June 10, 2016

Acceleron and Celgene Announce Updated Results from an Ongoing Phase 2 Study of Luspatercept in Myelodysplastic Syndromes at the 21st Congress of the European Hematology Association

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

- Acceleron to host conference call and live webcast today at 8:00 a.m. EDT (2:00 p.m. CEST) -

CAMBRIDGE, Mass. & SUMMIT, N.J.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN) and Celgene Corporation (NASDAQ: CELG), today announced preliminary results from an ongoing long-term Phase 2 extension study with luspatercept in patients with lower risk myelodysplastic syndromes (MDS) at the 21st Congress of the European Hematology Association (EHA) in Copenhagen, Denmark. Results highlighted in an oral presentation showed that 51% of patients with lower risk MDS treated with luspatercept (n=49) achieved increased hemoglobin levels and 35% of patients achieved transfusion independence in the 3-month base study. In the ongoing extension study, 81% (26/32) of patients had increased hemoglobin levels and of the patients eligible for transfusion independence (TI), 50% achieved TI with luspatercept treatment. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

"The results for luspatercept in lower risk MDS patients are increasingly encouraging as we gain longer term safety and efficacy experience with this agent," said Uwe Platzbecker, M.D., Professor of Hematology and Head of the MDS program at the University Hospital in Dresden, Germany. "There is a significant unmet need for new therapies that reduce the number of or eliminate the need for blood transfusions."

Highlights of the Luspatercept MDS Phase 2 Data Presented at EHA

Study Design

Data from two Phase 2 studies were presented at the conference: the completed dose-escalation study in which patients received treatment with luspatercept for three months and the ongoing long-term extension study in which patients receive treatment with luspatercept for an additional 24 months. In both the 3-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 3 weeks. The primary outcome measure for the 3 month 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 non-transfusion dependent patients or a reduction of either ≥ 4 units or ≥ 50% of units of RBCs transfused 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 with low or intermediate-1 risk MDS who were previously enrolled in the 3-month study.

Efficacy

	Response rate (% of patients)	
	3-month base study (n=49, higher dose levels)	**Long-term extension study** (n=32)
International Working Group Hematologic Improvement-Erythroid (IWG HI-E) Response Rate (reduction of ≥4 units RBC / 8 weeks or a hemoglobin increase ≥1.5 g/dL ≥ 8 weeks)	51% (25/49)	81% (26/32)
RBC Transfusion Independence (RBC-TI) Response Rate (Transfusion free ≥ 8 weeks for patients with ≥ 2 units RBC / 8 weeks prior to treatment)	35% (14/40)	50% (11/22)

Duration of RBC-TI	Range: 9-80+ weeks

For reference, results presented six months ago at the American Society of Hematology (ASH) annual meeting in December 2015 were as follows:

- IWG HI-E response rate was 69% (22/32)
- RBC-TI response rate was 50% (11/22)
- Duration of RBC-TI ranged from 9 to 50+ weeks

Safety

- There were three grade 3 adverse events possibly/probably related to study drug (blast cell count increase, myalgia and worsening of general condition).
- Adverse events at least possibly related to study drug that occurred in at least 2 patients during studies were fatigue, bone pain, diarrhea, myalgia, headache, hypertension and injection site erythema.

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 slides from this oral presentation are available on Acceleron's website (www.acceleronpharma.com) under the Science tab.

Acceleron EHA Conference Call Information

Acceleron will host a conference call and live webcast from EHA today at 8:00 a.m. EDT (2:00 p.m. CEST). To participate by teleconference, please dial 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the Acceleron EHA Congress Review.

To access the live webcast, please select "Events & Presentations" in the Investor 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 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 discovers and develops novel therapies to treat a wide range of 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 innovative approach to drug discovery has generated four therapeutic candidates currently in clinical trials. Acceleron'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

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.

Celgene:

This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. We undertake no obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond our control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, many of which are discussed in more detail in our Annual Report on Form 10-K and our other reports filed with the Securities and Exchange Commission.

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