



December 4, 2016

Acceleron and Celgene Announce Updated Results from Ongoing Phase 2 Studies of Luspatercept in Myelodysplastic Syndromes at the 58th Annual Meeting of the American Society of Hematology

- Preliminary results show that treatment with investigational drug luspatercept increases hemoglobin and achieves durable transfusion independence in patients with lower risk myelodysplastic syndromes -

- Preliminary data in ESA naïve and RS- patients are encouraging -

- Acceleron to host conference call and live webcast on Monday, December 5th at 9:00 a.m. EST (6:00 a.m. PST) -

CAMBRIDGE, Mass. & SUMMIT, N.J.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN) and Celgene Corporation (NASDAQ:CELG), today announced preliminary Phase 2 results from the ongoing three-month base and long-term extension studies with investigational drug luspatercept in patients with lower risk myelodysplastic syndromes (MDS) at the 58th 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.

"We are encouraged by the additional luspatercept data from the ongoing Phase 2 studies," said Michael Pehl, President, Hematology and Oncology for Celgene. "These data further support luspatercept's potential in treating a broader spectrum of MDS patients. We are evaluating opportunities to expand our clinical program to include additional MDS patient populations, as we advance our Phase 3 MEDALIST trial in RS+ patients."

Luspatercept Phase 2 Data in First-line, ESA treatment-naïve MDS Patients

In lower-risk MDS patients who have not received prior treatment with an erythropoiesis-stimulating agent (ESA) and have erythropoietin (EPO) levels \leq 500 IU, luspatercept three-month base study data demonstrated encouraging rates of transfusion independence and International Working Group Hematologic Improvement - Erythroid (IWG HI-E) response criteria.

| Transfusion Burden | IWG HI-E, n/N (%) | | RBC-TI, n/N (%) | |
|-------------------------|-------------------|----------------|-----------------|----------------|
| | Base N=64 | Extension N=42 | Base N=49 | Extension N=28 |
| All | 12/20 (60%) | 13/16 (81%) | 9/12 (75%) | 8/10 (80%) |
| Low Transfusion Burden | 6/13 (46%) | 8/11 (73%) | 5/5 (100%) | 5/5 (100%) |
| High Transfusion Burden | 6/7 (86%) | 5/5 (100%) | 4/7 (57%) | 3/5 (60%) |

Luspatercept Phase 2 Data in Ring Sideroblast Positive (RS+) and Negative (RS-) in MDS Patients

- ▮ In patients with EPO levels < 200, response rates were similar in both RS+ and RS- patients
- ▮ In the patients with EPO levels \geq 200 to \leq 500, luspatercept response rates remained encouraging in those patients who are RS+

| Baseline EPO (U/L) | RS Status | IWG HI-E, n/N (%) | | RBC-TI, n/N (%) | |
|--------------------------|-----------|-------------------|-----------------|-----------------|-----------------|
| | | Base N=64* | Extension N=42* | Base N=49* | Extension N=28* |
| < 200 | RS+ | 18/29 (62%) | 19/23 (83%) | 13/19 (68%) | 10/14 (71%) |
| | RS- | 2/5 (40%) | 3/3 (100%) | 1/4 (25%) | 1/2 (50%) |
| \geq 200 to \leq 500 | RS+ | 5/11 (46%) | 7/8 (88%) | 3/9 (33%) | 3/5 (60%) |
| | RS- | 0/3 (0%) | 0/1 (0%) | 2/2 (100%) | 1/1 (100%) |

*Table includes both ESA refractory and ESA naïve patients. Subjects treated at dose levels ≥ 0.75 mg/kg.

Luspatercept Phase 2 Safety Data

- | The majority of adverse events (AEs) were grade 1 or 2
- | There were four grade 3/serious AEs possibly or probably related to study drug as of November 28, 2016: blast cell count increase, myalgia, worsening of general condition, progression to AML
- | Adverse events at least possibly related to study drug that occurred in at least 2 patients during studies were diarrhea, fatigue, headache, hypertension, arthralgia, bone pain, injection site erythema, myalgia and peripheral edema.

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

The MEDALIST Trial, a global Phase 3 study in patients with very low, low, or intermediate risk, MDS with ring sideroblasts who require red blood cell transfusions, is currently enrolling.

The poster presentation of the ongoing Phase 2 studies is available on Acceleron's website (www.acceleronpharma.com) under the Science tab.

Acceleron ASH Conference Call Information

Acceleron will host a conference call and live webcast to discuss data presented at the ASH meeting on December 5, 2016, at 9:00 a.m. EST (6:00 a.m. PST). To participate by teleconference, please dial 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the Acceleron ASH Review.

To access the live webcast, please select "Events & Presentations" in the Investors section on Acceleron's website (www.acceleronpharma.com) at least 10 minutes beforehand to ensure time for any downloads that may be required.

An archived webcast recording will be available on the Acceleron website beginning approximately two hours after the event.

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 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, high transfusion burden patients (≥ 4 units RBC / 8 weeks) and low transfusion burden patients (< 4 units RBC / 8 weeks) were enrolled and treated with open-label luspatercept, dosed subcutaneously once every three weeks.

The primary outcome measure for the three-month base study was the proportion of patients who had an erythroid response. Erythroid response was defined as hemoglobin ≥ 1.5 g/dL from baseline for ≥ 14 days in low-transfusion burden patients and for high-transfusion burden patients a reduction of either ≥ 4 units or $\geq 50\%$ of units of RBCs transfused while receiving luspatercept compared to the patient's pretreatment transfusion burden. 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.

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. Follow Acceleron on Social Media: [@AcceleronPharma](#) and [LinkedIn](#).

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. Follow Celgene on Social Media: [@Celgene](#), [Pinterest](#), [LinkedIn](#), [FaceBook](#) and [YouTube](#).

Forward-Looking Statements

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.

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 the Company 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.

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