Acceleron Announces ACE-083 Phase 2 Trial Presentation at the American Academy of Neurology 70th Annual Meeting

April 26, 2018

– Oral presentation to highlight preliminary results of ACE-083's Phase 2 trial in patients with facioscapulohumeral muscular dystrophy –

– Part 2 of the ongoing Phase 2 trial in patients with facioscapulohumeral muscular dystrophy has been initiated –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 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 that preliminary results from the ongoing Phase 2 trial of ACE-083 in facioscapulohumeral muscular dystrophy (FSHD) are being presented at the American Academy of Neurology (AAN) 70th Annual Meeting in Los Angeles, California on April 26, 2018.

The oral presentation will include preliminary findings from cohorts 1 and 2 in the open-label, dose escalation Part 1 of the ongoing Phase 2 trial of ACE-083 in patients with FSHD.

Oral Presentation:

Title:	**Preliminary Results from a Phase 2 Study to Evaluate ACE-083, a Local Muscle Therapeutic, in Patients with Facioscapulohumeral Muscular Dystrophy**
	(Abstract #S38.001)
Session:	S38 “Best of” Session: Clinical Trial Updates in Neuromuscular Disorders
Presenter:	Dr. Jeffrey Statland, University of Kansas Medical Center
Date:	Thursday, April 26th
Time:	8:00 a.m. PDT (Los Angeles Convention Center, Room 408B)

The ACE-083 AAN presentation is available in the “Science” section on Acceleron’s website, www.acceleronpharma.com.

ACE-083 is currently being evaluated in two Phase 2 trials: one in FSHD and one in Charcot-Marie-Tooth (CMT) disease. The final Part 1 results from both Phase 2 trials are expected in the second of half of 2018.

The randomized, double-blind, placebo controlled Part 2 of the FSHD study has been initiated, with results expected in the second half of 2019. Part 2 of the CMT trial is expected to be initiated by the end of 2018.

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 muscular dystrophy (FSHD) and Charcot-Marie-Tooth (CMT) disease, in which improved muscle strength in targeted muscles may provide a clinical benefit and enhance quality of life.

About Facioscapulohumeral Muscular 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 planned Phase 2 trial of 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 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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