



Acceleron Announces Luspatercept Presentations at the 61st American Society of Hematology Annual Meeting

November 6, 2019

– Updated results from MEDALIST Phase 3 trial show 47.1% of patients with anemia associated with myelodysplastic syndromes treated with luspatercept achieved red blood cell transfusion independence for ≥ 8 weeks and median total duration of clinical benefit was 83.6 weeks for patients responding to luspatercept as of a January 2019 data cutoff date –

– In the BELIEVE Phase 3 trial, beta-thalassemia patients who were luspatercept responders had durable clinical benefit over the 64.1-week follow-up period –

– Interim results from the ongoing Phase 2 trial of luspatercept in patients with anemia associated with myelofibrosis suggest clinically meaningful activity, with 32% to 53% of transfusion-dependent patients achieving responses when treated in combination with ruxolitinib –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 6, 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 that data from six clinical abstracts on luspatercept will be presented at the 61st American Society of Hematology (ASH) Annual Meeting & Exposition in Orlando, Florida, on December 7-10, 2019. Luspatercept is being developed as part of a global collaboration between Acceleron and Celgene Corporation.

"It's a privilege to return to the world's largest hematology meeting to share updated results from the Phase 3 trials of luspatercept in patients with myelodysplastic syndromes and beta-thalassemia," said Habib Dable, President and Chief Executive Officer of Acceleron. "With the presentations of both trials having garnered 'Best of ASH' honors in 2018, we're thrilled to report that luspatercept has continued to demonstrate durable clinical benefit in both patient populations in the ongoing open-label studies. Moreover, interim Phase 2 data show clinically significant activity from luspatercept treatment in patients with myelofibrosis-associated anemia, further validating our confidence in luspatercept's potential as a therapy for a variety of chronic anemias of high unmet need."

Key Presentations

Oral Presentation, Monday, December 9, 4:30 p.m. EST

Session 637. Myelodysplastic Syndromes – Clinical Studies: Lower-Risk MDS and CMML

Title: Assessment of Longer-Term Efficacy and Safety in the Phase 3, Randomized, Double-Blind, Placebo-Controlled MEDALIST Trial of Luspatercept to Treat Anemia in Patients (Pts) with Revised International Prognostic Scoring System (IPSS-R) Very Low-, Low-, or Intermediate-Risk Myelodysplastic Syndromes (MDS) with Ring Sideroblasts (RS) Who Require Red Blood Cell (RBC) Transfusions

Presenter: Pierre Fenaux

Clinical benefit, defined as a patient achieving red blood cell transfusion independence (RBC-TI) ≥ 8 weeks and/or modified hematologic improvement erythroid (HI-E) response per International Working Group 2006 criteria, was assessed, along with total duration of clinical benefit (time from achieving clinical benefit to discontinuation due to loss of benefit, adverse events [AEs], or other reasons).

- 72 (47.1%) patients treated with luspatercept and 12 (15.8%) treated with placebo achieved RBC-TI ≥ 8 weeks.
- 97 patients responding to luspatercept experienced clinical benefit (as defined above). A median total duration of clinical benefit of 83.6 weeks or approximately 21 months for patients responding to luspatercept was observed.
- Most lower-risk MDS patients achieving RBC-TI and/or HI-E with luspatercept in the MEDALIST study had multiple periods of response with cumulative clinical benefit durability superior to that of patients receiving placebo, including those with a high baseline transfusion burden.
- AEs occurring more frequently with luspatercept vs placebo (fatigue, diarrhea, asthenia, dizziness) occurred early (Cycles 1–4), were mainly grade 1 or 2 and decreased over time.

Summary: Data in the abstract include MEDALIST Phase 3 trial results as of the January 7, 2019 cutoff date.

Poster Presentation, Monday, December 9, 6:00 p.m. EST

Session 112. Thalassemia and Globin Gene Regulation: Poster III

Title: Evaluating Luspatercept Responders in the Phase 3, Randomized, Double-Blind, Placebo-Controlled BELIEVE Trial of Luspatercept in Adult Beta-Thalassemia Patients (Pts) Who Require Regular Red Blood Cell (RBC) Transfusions

Presenter: Vip Viprakasit

Achievement and number of response episodes (defined as $\geq 33\%$ reduction in RBC transfusion from baseline over any consecutive 24 weeks) were assessed at a median follow-up of 64.1 weeks. Duration of clinical benefit, defined as the time of first response ($\geq 33\%$ reduction in RBC transfusion over any 24 weeks) to discontinuation due to any cause at that episode, was also assessed.

- Median duration of clinical benefit (as defined above) for luspatercept responders was 53.5 wks (range 24–93.7). Forty-seven (21.0%) pts receiving luspatercept had no loss of response within the entire study period.
- The average number of RBC units saved over any 24 wks in all luspatercept responders was 6.55 U (0.27 U/wk) and was 8.16 U (0.34 U/wk) with transfusion burden > 15 U/24 wks, compared to baseline.

- The incidence of frequent AEs (bone pain, arthralgia, and dizziness) was consistent with the previously reported 48-week safety profile for luspatercept and decreased over time on study drug with no impact on treatment modification or continuation.

Data in the abstract include results as of the May 11, 2018 cutoff date.

Oral Presentation, Monday, December 9, 7:00 a.m. EST

Session 634. Myeloproliferative Syndromes: Clinical: Emerging and Novel Targeted Therapies

Title: A Phase 2 Study of Luspatercept in Patients with Myelofibrosis-Associated Anemia

Presenter: Aaron T Gerds

Patients with myelofibrosis (MF) receiving a stable dose of ruxolitinib in combination with luspatercept treatment:

- 8 of 14 (57%) non-transfusion-dependent (NTD) patients (trial cohort 3A) achieved a mean hemoglobin (Hb) increase of \geq 1.5 grams per deciliter (g/dL)
- 6 of 19 (32%) transfusion-dependent (TD) patients (trial cohort 3B) achieved RBC-TI over any consecutive 12 weeks
 - 10 (53%) TD patients achieved a \geq 50% reduction in RBC transfusion burden from baseline.

Patients with MF receiving luspatercept without concomitant treatment with ruxolitinib:

- 3 of 20 (15%) NTD patients (trial cohort 1) achieved a mean Hb increase of \geq 1.5 g/dL
- 2 of 20 (10%) TD patients (trial cohort 2) achieved RBC-TI over any consecutive 12 weeks

Four of 74 (5%) patients had grade 3–4 treatment-related adverse events (AEs). Treatment-related AEs occurring in \geq 3% of patients were hypertension (11%), bone pain (8%), and diarrhea (4%). Seven (9%) patients had \geq 1 AE resulting in treatment discontinuation.

Data in the abstract include interim results of the ongoing open-label, phase 2 trial evaluating luspatercept in patients with MF and anemia as of the data cutoff date of May 10, 2019.

Other Luspatercept Abstracts

Poster Presentation, Sunday, December 8, 6:00 – 8:00 p.m. EST

Session 637. Myelodysplastic Syndromes – Clinical Studies: Poster II

Title: Luspatercept Significantly Reduces Red Blood Cell (RBC) Transfusion Burden, Regardless of Gene Mutation Frequency, Spectrum, and Prognostic Significance, Among Patients (Pts) with LR-MDS Enrolled in the MEDALIST Trial

Presenter: Uwe Platzbecker

Poster Presentation, Monday, December 9, 6:00 – 8:00 p.m. EST

Session 637. Myelodysplastic Syndromes – Clinical Studies: Poster III

Title: Hematologic Improvement—Neutrophil and –Platelet in the MEDALIST Trial: Multilineage Data from a Phase 3, Randomized, Double-Blind, Placebo- Controlled Study of Luspatercept to Treat Anemia in Patients with Very Low-, Low-, or Intermediate-Risk Myelodysplastic Syndromes (MDS) with Ring Sideroblasts (RS) Who Require Red Blood Cell (RBC) Transfusions

Presenter: Guillermo Garcia-Manero

Poster Presentation, Sunday, December 8, 6:00 – 8:00 p.m. EST

Session 112: Thalassemia and Globin Regulation: Poster II

Title: Effects of Luspatercept on Iron Overload and Impact on Responders to Luspatercept: Results from the BELIEVE Trial

Presenter: John B Porter

The clinical abstracts that contain additional trial details and results can be found on the ASH Annual Meeting website (<http://www.hematology.org/Annual-Meeting/Abstracts/>).

The luspatercept ASH presentations, which will include additional information beyond the abstracts, will be available in the “Science” section on Acceleron’s website, www.acceleronpharma.com, following the conference.

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

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 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.

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