Acceleron Announces ACE-083 Phase 1 Results at the 14th International Congress on Neuromuscular Diseases

-ACE-083 increased muscle volume of the tibialis anterior muscle by 8.9% in healthy volunteers-

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Acceleron Pharma Inc. (NASDAQ:XLRN), a clinical stage biopharmaceutical company focused on the discovery and development of novel therapeutic candidates that engage the body's ability to rebuild and repair its own cells and tissues, today announced ACE-083 Phase 1 clinical trial results in healthy volunteers at the 14th International Congress on Neuromuscular Diseases in Toronto, Canada. ACE-083 is designed to selectively increase muscle mass and strength in the muscles in which the drug is administered.

Results highlighted in the poster presentation (Abstract #266, "ACE-083, A Locally-Acting Muscle Agent, Increases Muscle Volume in Healthy Volunteers") showed that ACE-083 produced statistically-significant, dose-dependent increases in muscle volume, assessed by magnetic resonance imaging (MRI), of the tibialis anterior muscle. At the highest dose level, ACE-083 generated a mean increase in tibialis anterior muscle volume of 8.9%.

"The new ACE-083 results in the tibialis anterior muscle are very exciting as they confirm the significant biological activity that we previously observed in the rectus femoris muscle in healthy volunteers," said Matthew L. Sherman, M.D., Chief Medical Officer of Acceleron. "The positive results in the tibialis anterior extend our previously reported positive findings and provide us with valuable clinical experience in a target muscle for our planned Phase 2 trial in facioscapulohumeral muscular dystrophy (FSHD) patients."

Acceleron intends to advance ACE-083 into a Phase 2 clinical trial in patients with FSHD in the second half of 2016.

ACE-083 Phase 1 Results

Acceleron previously reported positive results from the Phase 1 randomized, double-blind, placebo-controlled, dose-ranging study in healthy volunteers for trial cohorts 1 through 5 assessing muscle volume changes of the rectus femoris muscle in the thigh. Today, the Company presented positive data from trial cohorts 6 and 7, which assessed muscle changes of the tibialis anterior muscle in the shin. In each new cohort, six subjects received ACE-083 (cohort 6: 100 mg and cohort 7: 150 mg) and three subjects received placebo. The subjects received two intramuscular doses in the tibialis anterior muscle three weeks apart with MRI evaluations at baseline, three weeks post last dose, and eight weeks post last dose.

At three weeks after the last dose, ACE-083 generated dose-dependent increases in muscle volume, assessed by MRI. In the placebo, ACE-083 100 mg and ACE-083 150 mg cohorts, tibialis anterior muscle volume increases were -0.1%, 5.0% ($p<0.05$ vs placebo) and 8.9% ($p<0.001$ vs placebo), respectively.

There were no serious adverse events, dose-limiting toxicities, or discontinuations due to adverse events. All adverse events were grade 1 or 2, transient, and most commonly injection-site related, and there was a similar incidence of adverse events observed in placebo and ACE-083 treated groups.

The clinical poster is available on Acceleron's website (www.acceleronpharma.com) under the Science tab.

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 in which improved muscle strength in a specific set of muscles may provide a clinical benefit, such as facioscapulohumeral muscular dystrophy. The Phase 1 clinical trial of ACE-083 in healthy volunteers has been completed. For additional information on this clinical trial, please visit www.clinicaltrials.gov, identifier NCT02257489.

About Acceleron

Acceleron discovers and develops novel therapies to treat a wide range of 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 innovative approach to drug discovery has generated four therapeutic candidates 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 Acceleron's strategy, future plans and prospects, including statements regarding the development of ACE-083, the timeline for clinical development and regulatory approval of Acceleron's compounds, the expected timing for the reporting of data from ongoing trials, and the structure of Acceleron'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 Acceleron'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 Acceleron expects it to be, that Acceleron will be unable to successfully complete the clinical development of Acceleron's compounds, that the development of Acceleron's compounds will take longer or cost more than planned, that Acceleron may be delayed in initiating or completing any clinical trials, and that Acceleron'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 Acceleron's Annual Report on Form 10-K which was filed with the Securities and Exchange Commission (SEC) on February 25, 2016, and other filings that Acceleron has made and may make with the SEC in the future. The forward-looking statements contained in this press release reflect Acceleron's current views with respect to future events, and Acceleron does not undertake and specifically disclaims any obligation to update any forward-looking statements.

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