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## **Acceleron Announces First Patient Treated in Phase 2 Trial of ACE-083 in Facioscapulohumeral Muscular Dystrophy**

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 facioscapulohumeral muscular dystrophy (FSHD).

"We are excited to start our first Phase 2 trial for ACE-083," said Matthew Sherman, M.D., Chief Medical Officer of Acceleron. "ACE-083 has generated a strong data set in both preclinical and clinical trials to date. Our understanding of its novel mechanism of action suggests that ACE-083 may become an important new treatment for FSHD patients with muscle weakness that negatively affects their strength and functional abilities."

The two-part Phase 2 clinical trial is designed to evaluate ACE-083 in FSHD patients with muscle weakness in either the tibialis anterior, a muscle in the lower leg involved in foot flexion, or the biceps. Part 1 is an open-label, dose-escalation study of ACE-083 administered by intramuscular injection once every three weeks in up to 36 patients to evaluate safety and increases in muscle volume over a three-month treatment period. Part 2 is a randomized, double-blind, placebo-controlled study using the optimal dose level selected in Part 1. Up to 40 patients will be randomized to receive either placebo or ACE-083 administered by intramuscular injection once every three weeks and evaluated for increases in muscle volume, strength, function and safety over a three-month treatment period.

For additional information on this clinical trial, please visit [clinicaltrials.gov](http://clinicaltrials.gov), identifier NCT02927080.

### **About ACE-083**

ACE-083 is a therapeutic candidate that acts as a ligand trap for members in the transforming growth factor-beta (TGF- $\beta$ ) 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 in which improved muscle strength in a specific set of muscles may provide a clinical benefit.

### **About Facioscapulohumeral Muscular Dystrophy (FSHD)**

FSHD is a rare genetic myopathy affecting approximately 19,000 people in the United States for which there are no approved treatments. The primary clinical phenotype of FSHD is debilitating skeletal muscle deterioration and weakness. The symptoms of FSHD develop in a descending pattern starting with the face and upper body to the lower body and progressing in a "muscle by muscle" fashion. The disease is typically diagnosed by the presence of a characteristic pattern of muscle weakness and other clinical symptoms, as well as through genetic testing.

### **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](http://www.acceleronpharma.com). Follow Acceleron on social media: [@AcceleronPharma](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleron-pharma).

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