May 29, 2014

Akebia Therapeutics Announces Upcoming Presentation at 51st ERA-EDTA Annual Congress

Company to Present Data from Phase 2a Study of Lead Clinical Compound, AKB-6548, a Potentially Novel Approach to Treat Anemia Related to Chronic Kidney Disease

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Akebia Therapeutics, Inc. (NASDAQ:AKBA), a biopharmaceutical company focused on harnessing the potential of hypoxia-inducible factor (HIF) biology to develop and commercialize novel therapeutics to treat kidney disease and other serious diseases, today announced that data on its lead clinical compound, AKB-6548, will be presented at the 2014 European Renal Association-European Dialysis and Transplant Association (ERA-EDTA) 51st Congress being held in Amsterdam May 31 - June 3, 2014. AKB-6548 is a once-daily, oral hypoxia-inducible factor prolyl hydroxylase (HIF-PHD) inhibitor in development for the treatment of anemia related to chronic kidney disease (CKD).

The oral presentation entitled, "Controlled Hemoglobin Response in a Double-Blind, Placebo-Controlled Trial of AKB-6548 in Subjects with Chronic Kidney Disease," will take place on Sunday, June 1 during the free communication session entitled "CKD Anemia" starting at 5:00 p.m. local time, and will feature data from a Phase 2a clinical study of AKB-6548 for the treatment of anemia related to CKD in patients who are not dependent on dialysis.

Maintaining hemoglobin (HGB) levels within the target range is important for optimal clinical outcomes. In the previously presented double-blind, placebo-controlled Phase 2a trial, AKB-6548 effectively increased HGB levels in patients with CKD in a controlled manner while keeping the maximum HGB level below 13 g/dL throughout the dosing period. AKB-6548 was generally well-tolerated across the dosing groups with no drug-related serious adverse events.

"There is a clear need for new therapies to treat the increasing patient population impacted by anemia related to chronic kidney disease," said Robert Shalwitz, Chief Medical Officer of Akebia. "Current therapy with injectable recombinant erythropoiesis-stimulating agents, or rESAs, carries significant, well-documented safety risks and may be associated with excessive increases in hemoglobin levels. These Phase 2a data demonstrate the potential of AKB-6548 to offer a titratable oral dosing schedule that may provide a more gradual and predictable therapeutic option for increasing hemoglobin levels relative to rESAs."

AKB-6548 is currently in Phase 2b development for the treatment of anemia related to CKD in patients who are not dependent on dialysis, with results expected in the fourth quarter of 2014. A Phase 2 study of AKB-6548 in patients who are undergoing dialysis is slated to start mid-year 2014.

About the Phase 2a Study Design and Results

The Phase 2a double-blind, placebo-controlled study enrolled patients with CKD stage 3, 4 or 5, not on dialysis, with hemoglobin (HGB) < =10.5 g/dL. Patients were randomized to receive either placebo (n=19) or AKB-6548 (n=72) 240 mg, 370 mg, 500 mg or 630 mg once daily for six weeks. HGB was monitored at each study visit during dosing and was used to determine if the dose of study medication was to be reduced or discontinued. A one-time dose reduction was allowed during the study for any subject responding in excess of protocol-defined HGB values.

As previously presented, AKB-6548 increased total iron-binding capacity and increased HGB levels with a clear dose-response relationship. There was no observed negative impact on key biomarkers of kidney health, with no change from baseline in vascular endothelial growth factor (VEGF), C-reactive protein and Cystatin-C, and there were no drug-related serious adverse events.

About Anemia Related to Chronic Kidney Disease

Representing a growing global health concern, patients with chronic kidney disease progressively lose kidney function resulting in the development of several serious medical conditions including anemia. Left untreated, anemia significantly accelerates patients' overall deterioration of health with increased morbidity and mortality. Anemia results from the body's inability to coordinate red blood cell production in response to lower oxygen levels due to the progressive loss of kidney function.

About AKB-6548

AKB-6548 is a once-daily, oral therapy currently in development for the treatment of anemia related to chronic kidney disease

(CKD). AKB-6548 is designed to stabilize hypoxia-inducible factor (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-PHD) 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. Due to its specific HIF effect, AKB-6548 has the potential to restore coordination of the interdependent processes of iron mobilization and EPO production that are disrupted in patients with anemia related to CKD.

A HIF stabilizer with best-in-class potential, AKB-6548 may raise hemoglobin levels and RBC count predictably and sustainably, with an optimal safety profile and a dosing regimen that allows for a gradual and controlled titration. Furthermore, AKB-6548 may improve iron mobilization, potentially eliminating intravenous iron administration and reducing the overall need for iron supplementation.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a biopharmaceutical company headquartered in Cambridge, Massachusetts, focused on harnessing the potential of hypoxia-inducible factor (HIF) biology to develop and commercialize novel, proprietary therapeutics to treat kidney disease and other serious diseases. Akebia's lead clinical compound, AKB-6548, is a once-daily, oral therapy currently in Phase 2b clinical development for the treatment of anemia related to chronic kidney disease, a serious medical condition that leads to increased morbidity and mortality if left untreated. For more information on Akebia, please visit www.akebia.com.

Forward-Looking Statements

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 potential indications and benefits of AKB-6548 and the expected timing of the announcement of data from the Phase 2b study and the commencement of the Phase 2 dialysis study. 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 actual time it takes to complete clinical trials and analyze the data; the rate of enrollment in the Phase 2 dialysis study; the ability of Akebia to successfully complete the clinical development of AKB-6548 or any other product candidate; the funding required to develop Akebia's product candidates and operate the company, and the actual expenses associated therewith; the content of decisions made by the FDA and other regulatory authorities; 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 or any other product candidates. 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 March 31, 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.

Source: Akebia Therapeutics, Inc.

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