Acceleron Provides Updated Results from Phase 2 Studies of Luspatercept in Beta-Thalassemia at the 22nd Congress of the European Hematology Association

Results from ongoing study demonstrate increases in hemoglobin and decreases in red blood cell transfusion burden sustained for up to 24 months, with patients still active on treatment

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 study of luspatercept in patients with beta-thalassemia during an oral presentation 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 beta-thalassemia, myelodysplastic syndromes (MDS), and myelofibrosis as part of a global collaboration between Acceleron and Celgene.

â€œWith beta-thalassemia patients now remaining on study for over two years, we continue to be highly encouraged by luspatercept's long-term efficacy results and safety profile,â€• said Habib Dable, President and Chief Executive Officer of Acceleron. â€œCombined with the rapid completion of enrollment in the BELIEVE Phase 3 trial, the programâ€™s momentum continues to build alongside our enthusiasm to potentially transform the treatment of beta-thalassemia patients globally.â€•

Phase 2 Results

A total of 32 transfusion-dependent beta-thalassemia patients have been treated with therapeutic dose levels of luspatercept in the ongoing study.

69% (22 of 32) achieved a reduction in red blood cell (RBC) transfusion burden of at least 33% in any 12-week treatment interval as compared to baseline. A 12-week fixed interval analysis was conducted to review RBC transfusion reduction during weeks 13 to 24 and weeks 37 to 48 compared to the baseline 12-week period pre-treatment in order to evaluate durability of response.

The ongoing BELIEVE Phase 3 trial will use this 12-week fixed interval analysis for evaluating the proportion of patients achieving at least a 33% reduction in RBC transfusion burden.

50% (12 of 24 patients with 6-20 units RBC / 24 weeks estimated pre-treatment) achieved a reduction in RBC transfusion burden of at least 33% in the fixed 12-week interval from weeks 13 to 24 as compared to baseline. 46% (11 of 24 patients with 6-20 units RBC / 24 weeks estimated pre-treatment) achieved a reduction in RBC transfusion burden of at least 33% in the fixed 12-week interval from weeks 37 to 48 as compared to baseline. A total of 31 non-transfusion-dependent beta-thalassemia patients have been treated with therapeutic dose levels of luspatercept in the ongoing study.

71% (22 of 31) achieved a clinically meaningful increase in hemoglobin of at least 1.0 g/dL compared to baseline (mean increase over 12 weeks). There are patients who remain on luspatercept with clinically meaningful increases in hemoglobin and reductions in RBC transfusion burden for up to 24 months.

Phase 2 Safety Summary

A total of 64 beta-thalassemia 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. The most common related AEs (occurring in â‰¥ 10% of patients) were bone pain, headache, myalgia, arthralgia, musculoskeletal pain, asthenia, injection site pain, and back pain. Grade 3 AEs probably related to study drug were bone pain (n=3), asthenia (n=2) and headache (n=1). There were no serious AEs related to study drug. â€œBeta-thalassemia remains an area of critical medical need for many patients around the world,â€• said Michael Pehl, President, Hematology/Oncology for Celgene. â€œThese longer-term results continue to illustrate the potential for luspatercept to affect transfusion dependence and hemoglobin levels, making a meaningful impact for patients with this serious blood disease.â€•

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

The BELIEVE trial, a global Phase 3 study of luspatercept in transfusion-dependent beta-thalassemia patients, is fully enrolled and top-line results are expected in mid-2018.

The EHA beta-thalassemia presentation is available under the Science page of the Companyâ€™s website at www.acceleronpharma.com/.

About the Phase 2 Study

Data from two open-label Phase 2 studies were presented at the conference: the base study in which patients received treatment with luspatercept for three months and the ongoing long-term safety extension study in which patients 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, red blood cell (RBC) transfusion-dependent patients (â‰¥ 4 units RBC / 8 weeks) and non-transfusion-dependent patients (< 4 units RBC / 8 weeks) were enrolled and treated with open-label luspatercept, dosed subcutaneously once every three weeks.

The primary outcome measure of the three-month base study was the proportion of patients who have an erythroid response, defined as 1) a hemoglobin increase of â‰¥ 1.5 g/dL from baseline for â‰¥ 14 days (in the absence of RBC transfusions) in non-transfusion dependent patients, or 2) â‰¥ 20% reduction in RBC transfusion burden compared to pretreatment in transfusion-dependent patients. The primary outcome for the long-term extension study is to evaluate the long-term safety and tolerability of luspatercept.

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 â€œMEDALISTâ€• study) and in patients with beta-thalassemia (the

â€œBELIEVEâ€• 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 bodyâ€™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 Companyâ€™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 visitÂ www.acceleronpharma.com/. Follow Acceleron on Social Media:Â @AcceleronPharmaÂ 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 Acceleronâ€™s compounds, the expected timing for the reporting of data from ongoing trials, and the structure of Acceleronâ€™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 Acceleronâ€™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 Acceleronâ€™s compounds, that the development of Acceleronâ€™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 Acceleronâ€™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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https://www.bioportfolio.com/news/article/3198698/Acceleron-Provides-Updated-Results-from-Phase-2-Studies-of-Luspatercept-in-Beta.html