September 26, 2017

Akebia Announces Positive Top-Line Results from Phase 2 Study of Vadadustat in Japanese Patients with Non-Dialysis-Dependent Chronic Kidney Disease

- Data Confirms Findings from Previous Studies -

- Phase 3 Study in Japan Expected to Commence by Year-End 2017 -

- Akebia Provides Mitsubishi Tanabe with Option to Access Global Phase 3 Vadadustat Data for Payments of up to $25 Million -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Akebia Therapeutics, Inc. (NASDAQ:AKBA), a biopharmaceutical company focused on delivering innovative therapies to patients with kidney disease through the biology of hypoxia-inducible factor (HIF), today announced positive top-line results from its Phase 2 study of vadadustat in Japanese patients with anemia associated with non-dialysis-dependent chronic kidney disease (NDD-CKD). The results confirm findings from previous studies of vadadustat. By the end of 2017, the Company, in collaboration with Mitsubishi Tanabe Pharma Corporation (MTPC), expects to announce top-line data from a Phase 2 study of vadadustat in dialysis patients with CKD and initiate a Phase 3 development program in non-dialysis patients in Japan. In addition, Akebia announced a new agreement with MTPC which grants MTPC an option to access data from Akebia's global Phase 3 vadadustat program for payments to Akebia of up to $25 million.

"We are pleased that the Phase 2 Japan results in NDD-CKD patients are consistent with data from our prior studies and we anticipate that the upcoming results in dialysis patients will provide further confirmation of vadadustat's potential," said Rita Jain, M.D., Senior Vice President and Chief Medical Officer of Akebia. "We look forward to the start of the Phase 3 program in Japan and a potential launch of vadadustat by MTPC in Japan in 2020."

The double-blind, placebo-controlled, dose-finding Phase 2 study was designed to evaluate the efficacy, safety, and tolerability of orally administered vadadustat in Japanese patients with anemia associated with NDD-CKD. This 16-week study evaluated 51 patients during a 6-week placebo-controlled, fixed-dose period and a 10-week active treatment, dose adjustment and maintenance period.

The primary efficacy endpoint was mean hemoglobin change from baseline to week 6 comparing vadadustat to placebo. Statistically significant improvements in the primary endpoint were observed in the vadadustat groups, 150mg, ($p < 0.0045$), 300mg, ($p < 0.0001$), and 600mg ($p < 0.0001$), compared to placebo. The data indicate a dose-response for vadadustat.

The incidence of any adverse events during the 6-week placebo-controlled, fixed-dose period in the vadadustat groups (150mg, 300mg, and 600mg) and placebo was 33%, 58%, 54%, and 36%, respectively. During the 16-week study, the most common adverse events reported were viral upper respiratory tract infection (12%) and hypertension (10%). Serious adverse events were consistent with prior studies and included 13 in 11 patients. No deaths were reported.

Akebia expects to present the data at an upcoming scientific meeting and publish the study results in a peer-reviewed journal.

Following consultation with the Japanese Pharmaceuticals and Medical Devices Agency (PMDA), Akebia and MTPC agreed to pursue a local Phase 3 development program for vadadustat in Japan. The companies believe that this strategy offers the potential for an accelerated path toward approval of vadadustat in Japan. MTPC will be responsible for all costs associated with the Phase 3 vadadustat program in Japan and expects to submit a Japanese New Drug Application (JNDA) for vadadustat with an anticipated launch in 2020.

"The local Japanese development strategy provides a clear and potentially expedited path forward for vadadustat in this large and growing market," said John P. Butler, President and Chief Executive Officer of Akebia. "We are confident that MTPC is well positioned to execute on a successful local Phase 3 program for vadadustat and a regulatory submission in Japan sooner than had we included Japanese patients in our global Phase 3 program. We are committed to ensuring that vadadustat is able to realize its full potential and will continue to work closely with MTPC to achieve this goal in Japan."

In December 2015, Akebia and MTPC signed a Collaboration Agreement for vadadustat in Japan and certain other

countries, which provided for committed capital and potential milestone payments to Akebia.

About Vadadustat

Vadadustat is an oral hypoxia-inducible factor (HIF) stabilizer currently in Phase 3 development for the treatment of anemia related to chronic kidney disease. Vadadustat exploits the same mechanism of action used by the body to adapt naturally to lower oxygen availability associated with a moderate increase in altitude. At higher altitudes, the body responds to lower oxygen availability with increased production of HIF, which coordinates the interdependent processes of iron mobilization and erythropoietin production to increase red blood cell production and, ultimately, improve oxygen delivery.

About Akebia Therapeutics

Akebia Therapeutics, Inc. is a biopharmaceutical company headquartered in Cambridge, Massachusetts, focused on delivering innovative therapies to patients with kidney disease through hypoxia-inducible factor biology. Akebia's lead product candidate, vadadustat, is an oral, investigational therapy in development for the treatment of anemia related to chronic kidney disease in both non-dialysis and dialysis patients. Akebia's global Phase 3 program for vadadustat, which includes the PRO$_2$TECT studies for non-dialysis patients with anemia secondary to chronic kidney disease and the INNO$_2$VATE studies for dialysis-dependent patients, is currently ongoing. In addition, the Company has initiated the Phase 2 FO$_2$RWARD study of vadadustat in dialysis-dependent chronic kidney disease patients who are hyporesponsive to erythropoiesis-stimulating agents (ESAs), and expects to commence the Phase 3 TRILO$_2$GY study to further evaluate a three-times-weekly dosing regimen for vadadustat. For more information, please visit our website at www.akebia.com.

Forward-Looking Statements

This press release includes forward-looking statements. Such forward-looking statements include those about Akebia's strategy, future plans and prospects, including statements regarding the potential commercialization of vadadustat in Japan if approved by regulatory authorities, the potential indications and benefits of vadadustat, the expected timing and results of clinical studies, and anticipated financial contributions from MTPC under the Collaboration Agreement. The words "anticipate," "appear," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions 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 risk that existing preclinical and clinical data may not be predictive of the results of ongoing or later clinical trials; the actual funding required to develop 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; early termination of Akebia's Collaboration Agreement with MTPC; Akebia's ability to satisfy its obligations under the Collaboration Agreement; the timing and content of decisions made by the PMDA and other regulatory authorities; the timing of any additional studies initiated by Akebia or MTPC for vadadustat; the rate of enrollment in clinical studies of vadadustat; the actual time it takes to initiate and complete research and clinical studies; the success of competitors in developing product candidates for diseases for which Akebia is currently developing its product candidates; and Akebia's ability to obtain, maintain and enforce patent and other intellectual property protection for vadadustat and its other product candidates. Other risks and uncertainties include those identified under the heading "Risk Factors" in Akebia's Annual Report on Form 10-Q for quarter ended June 30, 2017, and other filings that Akebia may make with the Securities and Exchange Commission in the future. Akebia does not undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this press release.

View source version on businesswire.com: http://www.businesswire.com/news/home/20170926005537/en/

Akebia:
Theresa McNeely, 617-844-6113
SVP, Corporate Communications and Investor Relations
tmcneely@akebia.com

Source: Akebia Therapeutics, Inc.