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Acceleron Announces First Patient Treated in Phase 2 Clinical Trial of ACE-083 in Charcot-Marie-Tooth Disease

- Phase 2 trial in CMT expands wholly-owned ACE-083 program and muscle franchise into new area of high unmet medical need -

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 the first patient has been treated in 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 neurological diseases leading to focal muscle weakness.

"People diagnosed with CMT currently have no drug therapy options to address the major consequences of their disease, such as impaired walking and falls due to progressive muscle weakness in the lower leg," said Colin Quinn, M.D., an Assistant Professor of Clinical Neurology in the Perelman School of Medicine at the University of Pennsylvania, who is the Principal Investigator for the Penn site of the multicenter trial. "ACE-083 has the potential to increase muscle growth and strength in the lower leg muscles we are targeting, and could improve patients' ability to walk."

"We are proud to have advanced ACE-083 into a second Phase 2 clinical trial," said Matthew Sherman, M.D., Executive Vice President and Chief Medical Officer at Acceleron. "We designed our clinical development strategy for ACE-083 to explore its activity in diseases with weakness in specific muscles due to an underlying neurological or muscle disorder. With Phase 2 trials now underway in both CMT and facioscapulohumeral muscular dystrophy, we will be able to evaluate ACE-083's effect on both muscle strength and function across a range of neuromuscular diseases."

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 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.

For additional information about this clinical trial, please visit clinicaltrials.gov, identifier NCT03124459.

About ACE-083

ACE-083 is a therapeutic candidate based on the naturally-occurring protein, follistatin, that acts as a ligand trap for members in the transforming growth factor-beta superfamily involved in regulating muscle growth and strength. ACE-083 has been 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 and Charcot-Marie-Tooth disease 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 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. 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. Acceleron is also advancing its ACE-083 clinical program in the field of neuromuscular disease, 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 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," "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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