January 6, 2017

Acceleron Outlines Corporate Goals and Priorities for 2017

- Luspatercept MEDALIST Phase 3 trial in MDS expected to complete enrollment in the 2nd half of 2017 -

- Luspatercept BELIEVE Phase 3 trial in beta-thalassemia expected to complete enrollment in the 2nd half of 2017 -

- Plan to initiate new luspatercept Phase 2 clinical trials in distinct patient segments in MDS and beta-thalassemia and initiate first Phase 2 trial in myelofibrosis -

- Advance and expand the ACE-083 program with first data in FSHD patients by year-end and initiate a Phase 2 trial in a 2nd neuromuscular disease this year -

- Further broaden pipeline by advancing our 5th internally discovered protein therapeutic, ACE-2494, in a Phase 1 trial -

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 its major corporate research and development goals and priorities for 2017.

"With our Phase 3 luspatercept programs in MDS and beta-thalassemia advancing on plan, 2017 will be a transformational year for Acceleron. We are looking ahead to the clinical, regulatory and commercial milestones that will help us achieve our vision of becoming a fully integrated biopharmaceutical company," said Habib Dable, President and Chief Executive Officer of Acceleron. "Additionally, we continue to advance and expand our wholly-owned portfolio of innovative protein therapeutics for patients with serious diseases. With multiple Phase 3 and Phase 2 trials ongoing and new INDs expected in 2017 and 2018, we believe that our pipeline of therapeutic candidates positions us to create significant value for our shareholders while making a meaningful difference in the lives of patients who have limited treatment options."

The Company's major research and development goals and priorities are highlighted below:

Luspatercept in Rare Blood Disorders

Luspatercept is being developed to treat patients who have anemia associated with rare blood disorders, including beta-thalassemia and malignant disorders such as myelodysplastic syndromes (MDS) and myelofibrosis.

Goals for luspatercept in myelodysplastic syndromes (MDS):

- Complete patient enrollment in the MEDALIST Phase 3 clinical trial in the second half of this year
- Release topline results for the MEDALIST Phase 3 trial by the end of next year
- Evaluate and design a clinical and regulatory strategy for luspatercept in first-line lower risk MDS patients

Goals for luspatercept in beta-thalassemia:

- Complete patient enrollment in the BELIEVE Phase 3 clinical trial in the second half of this year
- Release topline results for the BELIEVE Phase 3 trial by the end of next year
- Initiate a Phase 2 trial in patients with non-transfusion dependent beta-thalassemia by the end of this year

Goals for luspatercept in myelofibrosis:

- Initiate a Phase 2 trial in myelofibrosis by the end of this year

ACE-083 in Neuromuscular Disease

ACE-083 is being developed to increase muscle mass and strength in target muscle groups for diseases such as facioscapulohumeral muscular dystrophy (FSHD), where patients experience focal muscle loss. Acceleron plans to:

- Present initial topline results from the open label, dose-escalation stage of the Phase 2 study in FSHD in late 2017
- Initiate the randomized, double-blind, placebo-controlled stage of the Phase 2 study in 2018
- Initiate a Phase 2 clinical trial in a second neuromuscular disease

Pipeline Expansion

Acceleron continues its research on several preclinical protein therapeutics targeting fibrotic disorders, vascular disease, and musculoskeletal disease. Acceleron's current goals for research and pipeline expansion include:

- Initiate a Phase 1 healthy volunteer study with ACE-2494 this year
- Conduct IND-enabling development work to advance a new protein therapeutic to the clinic in 2018
- Host an investor and analyst research day to discuss ongoing preclinical research and potential future disease areas in the second quarter of this year

Dalantercept in Advanced Renal Cell Carcinoma

Dalantercept is being developed in advanced renal cell carcinoma in combination with axitinib to further inhibit tumor angiogenesis. Acceleron expects to present topline results from the Phase 2 DART study in the second half of 2017. The primary endpoint of this trial, progression-free survival (PFS), is an event-driven assessment.

A slide presentation describing these research and development goals and other information will be available on the Investors page on the Company's website at www.acceleronpharma.com on Monday, January 9, 2017.

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 compounds, including luspatercept, dalantercept, ACE-083, ACE-2494, the Company's IntelliTrap™ drug discovery platform, and the Company's TGF-beta superfamily program generally, the timeline for clinical development and regulatory approval of the Company's compounds, 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," "believe," "continue," "could," "estimate," "expect," "forecast," "goal", "intend," "may," "plan," "potential," "project," "should," "strategy," "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 or its collaboration partner, Celgene, 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 or Celgene may be delayed in initiating or completing any clinical trials, that the Company's drug discovery activities may not yield drug candidates for which the Company can commence clinical trials at the rate at which the Company currently anticipates or at all, 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 February 25, 2016, 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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