



May 6, 2017

Luspatercept Phase 2 Data Presented at the 14th International Symposium on Myelodysplastic Syndromes

- Preliminary results show that treatment with investigational drug luspatercept achieves meaningful erythroid response rates and transfusion independence in first-line, lower-risk myelodysplastic syndromes patients -
- Celgene and Acceleron plan to initiate a Phase 3 trial in first-line, lower-risk MDS patients in early 2018 -
- Acceleron to review MDS Symposium presentation in conjunction with its first quarter earnings call on Monday, May 8th at 8:00 a.m. EDT -

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 14th International Symposium on MDS in Valencia, Spain. Luspatercept is being developed as part of the global collaboration between Acceleron and Celgene.

"These positive data presented in lower-risk MDS confirm our optimism in new opportunities for luspatercept beyond our ongoing Phase 3 trials," said Michael Pehl, President, Hematology and Oncology for Celgene. "We are now planning a Phase 3 clinical trial to expand the development of luspatercept into this lower-risk MDS patient population."

"There is a high unmet medical need for a drug to treat patients earlier in the MDS treatment paradigm," said Habib Dable, President and CEO of Acceleron. "We continue to be motivated to find additional opportunities for luspatercept to treat anemia due to rare blood disorders and remain on track to initiate Phase 2 trials in myelofibrosis and non-transfusion dependent beta-thalassemia by year-end."

Luspatercept Phase 2 Data in First-Line, Lower-Risk MDS Patients

In lower-risk, erythropoiesis-stimulating agent (ESA)-naïve MDS patients, 48% (11/23) of patients treated with luspatercept achieved red blood cell transfusion independence (RBC-TI) and 51% (20/39) of patients achieved a clinically meaningful erythroid hematological improvement (HI-E) response per the International Working Group's (IWG) criteria. The response rates were positive in patients treated with luspatercept in both ESA-naïve and prior ESA-treated patients.

	IWG HI-E, N=82 n (%)	RBC-TI, N=56 n (%)
ESA-Naïve	20/39 (51%)	11/23 (48%)
Prior ESA	22/43 (51%)	11/33 (33%)

Luspatercept Phase 2 Data in RS+ and RS- Lower-Risk MDS Patients

In patients with baseline erythropoietin (EPO) levels \leq 500 international units per liter (IU/L), RBC-TI and IWG HI-E response rates were positive in both ring sideroblast-positive (RS+) and -negative (RS-) patients.

Baseline EPO (IU/L)	RS Status	IWG HI-E, N=82 n (%)	RBC-TI, N=56 n (%)
\leq 500	RS+	30/46 (65%)	16/29 (55%)
	RS-	6/14 (43%)	4/7 (57%)
> 500	RS+	5/9 (56%)	2/9 (22%)
	RS-	1/11 (9%)	0/9 (0%)
	Unknown	0/2 (0%)	0/2 (0%)

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

Luspatercept Phase 2 Safety Data

The majority of adverse events (AEs) were grade 1 or 2. AEs at least possibly related to study drug that occurred in at least 3 patients during the studies were fatigue, headache, hypertension, diarrhea, arthralgia, bone pain, injection site erythema, myalgia, and edema peripheral. Grade 3 non-serious AEs possibly or probably related to study drug were ascites, blast cell count increase, blood bilirubin increase, hypertension, platelet count increase, and pleural effusion. Grade 3 serious AEs possibly or probably related to study drug were general physical health deterioration and myalgia.

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

The oral presentation given at the 14th International Symposium on MDS is available on Acceleron's website (www.acceleronpharma.com) under the Science tab.

Acceleron MDS Symposium Conference Call Information

Acceleron will host a conference call and live webcast to discuss data presented at the MDS Symposium and its first quarter operational and financial results on May 8, 2017, at 8:00 a.m. EDT. To participate by teleconference, please dial 877-312-5848 (domestic) or 253-237-1155 (international) and refer to the Acceleron Earnings Call.

To access the live webcast, please select "Events & Presentations" in the Investors/Media 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, lower-risk MDS patients were enrolled and treated with open-label luspatercept, dosed subcutaneously once every three weeks.

The outcome measures for the studies included the proportion of patients who had an erythroid response (IWG HI-E) or achieved RBC transfusion independence (RBC-TI). IWG HI-E was defined as hemoglobin increase ≥ 1.5 g/dL sustained for ≥ 8 weeks in patients with < 4 units RBC / 8 weeks transfusion burden at baseline and hemoglobin levels below 10 g/dL. For patients with a ≥ 4 units RBC / 8 weeks transfusion burden at baseline, erythroid response was defined as a reduction of ≥ 4 units RBC sustained for ≥ 8 weeks. RBC-TI was defined as no RBC transfusions for ≥ 8 weeks in patients with a ≥ 2 units RBC / 8 weeks baseline transfusion burden.

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 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 <http://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

Celgene and Acceleron:

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. Each company's 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; 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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