April 15, 2014

Akebia Therapeutics Announces Completion of Enrollment in its Phase 2b Clinical Study of AKB-6548

Phase 2b Results Expected in Q4 2014

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 it has completed enrollment in its ongoing 200-patient Phase 2b study of AKB-6548 for the treatment of anemia associated with chronic kidney disease (CKD) in patients who are not dependent on dialysis.

AKB-6548, a once-daily, oral investigational therapy, is designed to achieve a coordinated and natural increase in red blood cell production and iron utilization that is similar to the body's adaptive response to hypoxia, or low oxygen levels, resulting from modest increases in altitude. AKB-6548 acts by inhibiting the hypoxia-inducible factor prolyl hydroxylase (HIF-PH) enzyme, leading to stabilization and increased levels of HIF, the primary regulator of this response to hypoxia.

"We are on track to execute our strategy to develop a convenient, oral therapy with the potential to provide patients a predictable, meaningful and sustained improvement in their hemoglobin levels," said John P. Butler, President and Chief Executive Officer of Akebia. "The completion of enrollment in our Phase 2b study for AKB-6548 is an important milestone for Akebia, and we are working to develop a clinical data package that can position AKB-6548 as the best-in-class new therapy for the treatment of anemia secondary to CKD."

The Phase 2b randomized, double-blind, placebo-controlled study is designed to evaluate the safety and efficacy of AKB-6548 for the treatment of anemia associated with CKD in patients who are not on dialysis. There are 209 patients enrolled in the study at more than 50 sites across the United States. The primary purpose of this study is to demonstrate an adaptive approach to dosing AKB-6548 that will enable subjects to appropriately raise their hemoglobin levels from baseline without excessive excursions to greater than 13.0 g/dL.

"Anemia associated with CKD remains a serious and under-treated public health risk, associated with higher rates of morbidity and mortality," said Robert Shalwitz, Chief Medical Officer of Akebia. "The well-established risks associated with injectable erythropoiesis-stimulating agents have greatly curtailed their use, creating an urgent need for an alternative for combating anemia that can safely and significantly increase hemoglobin levels in CKD patients with anemia."

Anemia associated with CKD affects about 1.8 million people in the U.S. The current standard of care, injectable erythropoiesis-stimulation agents (ESAs), induces the body to produce red blood cells using supra-physiological levels of erythropoietin-receptor agonists usually in conjunction with supplemental administration of iron. AKB-6548, by contrast, has the potential to provide a more predictable and sustained level of improvement in hemoglobin levels while avoiding the rapid spikes in erythropoietin levels that are commonly seen with ESAs.

Akebia expects to announce results from the Phase 2b study 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 secondary to chronic kidney disease (CKD). AKB-6548 is designed to achieve a meaningful and sustained increase in red blood cell production and iron utilization that is similar to the body's natural adaptive response to hypoxia, or low oxygen levels, resulting from modest increases in altitude. AKB-6548 acts by inhibiting the hypoxia-inducible factor prolyl hydroxylase (HIF-PH) enzyme, leading to stabilization and increased levels of hypoxia-inducible factor (HIF), the primary regulator of this response to hypoxia. AKB-6548 is currently in Phase 2b development for the treatment of anemia secondary to CKD in patients who are not dependent on dialysis, and plans are underway to expand clinical development with a Phase 2 study in the dialysis population. AKB-6548 is being developed as an important alternative to injectable erythropoiesis-stimulating agents (ESAs), which are associated with a higher risk for increased mortality and adverse cardiovascular events, must be injected intravenously or subcutaneously, and often require supplemental administration of iron. As a potential best-in-class HIF-PH inhibitor, AKB-6548 is designed to improve hemoglobin levels, reduce the need for IV or oral iron supplementation and offer a differentiated safety profile with the convenience of once-daily, oral therapy.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is 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. Akebia's lead product candidate, AKB-6548, is in a Phase 2b clinical trial in patients with anemia secondary to chronic kidney disease who are not dependent on dialysis. AKB-6548 is being developed as a once-daily, oral therapy to inhibit hypoxia-inducible factor prolyl hydroxylase, which is expected to stabilize and increase levels of HIFα and improve the production of hemoglobin and red blood cells.

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 development plan for AKB-6548, the design of Akebia's clinical trials, the potential indications and benefits of AKB-6548, and the expected timing for the announcement of clinical trial data. 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 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; the content and timing of decisions 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 Registration Statement on Form S-1, which was declared effective by the Securities and Exchange Commission (SEC) on March 19, 2014, and other filings that Akebia may make with the SEC 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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