Phone: 513-985-1920
Fax: 513-985-0999

www.akebia.com

Akebia Therapeutics, Inc.
9987 Carver Road
Suite 420
Cincinnati, OH 45242

FOR IMMEDIATE RELEASE

AKEBIA MEETS PRIMARY ENDPOINT IN PHASE 2 STUDY OF ONCE-DAILY, ORAL ANEMIA TREATMENT, AKB-6548

Cincinnati, OH, April 5, 2012 – Akebia Therapeutics, Inc., a pharmaceutical discovery and development company focused on anemia and vascular disorders, today announced that it has successfully completed a randomized, double-blinded, placebo-controlled Phase 2 dose-ranging study of AKB-6548 in patients with stage 3 and 4 chronic kidney disease (CKD). AKB-6548 is an orally bioavailable hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor designed to increase the natural production of erythropoietin (EPO) and cause a controlled, gradual rise in hemoglobin in patients with anemia. The study met its primary endpoint of a dose-responsive increase in hemoglobin from baseline over the 42 days of the study ($p<0.0001$).

"Safe, effective, oral therapies for patients that suffer from anemia associated with chronic kidney disease represents a major unmet need. The results from this Phase 2 study demonstrate AKB-6548's ability to safely and effectively increase hemoglobin levels in a controlled manner, which recapitulates results seen in our prior pilot dose escalation study. We have also identified an optimal dose to take into a larger Phase 2b study, preparations for which are underway," said Dr. Robert Shalwitz, SVP and Chief Medical Officer of Akebia. "We believe AKB-6548 could offer patients a treatment option that mimics the body's natural response to hypoxia, including erythropoietin production in a highly controllable manner and a well-coordinated iron response. These are key attributes that are unavailable with today's standard of care."

The Phase 2 randomized, double-blind, placebo-controlled dose range finding study was designed to evaluate the safety, tolerability and pharmacokinetics of AKB-6548 in patients with stage 3 and 4 CKD. Subjects were randomized into 5 different dosing groups, and AKB-6548 was administered orally on an outpatient basis once daily for 42 days. The study enrolled 93 subjects at multiple sites in the United States.

The results show a highly significant, dose-responsive increase in hemoglobin and overall red blood cell production. Specifically:

- On average, patients that received the placebo experienced a small decline, 0.1 g/dL, in hemoglobin by week 6 of treatment
- Patients that received AKB-6548 experienced an increase from baseline to day 42 in hemoglobin, ranging from 0.7 to 1.4 g/dL

In addition, there was a parallel increase in iron mobilization, with significant dose-related reductions in ferritin and hepcidin, as well as an increase in total iron binding capacity (predominantly transferrin).

AKB-6548 was generally well tolerated, with no dose-related changes in adverse events, liver function or renal function as compared to placebo. Of particular note, there were no dose-related changes in vascular endothelial growth factor (VEGF) levels, a HIF responsive gene, at the end of 6 weeks of dosing. Together, these data strongly suggest that AKB-6548 could provide a highly efficacious and well-tolerated oral alternative to currently approved anemia treatments.

About HIF-PH
Hypoxia-inducible factors (HIFs) are transcription factors that regulate the body's response to decreases in oxygen, or hypoxia, in the cellular environment. HIF-PH's are the hypoxia-inducible factor prolyl hydroxylase enzymes that normally regulate the levels of HIF in bodily tissues. By inhibiting HIF-PH enzymes, HIFs can be stabilized or up-regulated, allowing the body to better respond to reduced oxygen, injury and infection. The ability to stabilize HIFs may lead to treatments for many conditions including anemia, fractures, wounds and other conditions where the HIF mechanism is not functioning optimally.

About AKB-6548
AKB-6548 is an orally bioavailable HIF-PH inhibitor designed to increase natural production of EPO, a glycoprotein hormone that controls red blood cell production, and causes a controlled rise in hemoglobin levels. Inadequate EPO production by the kidney is a common cause of anemia. Akebia will initially target pre-dialysis patients with chronic kidney disease, a large patient population that is currently undertreated for anemia. AKB-6548 potentially promises to be a safe, cost effective, orally dosed drug that delivers the efficacy of injectable EPO stimulating agents.

The market for chronic anemia drugs, which generated over $9 billion in worldwide sales in 2010, is dominated by injectable forms of recombinant EPO. There are currently no approved orally dosed small molecule drugs for the treatment of chronic anemia.

About Akebia Therapeutics
Akebia Therapeutics is a discovery and development company focused on anemia and vascular disorders. Akebia's lead program, AKB-6548, an orally bioavailable HIF-prolyl hydroxylase (HIF-PH) inhibitor for patients with anemia, is in Phase 2 clinical trials. AKB-6548 potentially promises to be a safer, less expensive, orally dosed pharmaceutical to stimulate endogenous EPO production.

Website: **www.akebia.com**.

CONTACT: Bill Daly, SVP of Business Development, Akebia Therapeutics, Inc.
513.985.1928, wdaly@akebia.com

Michelle Avery, MacDougall Biomedical Communications, 781.235.3060,
mavery@macbiocom.com