Acceleron Announces Publication of ACE-083 Phase 1 Trial Results in Muscle & Nerve

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 28, 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 journal *Muscle & Nerve* has published the final results from the Phase 1 trial of ACE-083, the Company's locally acting, “Myostatin+”, muscle agent. ACE-083 treatment generated dose-dependent mean total muscle volume increases of up to 14.5% in the rectus femoris (RF) and 8.9% in the tibialis anterior (TA) three weeks after the last dose.

“These unprecedented increases in total muscle volume produced in the Phase 1 trial of ACE-083 were critical in leading us to rapidly advance into two Phase 2 trials in two distinct diseases of focal muscle weakness, and we are thrilled that the *Muscle & Nerve* journal has selected this study for publication,” said Matthew Sherman, M.D., Chief Medical Officer of Acceleron. “The significant level of activity observed with ACE-083 in this Phase 1 trial was recently confirmed in Part 1 of the facioscapulohumeral dystrophy (FSHD) Phase 2 trial, with preliminary results generating mean total muscle volume increases of over 12% in the two muscles evaluated. We believe ACE-083 could have a significant impact in increasing muscle mass, strength, and function in patients with neuromuscular disorders.”

The article, entitled “Locally Acting ACE-083 Increases Muscle Volume in Healthy Volunteers” is now available online and will be published in a future print issue of *Muscle & Nerve*. The Phase 1 trial was a randomized, double-blind, placebo-controlled, dose-ranging study in healthy volunteers. The key objectives of the trial were safety and tolerability, as well as assessing changes in total muscle volume of the treated muscles via magnetic resonance imaging (MRI) of the RF and TA muscles.

ACE-083 is currently being evaluated in two Phase 2 trials in FSHD and Charcot-Marie-Tooth (CMT) disease. The Company recently announced preliminary results from Part 1 of the FSHD trial with mean total volume increases of over 12% in the tibialis anterior and biceps brachii muscle cohorts. The final Part 1 results from both Phase 2 trials are expected in the second half of 2018. Part 2 of the FSHD trial is expected to be initiated in the second quarter of 2018 with results expected in the second half of 2019. Part 2 of the CMT trial is expected to be initiated before the end of 2018.

Presentations outlining the Phase 1 results included in the publication are available online under the science page on the Company’s website at www.acceleronpharma.com.

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 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.

Cautionary Note on 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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