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Acceleron Provides Updated Results from Ongoing Phase 2 Study of Luspatercept in Myelodysplastic Syndromes at the 22nd Congress of the European Hematology Association

- Results from ongoing trials demonstrate increases in hemoglobin and reductions in red blood cell transfusion burden sustained for more than 26 months -

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 preliminary results from the ongoing Phase 2 studies of luspatercept in patients with lower-risk myelodysplastic syndromes (MDS) at the 22nd Congress of the European Hematology Association (EHA) in Madrid, Spain. Luspatercept is being developed to treat a range of hematologic diseases including MDS, beta-thalassemia, and myelofibrosis as part of a global collaboration between Acceleron and Celgene.

"This Phase 2 update further supports our confidence that luspatercept could become a potential first-in-class treatment for lower-risk MDS patients. With some patients continuing on study for more than 26 months, we are very encouraged by both the durability of response and safety profile of luspatercept," said Habib Dable, President and Chief Executive Officer of Acceleron. "With Phase 3 trials across two indications ongoing and new studies planned, we and Celgene remain committed to exploring the full opportunity for luspatercept to transform patients' lives."

Phase 2 Results

A total of 88 lower-risk MDS patients have been treated with therapeutic dose levels of luspatercept in the ongoing Phase 2 study.

- | 50% (44 of 88) achieved a clinically meaningful erythroid response of an increase in hemoglobin or reduction in red blood cell (RBC) transfusion burden as per the International Working Group's Hematologic Improvement Erythroid (IWG HI-E) response criteria.
- | 38% (23 of 60 patients with ≥ 2 units RBC / 8 weeks transfusion burden at baseline) achieved RBC transfusion independence (RBC-TI) for ≥ 8 weeks.
- | Patients with a low transfusion burden (< 4 units / 8 weeks and hemoglobin < 10 g/dL) demonstrated a clinically meaningful increase in hemoglobin for up to 26 months, with several remaining on treatment.

The results presented at EHA confirm and extend previously reported results across the lower-risk MDS patient subpopulations, showing erythroid responses regardless of prior use of erythropoiesis-stimulating agents (ESA), baseline erythropoietin (EPO) levels, and ring sideroblast (RS) status.

Phase 2 Safety Summary

A total of 95 lower-risk MDS patients have been treated with luspatercept in the ongoing Phase 2 studies (all dose levels).

- | The majority of adverse events (AEs) were Grade 1 or 2. AEs possibly related to study drug that occurred in at least three patients during the studies were headache, fatigue, hypertension, bone pain, diarrhea, arthralgia, injection site erythema, myalgia, and edema peripheral.
- | Grade 3 non-serious AEs possibly related to study drug were ascites, blast cell count increase, blood bilirubin increase, bone pain, hypertension, platelet count increase, and pleural effusion. These Grade 3 non-serious AEs occurred in six individual patients with one patient accounting for both the ascites and pleural effusion AEs.
- | Grade 3 serious AEs (SAEs) possibly related to study drug were ataxia, general physical health deterioration, and myalgia; a Grade 2 SAE possibly related to study drug of muscle weakness was reported.

"The longer term results of these Phase 2 studies reinforce the potential of inhibiting ligands in the TGF-beta superfamily for patients with lower-risk MDS," said Michael Pehl, President, Hematology/Oncology for Celgene. "With the Phase 3 study

now fully enrolled, we look forward to advancing luspatercept as part of our ongoing commitment to individuals with MDS around the world."

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

The MEDALIST trial, a global Phase 3 study of luspatercept in patients with lower-risk MDS who require red blood cell transfusions, is fully enrolled and top-line results are expected in mid-2018.

The MDS poster presentation is available under the Science page of the Company's website at www.acceleronpharma.com/.

About the MDS Phase 2 Studies

Data from two Phase 2 studies were presented at the conference: the base study in which patients received treatment with luspatercept for three months and the long-term extension study in which patients who completed the base study may receive treatment with luspatercept for up to an additional five years. In both the three-month base study and the long-term extension study, lower-risk MDS patients were enrolled and treated with open-label luspatercept, dosed subcutaneously once every three weeks.

The outcome measures for the studies included the proportion of patients who had an erythroid response (IWG HI-E) or achieved RBC transfusion independence (RBC-TI). IWG HI-E was defined as hemoglobin increase ≥ 1.5 g/dL sustained for ≥ 8 weeks in patients with < 4 units RBC / 8 weeks transfusion burden at baseline and hemoglobin levels below 10 g/dL. For patients with ≥ 4 units RBC / 8 weeks transfusion burden at baseline, erythroid response was defined as a reduction of ≥ 4 units RBC sustained for ≥ 8 weeks. RBC-TI was defined as no RBC transfusions for ≥ 8 weeks in patients with ≥ 2 units RBC / 8 weeks baseline transfusion burden.

About Luspatercept

Luspatercept is a modified activin receptor type IIB fusion protein that acts as a ligand trap for members in 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. 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. 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 Acceleron's strategy, future plans and prospects, including statements regarding the development of luspatercept, 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 or its collaboration partner, Celgene, 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 or Celgene 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 March 1, 2017, 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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