Acceleron to Present Preliminary Data on ACE-083 at the 2018 Annual Meeting of the Peripheral Nerve Society

June 26, 2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 26, 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 oral and poster presentations on ACE-083 from the ongoing Phase 2 trial in patients with Charcot-Marie-Tooth (CMT) disease at the Peripheral Nerve Society (PNS) annual meeting held in Baltimore, Maryland, July 22-25, 2018.

Oral presentation

Title: **Preliminary Phase 2 Results for ACE-083, Local Muscle Therapeutic, in Patients with CMT1 and CMTX**

Session: Oral Abstract Session II
Date: Monday, July 23rd
Time: 9:00 - 9:15 a.m. EDT (Renaissance Baltimore Harborplace Hotel)

Poster presentation

Title: **Preliminary Phase 2 Results for ACE-083, Local Muscle Therapeutic, in Patients with CMT1 and CMTX**

Session: Poster Session II
Date: Monday, July 23rd
Time: 1:00 - 2:00 p.m. EDT (Renaissance Baltimore Harborplace Hotel)

The ACE-083 clinical presentations will be available immediately following the presentation at the conference in the “Science” section on Acceleron’s website, www.acceleronpharma.com.

ACE-083 is currently being evaluated in two Phase 2 trials: one in facioscapulohumeral muscular dystrophy (FSHD) and one in CMT disease. The final Part 1 results from both Phase 2 trials are expected in the second of half of 2018. Part 2 of the CMT trial is expected to be initiated by the end of 2018.

CMT Phase 2 Trial Design

The two-part Phase 2 clinical trial is designed to evaluate ACE-083 in CMT patients with muscle weakness in the tibialis anterior (TA), a muscle in the lower leg involved in foot dorsiflexion (raising the foot at the ankle). Part 1 is an open-label, dose-escalation study, with ACE-083 administered by injection into the TA muscle once every 3 weeks in up to 18 patients to evaluate safety and increases in muscle volume over a 3-month treatment period. Part 2 is a randomized, double-blind, placebo-controlled study using the optimal dose level selected in Part 1. A total of 40 patients will be randomized in Part 2 to receive either placebo or ACE-083 and will be evaluated for changes in muscle volume, fat fraction, strength, function 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, identifier NCT03124459.

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.

About Charcot-Marie-Tooth Disease (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 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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