Akebia Therapeutics Announces Full Enrollment of its Global Phase 3 Program of Vadadustat for the Treatment of Anemia Due to Chronic Kidney Disease

September 3, 2019

INNO$_2$VATE and PRO$_2$TECT now fully enrolled; Company continues to expect to report top-line study results in Q2 of 2020 and mid-2020, respectively, subject to accrual of MACE

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 3, 2019-- Akebia Therapeutics, Inc. (Nasdaq: AKBA) today announced full enrollment of its global Phase 3 program evaluating the safety and efficacy of vadadustat for the treatment of anemia due to chronic kidney disease (CKD). Vadadustat is an investigational oral hypoxia-inducible factor prolyl hydroxylase inhibitor (HIF-PHI) in global Phase 3 development for the treatment of anemia due to CKD in dialysis dependent (DD)-CKD patients and non-dialysis dependent (NDD)-CKD patients. The Company's global Phase 3 vadadustat program consists of the INNO$_2$VATE studies which were fully enrolled in April 2019 with 3,923 DD-CKD patients, and the PRO$_2$TECT studies which are now fully enrolled with 3,513 NDD-CKD patients.

The INNO$_2$VATE and PRO$_2$TECT studies are large, randomized, open-label, non-inferiority design studies that are assessing the safety and efficacy of vadadustat versus an active comparator, darbepoetin alfa, an injectable erythropoiesis-stimulating agent (ESA). The primary efficacy endpoint of these studies is a change in hemoglobin (Hb) from baseline, and the primary safety endpoint is an assessment of cardiovascular safety as measured by major adverse cardiovascular events (MACE), defined as all-cause mortality, non-fatal myocardial infarction and non-fatal stroke. Other safety endpoints being assessed include individual MACE components, thromboembolic events, hospitalization for heart failure, and Hb excursions. Other efficacy endpoints being assessed include time in range (Hb), intravenous iron use and CKD progression. The Company continues to expect to report top-line results from INNO$_2$VATE in Q2 2020 andPRO$_2$TECT in mid-2020, subject to the accrual of MACE.

"We have tremendous confidence in the global Phase 3 program that we've designed for vadadustat and believe we are well positioned for clinical, regulatory and commercial success. Importantly, our program includes multiple secondary efficacy and safety endpoints to assess both clinically and commercially important areas of differentiation between vadadustat and the current standard of care, ESAs," said John P. Butler, President and Chief Executive Officer of Akebia. "If successful with our program, we expect vadadustat to be the first HIF-PHI in the U.S. and EU markets with data directly comparing its outcomes to the current standard of care in both DD and NDD patients. We believe these data will be extremely relevant for physicians, patients and payers as they make important care decisions."

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 inhibitor currently in global Phase 3 development for the treatment of anemia due to CKD. Vadadustat's proposed mechanism of action is designed to mimic the physiologic effect of altitude on oxygen availability. At higher altitudes, the body responds to lower oxygen availability with increased production of hypoxia-inducible factor, which coordinates the interdependent processes of iron mobilization and erythropoietin production to increase red blood cell production and, ultimately, improve oxygen delivery. Vadadustat is an investigational therapy and is not approved by the 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 the anticipated timing of the availability and reporting of clinical trial data and results; statements regarding Akebia's positioning of vadadustat for clinical, regulatory and commercial success; and statements regarding the potential benefits of Akebia's clinical trial designs for vadadustat, including the potential benefits of data comparing vadadustat outcomes with the current standard of care for treatment of anemia due to CKD in DD and NDD patients. The terms "believe," "expect" 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 MACE rate in our global phase 3 clinical trials for vadadustat; the risk that clinical trials may not be successful; the risk that existing preclinical and clinical data may not be predictive of the results of ongoing or later clinical trials; manufacturing risks; the quality and manner of the data that will result from clinical studies of vadadustat; risks associated with management and key personnel changes and transitional periods; the actual funding required to develop and commercialize Akebia's product candidates and operate the Company, and the actual expenses associated therewith; the actual costs incurred in the clinical studies of vadadustat and the availability of financing to cover such costs; 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 timing and content of decisions made by regulatory authorities; the competitive landscape for vadadustat; the scope, timing, and outcome of any ongoing 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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