



Acceleron Discontinues Development of Phase 1 Molecule ACE-2494

April 4, 2019

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 4, 2019-- Acceleron Pharma Inc. (NASDAQ:XLRN), a leading biopharmaceutical company in the discovery and development of TGF-beta superfamily therapeutics to treat serious and rare diseases, today announced it is discontinuing development of ACE-2494, a systemic muscle agent the company had been studying in a Phase 1 healthy volunteer trial for the potential treatment of neuromuscular disorders.

"Although ACE-2494 showed promising early signs of target engagement in our recently completed Phase 1 trial, the frequency of anti-drug antibodies (ADAs) observed among participants has led us to discontinue the program," said Habib Dable, President and Chief Executive Officer of Acceleron. "The formation of ADAs was not associated with any adverse event, but the ADA profile is not consistent with a clinical program that Acceleron would advance. We greatly appreciate the efforts of those who participated in this trial, and plan to leverage the clinical data to help inform future drug discovery efforts in neuromuscular disease."

About Acceleron

Acceleron is a 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 program with ACE-083, a locally-acting Myostatin+ agent in Phase 2 development in facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth disease and is conducting a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit www.acceleronpharma.com. Follow Acceleron on social media: [@AcceleronPharma](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleron-pharma).

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 will be unable to successfully complete the clinical development of the Company's compounds, 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. 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.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190404005769/en/>

Source: Acceleron Pharma Inc.

Acceleron Pharma Inc.

Investors:

Todd James, IRC, 617-649-9393

Vice President, Investor Relations and Corporate Communications

Media:

Matt Fearer, 617-301-9557

Director, Corporate Communications