Akebia Announced Positive Top-Line Results from Global Phase 3 Program of Vadadustat for Treatment of Anemia Due to Chronic Kidney Disease in Adult Patients on Dialysis; Reports First Quarter 2020 Financial Results

May 5, 2020

- Vadadustat achieves primary efficacy and cardiovascular safety endpoints
- Company's cash runway extends well into 2021
- Company to host conference call today at 8:30 a.m. ET to discuss top-line data and its first quarter results

CAMBRIDGE, Mass., May 5, 2020 /PRNewswire/ -- Akebia Therapeutics, Inc. (Nasdaq: AKBA), today announced positive top-line results from INNO$_2$VATE, its global Phase 3 cardiovascular outcomes program evaluating the efficacy and safety of vadadustat, its investigational oral hypoxia-inducible factor prolyl hydroxylase inhibitor (HIF-PHI), versus darbepoetin alfa for the treatment of anemia due to chronic kidney disease (CKD) in adult patients on dialysis. (Please refer to Akebia's INNO$_2$VATE Data Announcement for the top-line data.) The Company also reported financial results for the first quarter ended March 31, 2020 and will host a conference call with slides today, Tuesday, May 5, 2020, at 8:30 a.m. Eastern Time to discuss INNO$_2$VATE top-line data, its first quarter financial results and recent business highlights.

"We are very excited about the positive top-line results from INNO$_2$VATE and expect to build on this momentum with many potentially transformational near-term milestones," said John P. Butler, President and Chief Executive Officer of Akebia Therapeutics. "We believe we've developed an exciting path forward for vadadustat and although the COVID-19 environment remains unpredictable, we continue to execute and deliver solid progress against these initiatives. In collaboration with our partner, Mitsubishi Tanabe, we are advancing key pre-commercial activities in support of the first regulatory approval of vadadustat expected in Japan this year. We have also significantly advanced PRO$_2$TECT, our global Phase 3 studies evaluating the safety and efficacy of vadadustat in adult patients not on dialysis with anemia due to CKD, and expect to report top-line data from these studies mid-year, as planned. In addition, we reinforced our intellectual property position for vadadustat, confirming the path for Akebia and our collaboration partner, Otsuka, to launch vadadustat in the U.K. and potentially the rest of Europe, upon approval."

"Akebia continues to provide and support our innovative therapies, which are critical to the care of adult patients with CKD, who are among the most at risk during this pandemic. To date, we have not experienced any significant adverse impact from COVID-19 and our fundamentals remain strong with a cash runway that extends well into 2021. However, these are unprecedented times and, due to COVID-19, we do not have clear visibility on how product demand and payer mix may be impacted. As a result, we continue to actively monitor and assess the potential impact of COVID-19 on our business and operations while continuing to support patients." Butler concluded, "We believe we have tremendous value-enhancing opportunities ahead and we remain focused on supporting patients and executing on our plans to position Akebia for long term growth."

Business Highlights

- Today, the Company announced positive top-line results from INNO$_2$VATE. Please refer to Akebia's INNO$_2$VATE Data Announcement for the top-line data.
- In April, the Patents Court of the United Kingdom issued a judgment in favor of Akebia and Otsuka Pharmaceutical Co. Ltd., which found that five of FibroGen, Inc.'s six HIF-related patents were invalid, and a sixth patent would not be infringed.
- In April, the Company announced that Myles Wolf, M.D., M.M.Sc., joined Akebia's Board of Directors. Dr. Wolf is a leading clinical nephrologist and physician-scientist in the fields of disordered mineral metabolism and cardiovascular disease in patients with CKD. Dr. Wolf serves as Chief of the Division of Nephrology and a Professor of Medicine at Duke University School of Medicine.
- In April, the Company settled Auryxia patent litigation with Teva Pharmaceuticals USA, Inc. (Teva) and its wholly owned, indirect subsidiary, Watson Laboratories, Inc. (Watson), resolving patent litigation brought in response to Abbreviated New Drug Application (ANDA) filings by Teva and Watson. Consistent with the Company's prior ANDA settlement with Par Pharmaceutical, Inc., the settlement allows Teva and Watson to market its generic version of Auryxia in the United States beginning on March 20, 2025 (subject to U.S. FDA approval), or earlier under certain circumstances customary for settlement agreements of this nature.
- In April, the Company entered into a new supply agreement with STA Pharmaceutical Hong Kong Limited, a subsidiary of Wuxi AppTec (WuXi), under which WuXi will manufacture vadadustat drug substance for commercial use. The WuXi supply agreement is the third commercial supply agreement the Company has entered into for vadadustat. The Company entered into a commercial supply agreement for vadadustat drug substance with Esteve Química, S.A. in April 2019, and a commercial supply agreement for vadadustat drug product with Patheon Inc. in March 2020.
- In April, the United States District Court for the District of Delaware dismissed a putative securities class action brought

against the Company's wholly owned subsidiary, Keryx Biopharmaceuticals, Inc. (Keryx), and former members of Keryx's board of directors, relating to the Company's 2018 merger with Keryx in response to a motion to dismiss filed by Keryx and the former directors.

- In February, the Company entered into a letter agreement with Vifor (International) Ltd. (Vifor Pharma) relating to Vifor Pharma's agreement with a third party to purchase a Priority Review Voucher (PRV) issued by the U.S. Food and Drug Administration (FDA), subject to satisfaction of customary closing conditions. Pursuant to the letter agreement, Akebia paid Vifor Pharma $10 million. Vifor Pharma is obligated to reserve the PRV for the vadadustat NDA for the treatment of anemia due to CKD in dialysis-dependent and non-dialysis dependent patients until Akebia and Vifor Pharma agree on the financial and other terms under which it will assign the PRV to Akebia or make a mutual decision to sell the PRV. A PRV entitles the holder to priority review of an NDA or a Biologics License Application for a new drug, which reduces the target FDA review time to six months after official acceptance of the submission and could lead to expedited approval.

First Quarter Financial Results

- **Revenues:** Total revenue increased 22 percent to $88.5 million for the first quarter of 2020 compared to $72.7 million for the first quarter of 2019.
  - Collaboration revenue was $59.3 million for the first quarter of 2020 compared to $49.6 million in the first quarter of 2019, an increase of 20 percent.
  - Net product revenue was $29.2 million for the first quarter of 2020 compared with $23.1 million in the first quarter of 2019, an increase of 26 percent.
- **COGS**: Cost of goods sold was $27.7 million for the first quarter of 2020, which includes non-cash charges, related to the application of purchase accounting as a result of the merger with Keryx, of $11.2 million for inventory step-up and $9.1 million for amortization of intangibles. Cost of goods sold was $31.3 million for the first quarter of 2019, and included non-cash merger-related charges of $14.6 million for inventory step-up and $9.1 million for amortization of intangibles.
- **R&D Expenses:** Research and development expenses were $81.2 million for the first quarter of 2020 compared to $82.4 million for the first quarter of 2019.
- **SG&A Expenses:** Selling, general and administrative expenses were $38.0 million for the first quarter of 2020 compared to $34.3 million for the first quarter of 2019.
- **Net Loss:** Net loss was $60.7 million for the first quarter of 2020 compared to $72.4 million for the first quarter of 2019.
- **Cash Position:** Cash, cash equivalents and available-for-sale securities as of March 31, 2020 were $115.4 million. The Company's cash runway extends well into 2021. The Company's cash runway, consistent with previous commentary, includes the receipt of a $15.0 million regulatory milestone from Mitsubishi Tanabe Pharma Corporation, Akebia's development and commercialization collaboration partner in Japan for vadadustat, assuming approval of vadadustat in Japan.

"Consistent with our prior first quarter periods, the decrease in the Company's cash, cash equivalents and available-for-sale securities from year-end 2019 was due to the timing of cash payments from our collaboration partner, Otsuka, for which $49.5 million was received in the current quarter rather than the first quarter of 2020. In addition, during the first quarter, we paid $10 million to Vifor Pharma in connection with Vifor Pharma's purchase of the PRV," stated Jason A. Amello, Chief Financial Officer of Akebia.

COVID-19 Response

The current severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) (COVID-19), pandemic has presented a substantial public health and economic challenge around the world and is affecting Akebia's employees, patients, customers, collaboration partners, vendors, communities and business operations. On March 15, 2020, Akebia issued a press release detailing its response to the COVID-19 pandemic. Consistent with that statement, the Company continues to take action to safeguard its employees, patients and customers, ensure business continuity, and support supply of its innovative therapies which are critical to the care of adult CKD patients, who are among the most at risk during this pandemic. Akebia continues to actively monitor and assess the potential impact of the COVID-19 pandemic on its business and operations. Patients with any questions about accessing Akebia's marketed therapy may refer to AkebiaCares, the Company's patient access program, for further information.

Conference Call:

Akebia will host a conference call today, Tuesday, May 5, 2020, at 8:30 a.m. Eastern Time to discuss its INNO$_2$VATE top-line data announced earlier this morning and its first quarter financial results. To listen to the conference call, please dial (877) 458-0977 (domestic) or (484) 653-6724 (international) using conference ID number 8464788. The call will also be webcast LIVE with slides and can be accessed via the Investors section of the Company's website at http://ir.akebia.com.

A replay of the conference call and the slides will be available two hours after the completion of the call through May 11, 2020. To access the replay, dial (855) 859-2056 (domestic) or (404) 537-3406 (international) and reference conference ID number 8464788. An online archive of the conference call can be accessed via the Investors section of the Company's website at http://ir.akebia.com.

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. 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.

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.

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 clinical trial data and results and the anticipated timing of the availability and reporting thereof; Akebia's momentum and potentially transformational near-term milestones; 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; access to a Priority Review Voucher for vadadustat and the agreement relating thereto; potential and anticipated payments from our collaborators, including the timing thereof; expectations regarding financial position, including cash runway and the components thereof; and our intellectual property position. The terms "anticipate," "believe," "expect," "opportunity," "planned," "potential," "target," "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 potential direct or indirect impact of the COVID-19 pandemic on our business, operations, and the markets and communities in which we and our partners, collaborators, vendors and customers operate; the risk that existing preclinical and clinical data may not be predictive of the results of ongoing or later clinical trials, including PRO_2TECT; the risk that clinical trials may not be successful; manufacturing risks; risks associated with the Priority Review Voucher for vadadustat; risks associated with management and key personnel changes and transitional periods; the actual funding required to develop and commercialize our commercial product, vadadustat and other 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; market acceptance and coverage and reimbursement of our commercial product and vadadustat, if approved; the risks associated with potential generic entrants for our commercial product and vadadustat, if approved; 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 timing of any additional studies initiated for vadadustat; the actual time it takes to initiate and complete preclinical and clinical studies; the competitive landscape for our commercial product and 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; the risk that we lose, or settle on less favorable terms, other ANDA litigation, or that other ANDA filers enter the market earlier than March 20, 2025, as well as any other potential settlements; and Akebia's ability to obtain, maintain and enforce patent and other intellectual property protection for our commercial product, vadadustat and any other product candidates. 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, 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.

Investor Contact:
Kristen K. Sheppard, Esq.
lr@akebia.com

AKEBIA THERAPEUTICS, INC.
Consolidated Statements of Operations
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended	
	March 31, 2020	**March 31, 2019**
Revenues:		
Product revenue, net	$ 29,209	$ 23,111
License, collaboration and other revenue	59,269	49,555
Total revenues	88,478	72,666
Cost of goods sold:		
Product	18,613	22,157
Amortization of intangibles	9,100	9,100
Total cost of goods sold	27,713	31,257
Operating expenses:		
Research and development	81,231	82,351
Selling, general and administrative	37,983	34,291
License expense	676	736
Total operating expenses	119,890	117,378
Operating loss	(59,125)	(75,969)
Other income (expense), net	(1,622)	791
Net loss before income taxes	(60,747)	(75,178)
Benefit from income taxes	—	(2,757)
Net loss	$ (60,747)	$ (72,421)
Net loss per share - basic and diluted	$ (0.47)	$ (0.62)

Weighted-average number of commons shares - basic and diluted	128,395,163	117,063,352

AKEBIA THERAPEUTICS, INC.
Selected Balance Sheet Data
(in thousands)
(unaudited)

	March 31, 2020	December 31, 2019
Cash, cash equivalents and available for sale securities	$ 115,374	$ 147,694
Working capital	126,604	101,415
Total assets	787,719	771,201
Total stockholders' equity	396,364	394,757

View original content to download multimedia:http://www.prnewswire.com/news-releases/akebia-announced-positive-top-line-results-from-global-phase-3-program-of-vadadustat-for-treatment-of-anemia-due-to-chronic-kidney-disease-in-adult-patients-on-dialysis-reports-first-quarter-2020-financial-results-301052863.html

SOURCE Akebia Therapeutics