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## **Acceleron and Celgene Announce Interim Clinical Data for Sotatercept Demonstrating Dose Dependent Increases in Hemoglobin in Patients with End Stage Renal Disease on Hemodialysis**

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Acceleron Pharma Inc. (NASDAQ:XLRN), a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of novel protein therapeutics for cancer and rare diseases, today announced that its collaboration partner, Celgene Corporation, presented interim data demonstrating dose dependent increases in hemoglobin in patients with end stage renal disease (ESRD) on hemodialysis. The data are from an ongoing phase 2a study of sotatercept and were presented at the National Kidney Foundation 2014 Spring Clinical Meeting in Las Vegas, NV.

The phase 2a clinical trial of sotatercept is designed as a randomized, placebo-controlled, dose escalation study to evaluate sotatercept for the correction of anemia in patients with end stage renal disease on hemodialysis. There are four sotatercept dose groups (0.3 mg/kg, 0.5 mg/kg, 0.7 mg/kg, or 0.7 mg/kg loading dose followed by 0.4 mg/kg). Highlights of the data from the two lowest dose level cohorts (0.3 mg/kg and 0.5 mg/kg) are summarized below:

- Hemoglobin increase  $\geq 1.0$  g/dL was achieved by 20%, 37% and 40% of patients in the placebo, sotatercept 0.3 mg/kg and sotatercept 0.5 mg/kg cohorts, respectively, in the first 28-day cycle following a single dose.
- The mean peak hemoglobin increase in the first 28-day dose cycle was 0.1 g/dL, 0.5 g/dL and 0.8 g/dL in patients in the placebo, sotatercept 0.3 mg/kg and sotatercept 0.5 mg/kg cohorts, respectively.
- Rescue therapy with erythropoietin, if hemoglobin levels fell to less than 9 g/dL, was required in 40% of placebo patients, 13% of patients at sotatercept 0.3 mg/kg, and 0% of patients at sotatercept 0.5 mg/kg.

The poster (NKF Spring Clinical Meeting, poster #81) is available on the Publications page of the Acceleron website ([www.acceleronpharma.com](http://www.acceleronpharma.com)).

### **About Sotatercept Clinical Trials in Patients with End Stage Renal Disease**

The Phase 2a clinical trial of sotatercept is designed as a randomized, placebo-controlled single dose and multi-dose, dose escalation study to evaluate the pharmacokinetics, safety, efficacy, tolerability and pharmacodynamics of sotatercept for the correction of anemia in patients with end stage renal disease on hemodialysis. Eligible subjects are randomized to one of two arms (sotatercept or placebo in a 3:1 ratio) in 4 sequential dose groups (sotatercept 0.3 mg/kg, 0.5 mg/kg, 0.7 mg/kg, or 0.7 mg/kg loading dose followed by 0.4 mg/kg). Each dose group will include up to 12 patients. For additional information on this clinical trial, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov), identifier NCT01146574.

In December, 2013, Celgene initiated a phase 2b clinical trial of sotatercept in patients with end stage renal disease (ESRD) who are on hemodialysis. This phase 2 clinical trial is designed as a two-part study to assess the safety and efficacy of sotatercept as a therapy to treat anemia and potentially to control the adverse manifestations of chronic kidney disease mineral and bone disorder (CKD-MBD). Part one of the study will enroll up to 60 patients and part two of the study will enroll up to 230 patients. For additional information on this clinical trial, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov), identifier NCT01999582.

### **About Chronic Kidney Disease**

Chronic Kidney Disease (CKD) is a serious condition characterized by the progressive loss of kidney function and is most often caused by diabetes or high blood pressure. CKD is classified in five stages according to the degree of kidney impairment. End Stage Renal Disease (ESRD), also known as kidney failure, is the most advanced stage of CKD and a life-threatening condition in which patients require a either kidney transplant or dialysis for survival. Anemia, low levels of red blood cells, is a common secondary disorder to Chronic Kidney Disease and worsens with progression of the disease. Disturbance in mineral and bone metabolism, known as Chronic Kidney Disease Mineral and Bone Disorder (CKD-MBD), is also a common complication in CKD patients that affects almost all patients who are on dialysis. CKD-MBD can lead to bone abnormalities and/or calcification in soft tissue, especially blood vessels, and is associated with increased morbidity and mortality in CKD patients.

### **About Sotatercept**

Sotatercept is an activin receptor type IIA 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). Sotatercept 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 sotatercept as part of a global collaboration. Sotatercept is currently in multiple phase 2 clinical trials. For more information, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### **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- $\beta$ ) 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](http://www.acceleronpharma.com).

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