



Akebia Announces Establishment of Rare Kidney Disease Pipeline

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Acquires next generation tissue-targeted C3d-Factor H fusion protein complement inhibitor from Q32 Bio

Phase 2 basket trial planned to evaluate complement inhibitor in multiple rare kidney disease indications

Phase 2 trial of Praliciguat, an sGC stimulator, initiated in focal segmental glomerulosclerosis (FSGS)

Both trials planned to start treating subjects in 2026

CAMBRIDGE, Mass., Dec. 01, 2025 (GLOBE NEWSWIRE) -- [Akebia Therapeutics, Inc.](#) (Nasdaq: AKBA), a biopharmaceutical company with the purpose to better the lives of people impacted by kidney disease, today announced the establishment of its rare kidney disease pipeline. The pipeline is comprised of two core product candidates: ADX-097, a potential next generation complement inhibitor (now referred to as AKB-097) and praliciguat, a soluble guanylate cyclase (sGC) stimulator. Akebia acquired all rights to AKB-097, a tissue-targeted C3d-Factor H fusion protein complement inhibitor, from Q32 Bio Inc. (Nasdaq: QTTB) and believes AKB-097 has applicability across a wide range of complement-mediated rare kidney diseases. AKB-097 is targeted to the sites of complement activation in tissues and is not expected to result in systemic complement inhibition seen with other inhibitors. Separately, Akebia is evaluating praliciguat initially in a clinical trial in the treatment of FSGS, a rare kidney disease, and also plans to assess its use in other rare podocytopathies.

"Our commitment to patients with kidney disease is supported by two pillars of our corporate strategy: first, to drive Vafseo to become standard of care in anemia due to CKD in dialysis, and second, to build and progress our kidney disease pipeline," said John P. Butler, Chief Executive Officer of Akebia. "While our commercial and medical teams continue to build on the momentum of our Vafseo launch, we are excited to take an important step forward as a company with the establishment of our rare kidney disease development pipeline. We believe our differentiated complement inhibitor program can play a key role in addressing numerous rare kidney diseases, as can praliciguat, which we intend to initially study in FSGS. We look forward to enrolling patients in Phase 2 trials with each product candidate next year and expect to begin generating clinical data from an AKB-097 Phase 2 basket trial beginning in 2027."

About Complement Inhibition and AKB-097

The body's complement system, a series of proteins that work together as part of the immune system, can become dysregulated resulting in numerous inflammatory and autoimmune conditions, including multiple diseases of the kidney. Complement inhibitors work by binding to and preventing the activation of specific complement proteins, halting the cascade of inflammatory responses and reducing the destruction of cells.

AKB-097 is a humanized anti-C3d monoclonal antibody (mAb) fusion protein designed to act as a complement inhibitor through a tissue-targeted mechanism. AKB-097 has the potential to drive improved clinical activity by achieving therapeutic levels of inhibition and to address the limitations of the currently available systemic approaches to complement inhibition, including infection risk and the need for high drug doses and frequent administration.

In preclinical studies conducted by Q32 Bio, AKB-097 distributed to affected tissues/organs and demonstrated durable tissue pharmacokinetic (PK) and pharmacodynamic (PD) properties. AKB-097 was shown to be well-tolerated and demonstrated minimal anti-drug antibodies in a completed Phase 1 clinical trial in healthy volunteers. Akebia is planning to initiate an open label Phase 2 basket study in the second half of 2026 with initial data generation expected in 2027.

Financial Terms of Asset Purchase Agreement

On November 28, 2025, Akebia and Q32 Bio signed an Asset Purchase Agreement (APA) under which Akebia acquired global rights to Q32 Bio's ADX-097 (now referred to as AKB-097). In consideration, Akebia paid Q32 Bio an upfront payment of \$7.0 million. Akebia will also make a \$3.0 million payment upon the six-month anniversary of the closing, as well as additional development and regulatory milestones, commercial milestones and tiered royalties on annual net sales of AKB-097.

About Praliciguat and Planned Phase 2 Trial in FSGS

Praliciguat, is an oral soluble guanylate cyclase (sGC) licensed by Akebia from Cycleron Therapeutics, Inc. in June 2021. No significant safety issues were observed with praliciguat in Phase 1 studies in healthy volunteers and Phase 2 studies in heart failure and diabetic kidney disease. Praliciguat adverse events were infrequent and consistent with its known blood pressure lowering effect.

Akebia filed its Investigational New Drug Application with the U.S. Food and Drug Administration (FDA) and recently initiated a Phase 2 clinical trial in FSGS. The Phase 2 clinical trial is expected to enroll up to 60 patients at U.S. sites. The primary efficacy endpoint is change in urine protein-to-creatinine ratio between baseline and Week 24.

FSGS is a chronic disease characterized by scarring in some parts of the kidney's filtering units known as glomeruli. Left untreated, FSGS can cause various symptoms including high cholesterol, high blood pressure and kidney failure. FSGS is heterogenous with a wide range of causes and

symptoms among those impacted and has been diagnosed in approximately 40,000 people in the U.S. No treatments specifically indicated for FSGS are currently on the market and most patients are treated with antihypertensives and non-specific immunosuppressive therapies.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a fully integrated biopharmaceutical company with the purpose to better the lives of people impacted by kidney disease. Akebia was founded in 2007 and is headquartered in Cambridge, Massachusetts. For more information, please visit our website at www.akebia.com, which does not form a part of this release.

About Vafseo® (vadadustat) tablets

Vafseo® (vadadustat) tablets is a once-daily oral hypoxia-inducible factor prolyl hydroxylase inhibitor that activates the physiologic response to hypoxia to stimulate endogenous production of erythropoietin, increasing hemoglobin and red blood cell production to manage anemia. Vafseo is approved for use in 37 countries.

INDICATION

SAFEO is indicated for the treatment of anemia due to chronic kidney disease (CKD) in adults who have been receiving dialysis for at least three months.

Limitations of Use

- SAFEO has not been shown to improve quality of life, fatigue, or patient well-being.
- SAFEO is not indicated for use:
 - As a substitute for red blood cell transfusions in patients who require immediate correction of anemia.
 - In patients with anemia due to CKD not on dialysis.

IMPORTANT SAFETY INFORMATION about SAFEO (vadadustat) tablets

WARNING: INCREASED RISK OF DEATH, MYOCARDIAL INFARCTION, STROKE, VENOUS THROMBOEMBOLISM, and THROMBOSIS OF VASCULAR ACCESS.

SAFEO increases the risk of thrombotic vascular events, including major adverse cardiovascular events (MACE).

Targeting a hemoglobin level greater than 11 g/dL is expected to further increase the risk of death and arterial and venous thrombotic events, as occurs with erythropoietin stimulating agents (ESAs), which also increase erythropoietin levels.

No trial has identified a hemoglobin target level, dose of SAFEO, or dosing strategy that does not increase these risks.

Use the lowest dose of SAFEO sufficient to reduce the need for red blood cell transfusions.

CONTRAINDICATIONS

- Known hypersensitivity to SAFEO or any of its components
- Uncontrolled hypertension

WARNINGS AND PRECAUTIONS

- **Increased Risk of Death, Myocardial Infarction (MI), Stroke, Venous Thromboembolism, and Thrombosis of Vascular Access**

A rise in hemoglobin (Hb) levels greater than 1 g/dL over 2 weeks can increase these risks. Avoid in patients with a history of MI, cerebrovascular event, or acute coronary syndrome within the 3 months prior to starting SAFEO. Targeting a Hb level of greater than 11 g/dL is expected to further increase the risk of death and arterial and venous thrombotic events. Use the lowest effective dose to reduce the need for red blood cell (RBC) transfusions. Adhere to dosing and Hb monitoring recommendations to avoid excessive erythropoiesis.

- **Hepatotoxicity**

Hepatocellular injury attributed to SAFEO was reported in less than 1% of patients, including one severe case with jaundice. Elevated serum ALT, AST, and bilirubin levels were observed in 1.8%, 1.8%, and 0.3% of CKD patients treated with SAFEO, respectively. Measure ALT, AST, and bilirubin before treatment and monthly for the first 6 months, then as clinically indicated. Discontinue SAFEO if ALT or AST is persistently elevated or accompanied by elevated bilirubin. Not recommended in patients with cirrhosis or active, acute liver disease.

- **Hypertension**

Worsening of hypertension was reported in 14% of SAFEO and 17% of darbepoetin alfa patients. Serious worsening of hypertension was reported in 2.7% of SAFEO and 3% of darbepoetin alfa patients. Cases of hypertensive crisis, including hypertensive encephalopathy and seizures, have also been reported in patients receiving SAFEO. Monitor blood pressure. Adjust anti-hypertensive therapy as needed.

- **Seizures**

Seizures occurred in 1.6% of SAFEO and 1.6% of darbepoetin alfa patients. Monitor for new-onset seizures, premonitory symptoms, or change in seizure frequency.

- **Gastrointestinal (GI) Erosion**

Gastric or esophageal erosions occurred in 6.4% of VAFSEO and 5.3% of darbepoetin alfa patients. Serious GI erosions, including GI bleeding and the need for RBC transfusions, were reported in 3.4% of VAFSEO and 3.3% of darbepoetin alfa patients. Consider this risk in patients at increased risk of GI erosion. Advise patients about signs of erosions and GI bleeding and urge them to seek prompt medical care if present.

- **Serious Adverse Reactions in Patients with Anemia Due to CKD and Not on Dialysis**

The safety of VAFSEO has not been established for the treatment of anemia due to CKD in adults not on dialysis and its use is not recommended in this setting. In large clinical trials in adults with anemia of CKD who were not on dialysis, an increased risk of mortality, stroke, MI, serious acute kidney injury, serious hepatic injury, and serious GI erosions was observed in patients treated with VAFSEO compared to darbepoetin alfa.

- **Malignancy**

VAFSEO has not been studied and is not recommended in patients with active malignancies. Malignancies were observed in 2.2% of VAFSEO and 3.0% of darbepoetin alfa patients. No evidence of increased carcinogenicity was observed in animal studies.

ADVERSE REACTIONS

- The most common adverse reactions (occurring at $\geq 10\%$) were hypertension and diarrhea.

DRUG INTERACTIONS

- **Iron supplements and iron-containing phosphate binders:** Administer VAFSEO at least 1 hour before products containing iron.
- **Non-iron-containing phosphate binders:** Administer VAFSEO at least 1 hour before or 2 hours after non-iron-containing phosphate binders.
- **BCRP substrates:** Monitor for signs of substrate adverse reactions and consider dose reduction.
- **Statins:** Monitor for statin-related adverse reactions. Limit the daily dose of simvastatin to 20 mg and rosuvastatin to 5 mg.

USE IN SPECIFIC POPULATIONS

- **Pregnancy:** May cause fetal harm.
- **Lactation:** Breastfeeding not recommended until two days after the final dose.
- **Hepatic Impairment:** Not recommended in patients with cirrhosis or active, acute liver disease.

Please note that this information is not comprehensive. Please click [here](#) for the Full Prescribing Information, including BOXED WARNING and Medication Guide.

Forward-Looking Statements

Statements in this press release regarding Akebia Therapeutics, Inc.'s ("Akebia's") strategy, plans, prospects, expectations, beliefs, intentions and goals are forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, as amended, and include, but are not limited to, statements regarding: Akebia's plans and expectations with respect to the establishment of its rare kidney disease development pipeline, including AKB-097 and praliguat; Akebia's beliefs that AKB-097 has applicability across a wide range of complement-mediated rare kidney diseases; Akebia's beliefs and expectations that AKB-097 is targeted to the sites of complement activation in tissues and is not expected to result in systemic complement inhibition seen with other inhibitors; Akebia's plans with respect to praliguat, including its use in a clinical trial in the treatment of FSGS and the expected timing of enrollment and number of patients therein, its potential use in other rare podocytopathies and the potential market size for FSGS; Akebia's plans and expectations about its corporate strategy, including to drive Vafseo to become the standard of care in anemia due to CKD in dialysis, and to build and progress its kidney disease pipeline; Akebia's plans to continue to build on the momentum of its Vafseo launch; Akebia's beliefs that AKB-097's differentiated complement inhibitor program can play a key role in addressing numerous rare kidney diseases, as can praliguat; and Akebia's plans and expectations with respect to the initiation of a Phase 2 basket trial to evaluate AKB-097 in multiple rare kidney disease indications, including the timing of patient enrollment and initial data generation.

The terms "intend," "believe," "plan," "goal," "potential," "anticipate," "estimate," "expect," "future," "will," "continue," derivatives of these words, and similar references are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results, performance or experience may differ materially from those expressed or implied by any forward-looking statement as a result of various risks, uncertainties and other factors, including, but not limited to, risks associated with: the potential therapeutic benefits, safety profile, and effectiveness of Vafseo and Akebia's development candidates; the results of preclinical and clinical research; Akebia's ability to enroll patients in its clinical trials; decisions made by health authorities, such as the FDA, with respect to regulatory filings and other interactions; the potential demand and market potential and acceptance of, as well as coverage and reimbursement related to, Vafseo, including estimates regarding the potential market opportunity; the competitive landscape for Vafseo, including generic entrants and the timing thereof; the ability of Akebia to attract and retain qualified personnel; Akebia's ability to achieve and maintain profitability and to maintain operating expenses consistent with its operating plan; manufacturing, supply chain and quality matters and any recalls, write-downs, impairments or other related consequences or potential consequences; early termination of any of Akebia's collaborations; and changes in the geopolitical environment and uncertainty surrounding U.S. trade policy on tariffs. 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, 2025, and other filings that Akebia may make with the U.S. Securities and Exchange Commission in the future. These forward-looking statements (except as otherwise noted) speak only as of the date of this press release, and, except as required by law, Akebia does not

undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this press release.

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