Akebia Therapeutics Receives Complete Response Letter from the FDA for Vadadustat for the Treatment of Anemia due to Chronic Kidney Disease in Adult Patients

March 30, 2022

Company to host conference call on Wednesday, March 30, 2022 at 6:00 p.m. ET

CAMBRIDGE, Mass., March 30, 2022 /PRNewswire/ -- Akebia Therapeutics®, Inc. (Nasdaq: AKBA), a biopharmaceutical company with the purpose to better the lives of people impacted by kidney disease, today announced that the U.S. Food and Drug Administration (FDA) has issued a complete response letter (CRL) to Akebia's New Drug Application (NDA) for vadadustat, an investigational oral hypoxia-inducible factor prolyl hydroxylase (HIF-PH) inhibitor under review for the treatment of anemia due to chronic kidney disease (CKD). The FDA issues a CRL to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form.

The FDA concluded that the data in the NDA do not support a favorable benefit-risk assessment of vadadustat for dialysis and non-dialysis patients. The FDA expressed safety concerns noting failure to meet non-inferiority in MACE in the non-dialysis patient population, the increased risk of thromboembolic events, driven by vascular access thrombosis in dialysis patients, and the risk of drug-induced liver injury. The CRL stated that Akebia could explore ways to potentially demonstrate a favorable benefit-risk assessment through new clinical trials. Akebia will discuss the details of the CRL with its collaboration partners and request a meeting with the FDA.

"We are extremely disappointed to receive a CRL for vadadustat, a therapy that has the potential to help patients with anemia due to CKD. We continue to believe the data are supportive of a positive benefit-risk assessment of vadadustat for patients with anemia due to CKD, particularly in dialysis patients," said John P. Butler, Chief Executive Officer of Akebia. "Despite this setback, we continue to work toward our purpose to better the lives of people impacted by kidney disease."

In October 2021, Akebia's collaboration partner, Otsuka Pharmaceutical Co., Ltd. (Otsuka), submitted an initial marketing authorization application (MAA) for vadadustat to the European Medicines Agency for vadadustat, for the treatment of anemia due to CKD in adults; the review is ongoing. In Japan, vadadustat is approved as a treatment for anemia due to CKD in both dialysis-dependent and non-dialysis dependent adult patients.

Conference Call
Akebia will host a conference call Wednesday, March 30 at 6:00 p.m. Eastern Time to discuss the CRL and next steps.

To listen to the conference call, please dial (877) 458-0977 (domestic) or (484) 653-6724 (international) using conference ID number 7961118. The call will also be webcast LIVE and can be accessed via the Investors section of Akebia's website at http://ir.akebia.com.

An online archive of the conference call can be accessed via the Investors section of Akebia's website at http://ir.akebia.com. A replay of the conference call will be available two hours after the completion of the call through April 6, 2022. To access the replay, dial (855) 859-2056 (domestic) or (404) 537-3406 (international) and reference conference ID number 7961118.

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 Anemia due to Chronic Kidney Disease (CKD)
Anemia is a condition in which a person lacks enough healthy red blood cells to carry adequate oxygen to the body's tissues. It commonly occurs in people with CKD because their kidneys do not produce enough erythropoietin (EPO), a hormone that helps regulate production of red blood cells. Anemia due to CKD can have a profound impact on a person's quality of life as it can cause fatigue, dizziness, shortness of breath and cognitive dysfunction. Left untreated, anemia leads to deterioration in health and is associated with increased morbidity and mortality in people with CKD.

About Vadadustat
Vadadustat is an oral hypoxia-inducible factor prolyl hydroxylase inhibitor designed to mimic the physiologic effect of altitude on oxygen availability. At higher altitudes, the body responds to lower oxygen availability with stabilization of hypoxia-inducible factor, which can lead to increased red blood cell production and improved oxygen delivery to tissues. Vadadustat is an investigational new drug and is not approved by the U.S. Food and Drug Administration (FDA). On March 29, 2022, the FDA issued a complete response letter to Akebia's New Drug Application for vadadustat for the treatment of anemia due to chronic kidney disease (CKD). Vadadustat is currently under review by the European Medicines Agency for the treatment of anemia due to CKD in adults. In Japan, vadadustat is approved as a treatment for anemia due to CKD in both dialysis-dependent and non-dialysis dependent adult patients.

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, strategies and prospects for its business, including with respect to planned interactions and communications with its collaboration partners and the U.S. Food and Drug Administration (FDA); vadadustat's potential to help patients with anemia due to CKD and Akebia's beliefs about the benefit-risk assessment of vadadustat. The terms "believe," "expect," "potential," "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: decisions made by health authorities, such as the FDA and the European Medicines Agency, with respect to regulatory filings, including the New Drug Application for vadadustat; the potential therapeutic benefits, safety profile, and effectiveness of vadadustat; the potential indications, demand, potential and acceptance of, and Akebia's estimates regarding the potential market opportunity for Akebia's product candidate, vadadustat, if approved, or any other products or product candidates and the size of eligible patient populations; the direct or indirect impact of the COVID-19 pandemic on regulators and Akebia's business, operations, and the markets and communities in which Akebia and its partners, collaborators, vendors and customers operate; 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, including the amended and restated license agreement; and Akebia's and Vifor Pharma's ability to satisfy their obligations under the amended and restated license agreement; the competitive landscape for vadadustat, if approved. Other risks and uncertainties include those identified under the heading "Risk Factors in Akebia's Annual Report on Form 10-K for the year ended December 31, 2021, 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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