



## Celgene Corporation and Acceleron Pharma Announce Results of the Phase 3 MEDALIST Trial Evaluating Luspatercept in Patients with Myelodysplastic Syndromes at the ASH 2018 Plenary Session

December 2, 2018

*Pivotal phase 3 data demonstrated treatment with investigational luspatercept resulted in statistically significant increased red blood cell transfusion independence compared to placebo*

*Regulatory submissions planned in the United States and Europe in the first half of 2019*

SUMMIT, N.J. & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Dec. 2, 2018-- Celgene Corporation (NASDAQ: CELG) and Acceleron Pharma Inc. (NASDAQ: XLRN) today announced results from the pivotal, phase 3 MEDALIST trial evaluating the efficacy and safety of investigational luspatercept to treat patients with ring sideroblast (RS+) myelodysplastic syndromes (MDS)-associated anemia who require red blood cell transfusions and who had failed, were intolerant to, or ineligible for erythropoietin therapy. Results were presented by Alan F. List, M.D. during the Plenary Scientific Session at the 60<sup>th</sup> American Society of Hematology (ASH) Annual Meeting and Exposition in San Diego, C.A. (Abstract #1).

"Severe anemia resulting in red blood cell transfusion dependence is a significant challenge for patients with low- and intermediate-risk MDS. Those who become resistant or refractory to currently available treatments have limited alternatives," said Dr. List, President and CEO of Moffitt Cancer Center. "The findings from MEDALIST are very exciting as they support the hypothesis that targeting red blood cell precursor maturation could help to address patients' anemia and allow them to achieve transfusion independence."

MEDALIST met the primary endpoint of red blood cell transfusion independence (RBC-TI) for 8 or more weeks during the first 24 weeks of the study. Treatment with luspatercept resulted in a statistically significantly greater proportion of patients achieving RBC-TI  $\geq$  8 weeks compared to placebo. The study also found in secondary endpoints that treatment with luspatercept resulted in a statistically significant higher percentage of patients achieving RBC-TI of 12 or more weeks in the first 24 or 48 weeks of the study, as well as hematologic improvement-erythroid (HI-E) of 8 or more weeks.

Endpoints	Luspatercept	Placebo	P-Value
RBC-TI $\geq$ 8 weeks (weeks 1-24)	37.9 % (58/153)	13.2 % (10/76)	< 0.0001
RBC-TI $\geq$ 12 weeks (weeks 1-24)	28.1 % (43/153)	7.9 % (6/76)	0.0002
RBC-TI $\geq$ 12 weeks (weeks 1-48)	33.3 % (51/153)	11.8 % (9/76)	0.0003
HI-E $\geq$ 8 weeks (IWG 2006, weeks 1-24)	52.9 % (81/153)	11.8 % (9/76)	< 0.0001

### MEDALIST Safety Summary

Treatment-emergent adverse events (TEAEs) of Grade 3 or 4 were reported in 42.5% (65/153) of patients receiving luspatercept and 44.7% (34/76) of patients receiving placebo. Progression to acute myeloid leukemia (AML) occurred in four patients, three patients (2.0%) receiving luspatercept and one patient (1.3%) receiving placebo. Five patients receiving luspatercept (3.3%) and four patients receiving placebo (5.3%) experienced one or more TEAE that resulted in death.

### Most common TEAEs of any Grade in Greater than 10% of Patients in Either Arm

	Luspatercept		Placebo	
	N=153	%	N=76	%
Fatigue	26.8	%	13.2	%
Diarrhea	22.2	%	9.2	%
Asthenia	20.3	%	11.8	%
Nausea	20.3	%	7.9	%
Dizziness	19.6	%	5.3	%
Back pain	19.0	%	6.6	%

"The MEDALIST results demonstrate the potential clinical benefit of luspatercept in achieving red blood cell transfusion independence in patients with low-to-intermediate risk RS+ MDS, an area in need of new treatments," said Alise Reicin, MD, President, Global Clinical Development for Celgene. "Based on these results, we are encouraged that this first-in-class erythroid maturation agent may help these patients address the underlying cause of their disease-related chronic anemia."

"It's truly an honor to showcase the results from the MEDALIST trial as the first presentation of the ASH Plenary Session," said Habib Dable, President and Chief Executive Officer of Acceleron. "The results from the MEDALIST trial increase our confidence in the potential of luspatercept to provide a meaningful treatment option for patients suffering from lower-risk RS+ MDS worldwide. We're excited to continue our clinical development program in

MDS, beta-thalassemia, and myelofibrosis, while also exploring additional applications for luspatercept in a range of diseases associated with anemia.”

Luspatercept is not approved in any region for any indication. The companies are planning regulatory application submissions of luspatercept in the United States and Europe in the first half of 2019.

#### **About MEDALIST**

MEDALIST is a phase 3, randomized, double blind, placebo-controlled, multi-center study evaluating the safety and efficacy of luspatercept in patients with very low-, low-, or intermediate-risk non-del(5q) myelodysplastic syndromes (MDS). All patients were RBC transfusion dependent and were either refractory or intolerant to prior erythropoiesis-stimulating agent (ESA) therapy, or were ESA naïve with endogenous serum erythropoietin  $\geq$  200 U/L, and had no prior treatment with disease modifying agents. The median age of the patients enrolled in the trial was 71 years in the luspatercept treatment group and 72 years in the placebo group. Median transfusion burden in both treatment arms was 5 RBC units/8 weeks. 229 patients were randomized to receive either luspatercept 1.0 mg/kg (153 patients) or placebo (76 patients) via subcutaneous injection once every 21 days. The study was conducted at 65 sites in 11 countries.

#### **About Luspatercept**

Luspatercept is a first-in-class erythroid maturation agent (EMA) that is believed to regulate late-stage red blood cell maturation. Acceleron and Celgene are jointly developing luspatercept as part of a global collaboration. Phase 3 clinical trials continue to evaluate the safety and efficacy of luspatercept in patients with MDS (the MEDALIST trial) and in patients with beta-thalassemia (the BELIEVE trial). The COMMANDS phase 3 trial in first-line, lower-risk, MDS patients, the BEYOND phase 2 trial in non-transfusion-dependent beta-thalassemia, and a phase 2 trial in myelofibrosis are ongoing. For more information, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

#### **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).

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#### **About Acceleron**

Acceleron is a Cambridge-based, 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 franchise with two distinct Myostatin+ agents, ACE-083 and ACE-2494, and a Phase 2 pulmonary program with sotatercept in pulmonary arterial hypertension.

For more information, please visit [www.acceleronpharma.com](http://www.acceleronpharma.com). Follow Acceleron on Social Media: [@AcceleronPharma](#) and [LinkedIn](#).

#### **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," "will," "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, that the results of any clinical study will be predictive for other clinical studies of the same product candidate, 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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