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Acceleron and Celgene Announce Interim Clinical Data for Sotatercept in Patients with End Stage Renal Disease on Hemodialysis at the 2014 National Kidney Foundation Spring Clinical Meeting

Sotatercept produces dose dependent increases in hemoglobin with no dose dependent changes in blood pressure

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, will present interim data from an ongoing phase 2a study of sotatercept in patients with end stage renal disease (ESRD) on hemodialysis at the National Kidney Foundation 2014 Spring Clinical Meeting. The meeting will take place on April 22-26, 2014 at the MGM Grand in Las Vegas, NV.

The data to be presented are from an ongoing phase 2a dose escalation study of sotatercept administered subcutaneously every 28 days to evaluate the pharmacokinetics, safety, tolerability, efficacy, and pharmacodynamics of sotatercept for the correction of anemia in patients with ESRD on hemodialysis. Dr. William Smith of Celgene Corporation, Acceleron's collaboration partner, will present interim safety, pharmacokinetic and hemoglobin data from the first 28-day dose cycle for two dose groups of sotatercept (0.3 mg/kg and 0.5 mg/kg) versus placebo. Following a single dose in the placebo, sotatercept 0.3 mg/kg and sotatercept 0.5 mg/kg dose groups, peak mean hemoglobin changes observed in the first 28 days were 0.1, 0.5 and 0.8 g/dL, respectively. Adverse events were mostly mild to moderate, unrelated to study drug, and generally consistent with patients' medical histories. No dose-dependent changes in blood pressure were observed.

The data will be presented in a poster presentation on Wednesday April 23rd from 6:00 - 7:30 PM PST.

About the Phase 2a Clinical Trial

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. The first dose group evaluated was a single dose of 0.1 mg/kg of sotatercept administered subcutaneously. Subsequent dose groups to be studied are 0.3, 0.5 and 0.7 mg/kg administered subcutaneously once every four weeks for up to eight cycles of treatment. Each dose group will include up to 12 patients randomized at a 3:1 ratio to receive either sotatercept or placebo. The trial is being conducted at sites in the United States and may enroll up to 56 patients. The primary endpoints are pharmacokinetics and safety. Celgene has completed enrollment in the 0.1, 0.3 and 0.5 mg/kg dose groups and is now enrolling patients in the 0.7 mg/kg dose group. Secondary endpoints in the study include effects on hemoglobin and serum markers of bone metabolism. For additional information on this clinical trial, please visit www.clinicaltrials.gov, identifier NCT01146574.

In December, 2013, Celgene initiated a second phase 2 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, 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-β) 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.

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.

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