Akebia Announces Positive 52-week Efficacy and Safety Data for Vadadustat from Two Pivotal Phase 3 Studies in Japanese Patients with Anemia Due to Chronic Kidney Disease

November 9, 2019

- *Each Study Met Primary Endpoint at 24-Weeks and Showed Vadadustat's Effect on Hemoglobin Was Sustained Through to 52-Weeks*
- *Data Presented at the American Society of Nephrology (ASN) Kidney Week 2019 by Mitsubishi Tanabe Pharma Corporation (MTPC)*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 9, 2019-- Akebia Therapeutics, Inc. (Nasdaq: AKBA), a biopharmaceutical company focused on the development and commercialization of therapeutics for people living with kidney disease, today announced that MTPC, Akebia's development and commercialization collaboration partner in Japan for vadadustat, presented positive 24-week and 52-week data from two Phase 3 active-controlled pivotal studies evaluating the efficacy and safety of vadadustat, an investigational oral hypoxia-inducible factor prolyl hydroxylase inhibitor (HIF-PHI), in Japanese patients with anemia due to chronic kidney disease (CKD), at ASN Kidney Week 2019. Each study met its primary endpoint based on mean hemoglobin level at weeks 20 and 24, and showed vadadustat's effect on hemoglobin was sustained through to 52 weeks in each study.

In July, MTPC submitted a Japanese New Drug Application (JNDA) to the Ministry of Health, Labor and Welfare in Japan for marketing approval of vadadustat as a treatment for anemia due to CKD. The JNDA is the first regulatory submission for marketing approval of vadadustat and, if approved, is expected to lead to the first launch of vadadustat worldwide, next year. Details regarding these study results are provided below.

"Correcting and maintaining hemoglobin levels within a target range is paramount in the treatment of anemia due to chronic kidney disease," said John P. Butler, President and Chief Executive Officer of Akebia. "The 52-week data from MTPC's studies reinforce our belief in vadadustat's potential to make a difference in the lives of people impacted by anemia due to CKD."

Top-Line Results from Pivotal Phase 3 Study in Non-Dialysis Dependent CKD Subjects (J01 Study)

MTPC's phase 3 randomized, open-label, active-controlled correction and conversion study assessed the efficacy and safety of vadadustat compared to darbepoetin alfa, an erythropoiesis stimulating agent (ESA), in 304 Japanese non-dialysis dependent subjects with anemia due to CKD, with a treatment duration of 52 weeks. Data from the planned analysis at 24 weeks were announced in March 2019 and showed that the study met its primary endpoint: The difference in mean hemoglobin (Hb) was -0.26 g/dL (95% CI -0.50, -0.02 g/dL), achieving the pre-specified non-inferiority criterion of -0.75 g/dL. The mean Hb level at week 20 and week 24 were 11.66 g/dL (95% CI 11.49, 11.84 g/dL) for vadadustat-treated subjects compared to 11.93 g/dL (95% CI 11.76, 12.10 g/dL) for darbepoetin alfa-treated subjects. Data from the planned analysis at 52 weeks are provided here. The mean Hb level at week 52 was 11.51 g/dL (95% CI 11.35, 11.67 g/dL) for vadadustat-treated subjects compared to 11.58 g/dL (95% CI 11.43, 11.74 g/dL) for darbepoetin alfa-treated subjects. The incidence of adverse events (AEs) was 90.1% in the vadadustat-treated group compared to 92.2% in the darbepoetin alfa-treated group. The top three most common AEs reported in vadadustat-treated subjects were nasopharyngitis (24.5%), diarrhea (11.9%), and constipation (9.3%). The incidence of serious adverse events (SAEs) was 27.8% in the vadadustat-treated group compared to 32.0% in the darbepoetin alfa-treated group; no SAE was considered related to study drug. No deaths were reported in the vadadustat-treated group.

Top-Line Results from the Pivotal Phase 3 Study in Dialysis-Dependent CKD Subjects (J03 Study)

MTPC's phase 3 randomized, double-blind, active-controlled conversion study assessed the efficacy and safety of vadadustat compared to darbepoetin alfa in 323 Japanese hemodialysis subjects with anemia due to CKD who had been receiving ESA therapy prior to study screening, with a treatment duration of 52 weeks. Data from the planned analysis at 24 weeks were announced in March 2019 and showed that the study met its primary endpoint: The difference in mean Hb was -0.05 g/dL (95% CI -0.26, 0.17 g/dL), achieving the pre-specified non-inferiority criterion of -0.75 g/dL. The mean Hb level at week 20 and week 24 at 10.61 g/dL (95% CI 10.45, 10.76 g/dL) for vadadustat-treated subjects compared to 10.65 g/dL (95% CI 10.50, 10.80 g/dL) for darbepoetin alfa-treated subjects. The mean Hb level at week 52 was 10.39 g/dL (95% CI 10.24, 10.54 g/dL) for vadadustat-treated subjects compared to 10.62 g/dL (95% CI 10.48, 10.76 g/dL) for darbepoetin alfa-treated subjects. The incidence of AEs was 95.1% in the vadadustat-treated group compared to 98.1% in the darbepoetin alfa-treated group. The top three most common AEs reported in vadadustat-treated subjects were nasopharyngitis (45.7%), diarrhea (15.4%), and shunt stenosis (14.2%). The incidence of SAEs was 25.3% in the vadadustat-treated group compared to 27.3% in the darbepoetin alfa-treated group; no SAE was considered related to study drug. There were two deaths reported in the vadadustat-treated group and one death in the darbepoetin alfa-treated group and all three were assessed as not related to either vadadustat or darbepoetin.

In Japan, an estimated 13 million people have advanced stages of CKD. Anemia is common in patients with CKD and its prevalence increases as CKD progresses. Injectable ESAs are currently the standard of care. Vadadustat, if approved for marketing in Japan, would provide patients with a once-daily oral treatment option and has the potential to set a new oral standard of care for the treatment of anemia due to CKD.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a fully integrated biopharmaceutical company focused on the development and commercialization of therapeutics for

people living with kidney disease. The Company 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 Vadadustat

Vadadustat is an oral hypoxia-inducible factor prolyl hydroxylase (HIF-PH) inhibitor currently in global Phase 3 development for the treatment of anemia due to CKD. Vadadustat is 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 therapy and is not approved by the U.S. Food and Drug Administration (FDA) or any regulatory authority.

Forward-Looking Statements

Statements in this press release regarding 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, including but not limited to statements regarding the safety and efficacy of vadadustat, the potential launch of vadadustat, the potential indications for and benefits of vadadustat, and market size, commercial potential, prevalence, and the growth in, and potential demand for, vadadustat. The terms "anticipate," "expect," "potential," "will" and similar references 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 timing and content of decisions made by regulatory authorities; manufacturing risks; the quality and manner of the data that will result from clinical studies of vadadustat; the risk that clinical studies are discontinued or delayed for any reason, including for safety, tolerability, enrollment, manufacturing or economic reasons; early termination of any of Akebia's collaborations; Akebia's and its collaborators' ability to satisfy their obligations under Akebia's collaboration agreements; the competitive landscape for vadadustat; the scope, timing, and outcome of any legal, regulatory and administrative proceedings; changes in the economic and financial conditions of the businesses of Akebia and its partners; and Akebia's ability to obtain, maintain and enforce patent and other intellectual property protection for vadadustat. Other risks and uncertainties include those identified under the heading "Risk Factors" in Akebia's Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2019 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 Akebia does not undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this press release.

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Source: Akebia Therapeutics, Inc.

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