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Akebia Completes Enrollment in the Two Planned Cohorts of its Phase 2 Study of AKB-6548 in Patients with Anemia Related to Chronic Kidney Disease Undergoing Dialysis

-- Enrollment Completed Ahead of Schedule --

-- Expanding Trial to Include Third Cohort, with Top-Line Data Still Expected in Third Quarter of 2015 --

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Akebia Therapeutics, Inc. (NASDAQ:AKBA), a biopharmaceutical company focused on delivering innovative therapies to patients with kidney disease through the biology of hypoxia inducible factor (HIF), today announced that it has completed enrollment ahead of schedule in the original two cohorts of its Phase 2 clinical study evaluating AKB-6548, a once-daily, oral therapy, in patients with anemia related to chronic kidney disease (CKD) who are undergoing dialysis. The company also announced plans to expand the study, adding an additional cohort designed to evaluate the safety, efficacy and tolerability of AKB-6548 dosed three times per week, administered in conjunction with a patient's hemodialysis schedule.

"The rate of enrollment in this Phase 2 trial reflects the enthusiasm surrounding AKB-6548 as a potential treatment for CKD patients undergoing dialysis who are suffering from anemia," stated Brad Maroni, M.D., Senior Vice President and Chief Medical Officer of Akebia. "Given the rapid enrollment of the first two cohorts, we now plan to expand the study and evaluate the potential to dose AKB-6548 three times weekly in this patient population, which may provide additional flexibility for use of AKB-6548 in hemodialysis treatment centers."

The Phase 2 multi-center, open-label study has already enrolled two cohorts, each consisting of 30 patients with anemia undergoing dialysis who were switched from injectable recombinant erythropoiesis-stimulating agent therapy to once-daily, oral doses of AKB-6548. The trial will now include an additional cohort of 30 patients who will receive AKB-6548 three times per week. The study will evaluate a total of 90 patients for 16 weeks of treatment, including an assessment of hemoglobin (HGB) response to AKB-6548 during an initial eight-week dosing period, followed by an assessment of HGB response to algorithm-guided dose adjustments of AKB-6548 during an additional eight weeks of treatment.

"Completing enrollment in the two cohorts ahead of schedule represents a significant achievement for the AKB-6548 program, and allows us to more fully explore the range of dosing options for renal anemia patients undergoing dialysis as we advance AKB-6548 toward commercialization," stated John P. Butler, President and Chief Executive Officer of Akebia. "Importantly, the expansion of the trial does not impact our timelines, and we remain on track to report top-line data in the third quarter of 2015."

About AKB-6548

AKB-6548 is a once-daily, oral therapy currently in development for the treatment of anemia related to CKD. AKB-6548 is designed to stabilize HIF, a transcription factor that regulates the expression of genes involved with red blood cell (RBC) production in response to changes in oxygen levels, by inhibiting the hypoxia-inducible factor prolyl hydroxylase (HIF-PH) enzyme. AKB-6548 exploits the same mechanism of action used by the body to naturally adapt to lower oxygen availability associated with a moderate increase in altitude. At higher altitudes, the body responds to lower oxygen availability with increased production of HIF, which coordinates the interdependent processes of iron mobilization and erythropoietin (EPO) production to increase RBC production and, ultimately, improve oxygen delivery.

As a HIF stabilizer with best-in-class potential, AKB-6548 raises hemoglobin levels predictably and sustainably, with a dosing regimen that allows for a gradual and controlled titration. AKB-6548 has been shown to improve iron mobilization, potentially eliminating the need for intravenous iron administration and reducing the overall need for iron supplementation.

About Anemia Related to CKD

Approximately 30 million people in the United States have CKD, with an estimated 1.8 million of these patients suffering from anemia. Anemia results from the body's inability to coordinate RBC production in response to lower oxygen levels due to the progressive loss of kidney function, which occurs in patients with CKD. Left untreated, anemia significantly accelerates patients' overall deterioration of health with increased morbidity and mortality. Renal anemia is currently treated with injectable recombinant erythropoiesis-stimulating agents, or rESAs, which are associated with inconsistent hemoglobin responses and well-documented safety risks.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a biopharmaceutical company headquartered in Cambridge, Massachusetts, focused on delivering innovative therapies to patients with kidney disease through HIF biology. Akebia's lead product candidate, AKB-6548, is a once-daily, oral therapy, which has completed a Phase 2b study for the treatment of anemia related to CKD in non-dialysis patients and is being tested in a Phase 2 study for the treatment of anemia in patients undergoing dialysis. For more information on Akebia, please visit www.akebia.com.

Forward-Looking Statement

This press release includes forward-looking statements. Such forward-looking statements include those about Akebia's strategy, future plans and prospects, including statements regarding the development plan for the Phase 2 study in dialysis patients with anemia related to CKD including plans for a third cohort in the study, the potential indications and benefits of AKB-6548, the expected timing of results from the Phase 2 study, and the potential of AKB-6548 to be a "best-in-class" product. 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, including the risk that existing preclinical and clinical data may not be predictive of the results of ongoing or later clinical trials; the ability of Akebia to successfully complete the clinical development of AKB-6548; the funding required to develop Akebia's product candidates and operate the company, and the actual expenses associated therewith; the timing and content of decisions made by the FDA and other regulatory authorities; the rate of enrollment in the Phase 2 study; the actual time it takes to complete the Phase 2 study and analyze the data; the success of competitors in developing product candidates for diseases for which Akebia is currently developing its product candidates; and Akebia's ability to obtain, maintain and enforce patent and other intellectual property protection for AKB-6548. Other risks and uncertainties include those identified under the heading "Risk Factors" in Akebia's Quarterly Report on Form 10-Q for the quarter ended September 30, 2014, and other filings that Akebia may make with the Securities and Exchange Commission in the future. Akebia does not undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this press release.

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