



Acceleron, Celgene and Collaborators Announce Two Publications in Nature Medicine Describing Sotatercept and ACE-536 Therapeutic Potential in Beta-thalassemia and Myelodysplastic Syndromes

Two papers in the journal Nature Medicine describe role of TGF-beta superfamily signaling in red blood cell formation and the ability of sotatercept and ACE-536 to promote late-stage erythropoiesis through an EPO-independent mechanism

Summit, NJ and Cambridge, Mass. – March 24, 2014 (Business Wire) – Celgene Corporation (NASDAQ: CELG) and Acceleron Pharma Inc. (NASDAQ: XLRN), announced the publication of two papers available online today and in the April issue of the journal *Nature Medicine* that describe how sotatercept and ACE-536 promote red blood cell formation through an erythropoietin (EPO) independent mechanism in mice. EPO stimulates the proliferation of early-stage red blood cell precursors to form new red blood cells. However, anemias associated with defects in the late-stages of red blood cell formation, known as ineffective erythropoiesis, are resistant to EPO treatment. These studies suggest that sotatercept and ACE-536 may promote and regulate the maturation of late-stage red blood cell precursors and this distinct activity supports the rationale for sotatercept and ACE-536 as potential novel therapies to correct anemia, including the EPO-resistant anemia, in diseases such as beta-thalassemia and myelodysplastic syndromes.

The two papers provide evidence that at least one TGF-beta superfamily member, GDF-11, reduces the maturation of late-stage red blood cell precursors that can cause anemia. In these models, the mouse versions of sotatercept and ACE-536 block GDF-11, among other ligands, stimulating the maturation of red blood cell precursors and restoring the production of functional red blood cells in mouse models of myelodysplastic syndromes and beta-thalassemia.

“Collectively these two publications reveal a potentially exciting approach to treat a category of red blood cell disorders for which there is significant unmet medical need”, said Rajesh Chopra, M.D., Ph.D., Corporate Vice President Translational and Early Drug Development at Celgene. “We are actively pursuing a broad development program, including clinical trials in patients with MDS and beta-thalassemia, that leverage this understanding to treat patients with diseases that are generally resistant to treatment with existing agents.”

The two papers will be available on the Publications page of the Acceleron website (www.acceleronpharma.com):

- “TGF-beta superfamily ligand trap ACE-536 corrects anemia by promoting late-stage erythropoiesis” (doi: 10:1038.nm.3512) was authored by Acceleron scientist Rajashekar Suragani and colleagues at Acceleron and Celgene
- “An activin receptor IIA ligand trap corrects ineffective erythropoiesis in β -thalassemia” (doi: 10:1038.nm.3468) was authored by collaborators Michael Dussiot and colleagues at INSERM and scientists at Celgene

About Acceleron

Acceleron is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of novel protein therapeutics for cancer and rare diseases. The company is a leader in understanding the biology of the Transforming Growth Factor-Beta (TGF- β) protein superfamily, a large and diverse group of molecules that are key regulators in the growth and repair of tissues throughout the human body, and in targeting these pathways to develop important new medicines. Acceleron has built a highly productive R&D platform that has generated innovative clinical and preclinical protein therapeutic candidates with novel mechanisms of action. These protein therapeutic candidates have the potential to significantly improve clinical



outcomes for patients with cancer and rare diseases. For more information, please visit www.acceleronpharma.com.

About Celgene

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global pharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through gene and protein regulation. For more information, please visit www.celgene.com.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements about Acceleron's strategy, future plans and prospects, including statements regarding the development of Acceleron's compounds, including sotatercept and ACE-536 and Acceleron's TGF- β superfamily program generally, 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," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," 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 Acceleron's cash position will be insufficient to fund operations through the first half of 2017, that preclinical testing of Acceleron's compounds and preliminary 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 we expect it to be, that Acceleron or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of its compounds, that the development of Acceleron's compounds will take longer or cost more than planned, that Acceleron 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 Registration Statement on Form S-1 which was declared effective by the Securities and Exchange Commission (SEC) on January 22, 2014, and other filings that Acceleron 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.

Source: Acceleron Pharma

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