking Statements

Cautionary Note Regarding 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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