March 16, 2017

Acceleron Announces Plans to Initiate a Phase 2 Trial of ACE-083 in Charcot-Marie-Tooth Neuromuscular Disease and Host Educational Webinar

- Second Phase 2 study for ACE-083 expected to begin 2H 2017 -

- Company to host educational webinar with leading clinical researcher on April 3rd at 11:00 a.m. EDT -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN), a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases, today announced that in the second half of 2017 it plans to initiate a Phase 2 clinical trial of ACE-083, the Company's locally acting muscle agent, for the treatment of patients with Charcot-Marie-Tooth disease (CMT), one of the most common inherited neurologic diseases leading to focal muscle weakness.

"We are pleased to expand our clinical development program for ACE-083 into a second neuromuscular disease," said Matthew Sherman, M.D., Executive Vice President and Chief Medical Officer at Acceleron. "We believe ACE-083 can strengthen the targeted leg muscles in CMT patients and thereby improve their ability to walk and avoid falls. We look forward to hosting the educational webinar during which we will describe CMT and our enthusiasm for the potential of ACE-083 to address the substantial unmet medical needs of patients."

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 phase of the 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 phase using the optimal dose level selected in Part 1. A total of 24 patients will be randomized in Part 2 to receive either placebo or ACE-083 and will be evaluated for increases in muscle volume, strength, function and safety over a 3-month treatment period.

Webinar

The Company will host an educational webinar providing an overview of CMT and a detailed review of the Company's Phase 2 clinical trial. The webinar will be led by neuromuscular disease expert David Walk, M.D., Director of the multidisciplinary CMT and ALS clinics at the University of Minnesota Medical Center Fairview, and Acceleron management, and will take place on Monday, April 3, 2017 at 11:00 a.m. EDT.

Conference Call and Webinar Details

Date: Monday, April 3, 2017
Time: 11:00 a.m. (EDT)

Participants can access the live conference call by dialing 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the Acceleron CMT Webinar.

The live webinar can be accessed on the Investors page of the Company's website at www.acceleronpharma.com.

A replay of the webinar will be available approximately two hours after the event on the Acceleron website.

About ACE-083

ACE-083 is a therapeutic candidate that acts as a ligand trap for members in the transforming growth factor-beta (TGF-β) superfamily involved in the regulation of muscle mass and strength. ACE-083 has been designed to increase muscle mass and strength selectively in the muscles into which the drug is administered. Acceleron is developing ACE-083 for diseases

such as facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease in which improved muscle strength in target muscles may provide a clinical benefit.

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 and a high-stepped gait leading to frequent tripping or falls. The disease is typically diagnosed by the presence of a characteristic pattern of muscle weakness and other clinical signs and symptoms, as well as through genetic testing. There are no FDA approved drug therapies for CMT.

About Acceleron

Acceleron is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative therapeutics to treat serious and rare diseases. Its pioneering research platform leverages the powerful biology behind the body's ability to rebuild and repair its own cells and tissues. This approach to drug discovery has generated four therapeutic candidates that are currently in clinical trials. The Company's lead therapeutic candidate, luspatercept, is being evaluated in Phase 3 studies for the treatment of the hematologic diseases myelodysplastic syndromes (MDS) and beta-thalassemia under a global partnership with Celgene Corp. Acceleron is also advancing clinical programs in the fields of oncology and neuromuscular diseases and has a comprehensive preclinical research effort targeting fibrotic and other serious diseases.

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 compound ACE-083, the timeline for clinical development and regulatory approval of the ACE-083, the expected timing for the reporting of data from ongoing trials, and the structure of the Company's planned or pending clinical trials. The words "anticipate," "appear," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "predict," "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.

Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks 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 data may not be available when the Company expects it to be, that the Company will be unable to successfully complete the clinical development of the Company's compounds, that the development of the Company's compounds will take longer or cost more than planned, 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.

Other risks and uncertainties include those identified under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K which was filed with the Securities and Exchange Commission (SEC) on March 1, 2017, 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 reflect the Company's current views 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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Acceleron Pharma Inc.
Todd James, IRC, 617-649-9393
Senior Director, Investor Relations and Corporate Communications
or
Media:
BMC Communications
Brad Miles, 646-513-3125

Source: Acceleron Pharma