



Acceleron Announces Topline Results from the Phase 2 Trial of ACE-083 in Patients with Facioscapulohumeral Muscular Dystrophy

September 16, 2019

- ACE-083 did not achieve statistically significant improvements in functional endpoints relative to placebo –
- Acceleron to discontinue development of ACE-083 in facioscapulohumeral muscular dystrophy –
- Topline results from the Phase 2 trial of ACE-083 in Charcot-Marie-Tooth disease expected in Q1 2020 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 16, 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 treatment with ACE-083 in patients with facioscapulohumeral muscular dystrophy (FSHD) did not achieve functional secondary endpoints in the Phase 2 trial.

Although ACE-083 demonstrated a robust, statistically significant increase in mean total muscle volume, the primary endpoint of the trial, the increase failed to translate to statistically significant improvements in functional tests. As a result, Acceleron will not conduct further clinical trials of ACE-083 in FSHD.

"We are certainly disappointed with these results. As we have stated consistently, for ACE-083 to become an important new therapy for patients with FSHD, it would have to deliver a meaningful functional benefit on top of an ability to grow muscle," said Habib Dable, President and Chief Executive Officer of Acceleron. "Unfortunately, in this case, the data show no evidence of such a benefit and, therefore, do not support further development of ACE-083 for FSHD. We're grateful to the patients, families, caregivers, and investigators who participated in this research."

Dable added: "We now look toward the first quarter of next year, when we expect topline results from the placebo-controlled Phase 2 trial of ACE-083 in patients with Charcot-Marie-Tooth disease—a neuromuscular disorder of different pathophysiology."

In this Phase 2 trial in patients with FSHD, ACE-083 was generally well tolerated. Adverse events were mostly mild to moderate (Grade 1 or 2) and injection-site related. Acceleron expects to present results at a future medical meeting.

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

FSHD Phase 2 Trial Design

The two-part Phase 2 clinical trial was designed to evaluate ACE-083 in FSHD patients with muscle weakness in the biceps brachii (BB) and the tibialis anterior (TA), a muscle in the lower leg involved in foot dorsiflexion (raising the foot at the ankle). Part 1 was an open-label, dose-escalation study, with ACE-083 administered by injection into the BB or TA muscle to evaluate safety and increases in muscle volume over a 3-month treatment period. Part 2 was a randomized, double-blind, placebo-controlled study using the optimal dose level selected in Part 1. A total of 56 patients were randomized in Part 2 to receive either placebo or ACE-083 and were evaluated for changes in muscle volume, fat fraction, strength, function, quality of life, and safety over a 6-month primary treatment period, followed by a 6-month open-label treatment period.

For additional information about this clinical trial, please visit www.clinicaltrials.gov.

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 program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in Charcot-Marie-Tooth disease, and is conducting 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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