September 5, 2017

Acceleron Announces Publication of Luspatercept Phase 2 Myelodysplastic Syndromes Study Results in The Lancet Oncology

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, announced that *The Lancet Oncology* has published results from the Phase 2 study of luspatercept in patients with lower-risk myelodysplastic syndromes (MDS).

"The efficacy and safety results in this Phase 2 study support continued research into luspatercept for the treatment of refractory anemia which often requires red blood cell transfusions in lower-risk MDS patients," said Uwe Platzbecker, M.D., Professor of Hematology and Head of the MDS program at the University Hospital in Dresden, Germany. "This novel investigational therapy has the potential to address a significant need in MDS patients who currently have very limited options for managing chronic anemia. We have already begun exploring luspatercept's activity across a range of MDS patient populations."

"We are pleased that *The Lancet Oncology* chose to publish these results," said Matthew Sherman, M.D., Chief Medical Officer of Acceleron. "The positive clinical activity demonstrated in this study informed our earlier decision to initiate the pivotal MEDALIST Phase 3 trial in lower-risk MDS, and we expect to report top-line results from this trial in mid-2018."

The article, entitled "Luspatercept for the treatment of anaemia in patients with lower-risk myelodysplastic syndromes: a phase 2 dose-finding study with long-term extension study" is now available online and will be published in a future print issue of *The Lancet Oncology*. The journal also published online a companion Comment article, "Defeating anaemia in myelodysplastic syndromes: another step forward," by Valeria Santini, M.D., Associate Professor of Hematology at the University of Florence Medical School in Florence, Italy.

Phase 2 presentations of luspatercept in MDS presented at recent medical conferences include updated longer-term follow-up and new expansion cohort preliminary results beyond that incorporated in this publication. Presentations outlining these results are available online under the science page on the Company's website at www.acceleronpharma.com.

The MEDALIST trial, a global Phase 3 study of luspatercept in lower-risk MDS patients, is fully enrolled and top-line results are expected in mid-2018. The MEDALIST trial enrolled patients who are ring sideroblast-positive, red blood cell transfusion dependent, and are erythropoiesis-stimulating agent (ESA)-refractory or ESA-treatment ineligible, based on erythropoietin levels greater than 200 units per liter at baseline. In early 2018, Acceleron and Celgene plan to initiate the COMMANDS Phase 3 trial in first-line, lower-risk MDS patients.

Luspatercept is an investigational product that is not approved for use in any country.

About Luspatercept

Luspatercept is a modified activin receptor type IIB fusion protein that acts as a ligand trap for members of the transforming growth factor-beta superfamily involved in the late stages of erythropoiesis (red blood cell production). Luspatercept regulates late-stage erythrocyte (red blood cell) precursor cell differentiation and maturation. This mechanism of action is distinct from that of erythropoiesis stimulating agents (ESAs), which stimulate the proliferation of early-stage erythrocyte precursor cells. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Phase 3 clinical trials are underway to evaluate the safety and efficacy of luspatercept in patients with myelodysplastic syndromes (the MEDALIST study) and in patients with beta-thalassemia (the BELIEVE study). For more information, please visit www.clinicaltrials.gov.

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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