



December 5, 2016

## Acceleron and Celgene Announce Updated Results from Phase 2 Studies of Luspatercept in Beta-Thalassemia Presented at the 58th Annual Meeting of the American Society of Hematology

*- Interim longer term data with investigational drug luspatercept show sustained increases in hemoglobin levels, and reduced transfusion burden in patients with beta-thalassemia -*

CAMBRIDGE, Mass. & SUMMIT, N.J.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ: XLRN) and Celgene Corporation (NASDAQ: CELG) today announced Phase 2 results from an open-label three-month base study and the ongoing long-term safety extension study with luspatercept in patients with beta-thalassemia during an oral presentation at the 58<sup>th</sup> Annual Meeting of the American Society of Hematology (ASH) in San Diego, California. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

"Beta-thalassemia is a severe, chronic disease with no pharmaceutical treatment options to correct or improve the underlying anemia in patients," said Michael Pehl, President, Hematology and Oncology for Celgene. "These longer term luspatercept Phase 2 data are encouraging, and we are continuing to enroll patients in the Phase 3 BELIEVE study in transfusion dependent beta-thalassemia patients."

### Luspatercept Beta-Thalassemia Data Presented at ASH

#### Results in Transfusion Dependent (TD) Beta-Thalassemia Patients

| RBC transfusion reduction over any 12 weeks versus 12 weeks pre-treatment | Response rate (% of patients) |                                  |
|---|-------------------------------|----------------------------------|
|   | 3-month base study (n=31)     | Long-term extension study (n=24) |
| ≥ 20%   | 81% (25/31)                   | 96% (23/24)                      |
| ≥ 33%   | 71% (22/31)                   | 83% (20/24)                      |
| ≥ 50%   | 55% (17/31)                   | 71% (17/24)                      |

#### Results in Non-Transfusion Dependent (NTD) Beta-Thalassemia Patients

| Hemoglobin (Hb) response over any 12 weeks versus 12 weeks pre-treatment | Response rate (% of patients) in patients treated with ≥ 0.6 mg/kg |                                  |
|--|--|----------------------------------|
|  | 3-month base study (n=21)  | Long-term extension study (n=27) |
| Increase in mean Hb ≥ 1.0 g/dL   | 62% (14/21)  | 78% (21/27)                      |
| Increase in mean Hb ≥ 1.5 g/dL   | 33% (7/21)   | 52% (14/27)                      |

In the long-term extension study, the median duration of a hemoglobin increase ≥ 1.0 g/dL maintained for at least 12 weeks in responders is 13.5 months (N=21) with treatment still ongoing.

#### Safety

- ┆ There were no related serious adverse events and related grade 3 adverse events included: bone pain (n=2 base, n=1 extension), asthenia (n=2 base) and headache (n=1 extension)
- ┆ The most common related adverse events (all grades) were bone pain, myalgia, headache, musculoskeletal pain, arthralgia, and injection site pain.

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

The BELIEVE Trial, a global Phase 3, double-blind, randomized, placebo-controlled, multicenter study in transfusion dependent beta-thalassemia patients, is currently enrolling.

The slides from the ASH beta-thalassemia presentation will be available immediately following the presentation at the conference on Acceleron's website ([www.acceleronpharma.com](http://www.acceleronpharma.com)) under the Science tab.

### **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 ( $\geq 4$  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  $\geq 1.5$  g/dL from baseline for  $\geq 14$  days (in the absence of RBC transfusions) in non-transfusion dependent patients, or 2)  $\geq 20\%$  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 (TGF-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 erythropoietin (EPO), which stimulates the proliferation of early-stage erythrocyte precursor cells. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Acceleron and Celgene are enrolling Phase 3 clinical trials that are designed 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](http://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 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](http://www.acceleronpharma.com). Follow Acceleron on Social Media: [@AcceleronPharma](https://twitter.com/AcceleronPharma) and [LinkedIn](https://www.linkedin.com/company/acceleron-pharma).

### **About Celgene**

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit [www.celgene.com](http://www.celgene.com). Follow Celgene on Social Media: [@Celgene](https://twitter.com/Celgene), [Pinterest](https://www.pinterest.com/celgene/), [LinkedIn](https://www.linkedin.com/company/celgene), [FaceBook](https://www.facebook.com/celgene) and [YouTube](https://www.youtube.com/c/celgene).

### **Forward-Looking Statements**

*Acceleron:*

*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 February 25, 2016, 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.

Celgene:

#### Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of, and plans relating to the collaboration between Acceleron and Celgene; the potential of luspatercept as a therapeutic drug; and the benefit of each company's strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs. For example, there can be no guarantee that any product candidate will be successfully developed or complete necessary preclinical and clinical phases, or that development of any of product candidates will successfully continue. There can be no guarantee that any positive developments will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including: results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; the ability to obtain and maintain requisite regulatory approvals and to enroll patients in planned clinical trials; unplanned cash requirements and expenditures; competitive factors; the ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates; the ability to maintain key collaborations; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in each company's public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and neither company has any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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