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Akebia Announces Dosing of First Patient in Phase 2 Study of AKB-6548 in Patients with Anemia Related to Chronic Kidney Disease Undergoing Dialysis

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Akebia Therapeutics, Inc. (NASDAQ:AKBA), a biopharmaceutical company focused on the development of novel, proprietary therapeutics based on hypoxia-inducible factor (HIF) biology and the commercialization of these products for patients with kidney disease, today announced that the first patient has been dosed in a Phase 2 clinical study of AKB-6548 in patients with anemia related to chronic kidney disease (CKD) who are undergoing dialysis. AKB-6548 is being developed as a once-daily, oral therapy with best-in-class potential for the treatment of anemia related to CKD.

"With this study, we are expanding our clinical experience with AKB-6548 beyond the non-dialysis CKD setting to include the treatment of anemia in patients with end-stage renal disease receiving dialysis," said Brad Maroni, M.D., Senior Vice President and Chief Medical Officer at Akebia. "Current therapy with injectable recombinant erythropoiesis-stimulating agents, or rESAs, carries well-documented safety risks and may be associated with excessive increases or fluctuations in hemoglobin levels. We look forward to evaluating the potential of AKB-6548 to treat anemia in patients undergoing dialysis while controlling hemoglobin levels in a safe and predictable manner."

The Phase 2 multi-center, open-label study will enroll approximately 60 patients with anemia related to CKD who are undergoing dialysis. The study will evaluate patients for a total of 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. Secondary objectives include pharmacodynamic response, safety, and tolerability of AKB-6548 in this patient population. Patients will receive AKB-6548 in place of their existing injectable rESA therapy. Data from this clinical study are expected in the third quarter of 2015.

"The initiation of this study is an important milestone in our development program for AKB-6548," said John P. Butler, President and Chief Executive Officer of Akebia. "We believe AKB-6548 holds best-in-class potential to address the urgent need for safer and more effective treatment options for patients undergoing dialysis who are trying to manage this difficult condition. We look forward to seeing the results of this study next year."

Akebia is also evaluating AKB-6548 in a Phase 2b trial for the treatment of anemia related to CKD in non-dialysis patients, with results expected in the fourth quarter of 2014.

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 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-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. 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 Anemia Related to CKD

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 RBC production in response to lower oxygen levels due to the progressive loss of kidney function.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a biopharmaceutical company headquartered in Cambridge, Massachusetts, focused on the development of novel, proprietary therapeutics based on HIF biology and the commercialization of these products for patients with kidney disease. Akebia's lead clinical program, AKB-6548, is a once-daily, oral therapy currently in Phase 2b clinical development for the treatment of anemia related to CKD in non-dialysis patients and in Phase 2 clinical development for the treatment of anemia related to CKD in patients undergoing dialysis, serious medical conditions that lead 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, the development plan for the Phase 2 study in dialysis patients with anemia related to CKD, and the expected timing of the announcement of data from the Phase 2 study and the Phase 2b 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 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 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 and Phase 2b studies 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 June 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.

Source: Akebia Therapeutics, Inc.

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