Akebia Reports Second Quarter 2020 Financial Results

August 10, 2020

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- *Top-line data readout of global Phase 3 PRO_2TECT program of vadadustat for treatment of anemia due to CKD in adult patients not on dialysis on track for early September*
- *Cash runway extends beyond expected U.S. launch of vadadustat*

Akebia Therapeutics, Inc. (Nasdaq: AKBA), a biopharmaceutical company with the purpose of bettering the lives of people impacted by kidney disease, today reported financial results for the second quarter ended June 30, 2020. As previously announced, in lieu of a financial results and business update call, Akebia management plans to host a conference call and webcast in early September to report top-line data from PRO_2TECT, the second of its two global Phase 3 cardiovascular outcomes programs. The two PRO_2TECT studies evaluated the efficacy and safety of vadadustat, the Company's investigational oral hypoxia-inducible factor prolyl hydroxylase inhibitor (HIF-PHI), for the treatment of anemia due to chronic kidney disease (CKD) in adult patients not on dialysis.

"We had an incredible quarter in terms of advancing our vadadustat clinical development program, bringing us that much closer to achieving our purpose to better the life of each person impacted by kidney disease. We reported exciting, positive top-line data from our global Phase 3 $INNO_2$VATE program highlighting vadadustat's potential as a new oral standard of care for treating anemia due to CKD in adult patients on dialysis, and topped off the quarter with the first regulatory approval of vadadustat in Japan," said John P. Butler, President and Chief Executive Officer of Akebia Therapeutics. "The next chapter of Akebia's growth story is starting to unfold and as previously announced, we plan to share top-line data from PRO_2TECT in early September."

Recent Business Highlights

- In August, the Company announced database lock for PRO_2TECT and plans to report top-line data from PRO_2TECT in early September. This announcement follows Akebia's earlier update provided in May that it had achieved the target number of major adverse cardiovascular events (MACE) for the PRO_2TECT studies.
- In July, the Company announced an investigator-sponsored research study by The University of Texas Health Science Center at Houston (UTHealth) in Houston, Texas, evaluating the use of vadadustat as a potential therapy to prevent and lessen the severity of acute respiratory distress syndrome (ARDS), a complication of COVID-19 infection.
- In June, Mitsubishi Tanabe Pharma Corporation (MTPC), Akebia's collaboration partner in Japan for vadadustat, obtained the first regulatory approval of vadadustat (Japan trade name: VAFSEO™), as a treatment for anemia due to CKD in both dialysis-dependent and non-dialysis dependent adult patients, by the Ministry of Health, Labour and Welfare in Japan.
- In May, the Company reported positive top-line data from $INNO_2$VATE, the first of its two global Phase 3 cardiovascular outcomes programs, which evaluated the efficacy and safety of vadadustat versus darbepoetin alfa for the treatment of anemia due to CKD in adult patients on dialysis. Vadadustat showed consistency across both efficacy and all MACE components, achieving the primary efficacy and safety endpoints, as well as the key secondary efficacy endpoint, of the studies. Please refer to Akebia's $INNO_2$VATE Data Announcement for the top-line data.
- In May, the Company completed a public offering of its common stock raising net proceeds of $142.4 million.
- In May, the Company announced that its collaboration partner, Japan Tobacco, Inc., filed a supplemental New Drug Application with the Pharmaceuticals and Medical Devices Agency (PMDA) seeking an additional indication for Riona® (generic name in Japan: ferric citrate hydrate) to treat adult patients with iron deficiency anemia (IDA) in Japan.

Second Quarter Financial Results

- **Revenues:** Total revenue was $90.1 million for the second quarter of 2020 compared to $100.8 million for the second quarter of 2019. The decline versus the prior year period was driven by lower collaboration revenue consistent with the Company completing the $INNO_2$VATE studies and nearing completion of the PRO_2TECT studies.
  - Collaboration revenue was $59.4 million for the second quarter of 2020 compared to $71.7 million in the second quarter of 2019.
  - Net product revenue for Auryxia® (ferric citrate) was $30.7 million for the second quarter of 2020 compared with

$29.1 million in the second quarter of 2019, an increase of 5.5 percent.

- **COGS:** Cost of goods sold increased $136.9 million compared to the prior year period primarily due to a non-cash impairment charge of $115.5 million related to Auryxia, and higher non-cash inventory write-downs, which included $12.4 million largely related to a manufacturing quality issue related to Auryxia identified in the second quarter of 2020.
- **R&D Expenses:** Research and development expenses were $52.8 million for the second quarter of 2020 compared to $85.7 million for the second quarter of 2019. The decline versus the prior year period was primarily driven by a decrease in costs consistent with the Company completing the INNO$_2$VATE studies and nearing completion of the PRO$_2$TECT studies.
- **SG&A Expenses:** Selling, general and administrative expenses were $35.5 million for the second quarter of 2020 compared to $36.1 million for the second quarter of 2019.
- **Net Loss:** Net loss was $175.8 million for the second quarter of 2020 compared to $58.2 million for the second quarter of 2019. The increase in net loss compared to the prior year period was due primarily to the non-cash impairment charge and higher non-cash inventory write-downs.
- **Cash Position:** Cash, cash equivalents and available-for-sale securities as of June 30, 2020 were $295.3 million. The increase in the Company's cash position is primarily attributable to net proceeds of $142.4 million from Akebia's public offering of common stock, which was completed in May 2020. The Company believes that its cash runway extends beyond the expected U.S. launch of vadadustat.

"The non-cash impairment charge reflects the change in value of the Auryxia intangible asset on our balance sheet, primarily driven by the compounding impact of the 2018 decision by the Centers for Medicare and Medicaid Services (CMS) rescinding Medicare Part D coverage of Auryxia for its IDA indication and imposing a prior authorization requirement for the hyperphosphatemia indication," stated David A. Spellman, Chief Financial Officer of Akebia Therapeutics. "While we are frustrated and disappointed that a resolution has not been reached on this matter for the benefit of patients, we remain optimistic about Auryxia's growth prospects."

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 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 in global Phase 3 development for the treatment of anemia due to CKD and is not approved by the U.S. Food and Drug Administration (FDA) or any regulatory authority with the exception of Japan's Ministry of Health, Labour and Welfare (MHLW). In Japan, vadadustat is approved as a treatment for anemia due to CKD in both dialysis-dependent and non-dialysis dependent adult patients.

About Auryxia® (ferric citrate) Tablets
Auryxia (ferric citrate) was approved by FDA on September 5, 2014 for the control of serum phosphorus levels in adult patients with CKD on dialysis (the "hyperphosphatemia indication") and approved by FDA on November 6, 2017 for the treatment of iron deficiency anemia in adult patients with CKD not on dialysis (the "IDA indication"). For more information about Auryxia and the U.S. full prescribing information, please visit www.auryxia.com.

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.

IMPORTANT U.S. SAFETY INFORMATION FOR AURYXIA® (ferric citrate) CONTRAINDICATION
AURYXIA® (ferric citrate) is contraindicated in patients with iron overload syndromes, e.g., hemochromatosis.

WARNINGS AND PRECAUTIONS

- **Iron Overload:** Increases in serum ferritin and transferrin saturation (TSAT) were observed in clinical trials with AURYXIA in patients with chronic kidney disease (CKD) on dialysis treated for hyperphosphatemia, which may lead to excessive elevations in iron stores. Assess iron parameters prior to initiating AURYXIA and monitor while on therapy. Patients receiving concomitant intravenous (IV) iron may require a reduction in dose or discontinuation of IV iron therapy.

- **Risk of Overdosage in Children Due to Accidental Ingestion:** Accidental ingestion and resulting overdose of iron-containing products is a leading cause of fatal poisoning in children under 6 years of age. Advise patients of the risks to children and to keep AURYXIA out of the reach of children.

ADVERSE REACTIONS
Most common adverse reactions with AURYXIA were:

- **Hyperphosphatemia in CKD on Dialysis:** Diarrhea (21%), discolored feces (19%), nausea (11%), constipation (8%), vomiting (7%) and cough (6%).
- **Iron Deficiency Anemia in CKD Not on Dialysis**: Discolored feces (22%), diarrhea (21%), constipation (18%), nausea

(10%), abdominal pain (5%) and hyperkalemia (5%).

SPECIFIC POPULATIONS

- **Pregnancy and Lactation:** There are no available data on AURYXIA use in pregnant women to inform a drug-associated risk of major birth defects and miscarriage. However, an overdose of iron in pregnant women may carry a risk for spontaneous abortion, gestational diabetes and fetal malformation. Data from rat studies have shown the transfer of iron into milk, hence, there is a possibility of infant exposure when AURYXIA is administered to a nursing woman.

To report suspected adverse reactions, contact Akebia Therapeutics at 1-844-445-3799.

Please see full Prescribing Information

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 plans to report top-line data from PRO_2TECT and the anticipated timing thereof; Akebia's growth story and growth prospects; the safety and efficacy of vadadustat and the potential indications for and benefits of vadadustat; and estimates, beliefs and judgments related to the impairment charge, including the drivers thereof. 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 and our partners', collaborators', vendors' and customers' businesses, 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; the risks associated with potential generic entrants for our commercial product and vadadustat; early termination of any of Akebia's collaborations and material contracts; Akebia's and its collaborators' ability to satisfy their obligations under Akebia's collaboration agreements and material contracts; 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; 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 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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AKEBIA THERAPEUTICS, INC.
Consolidated Statements of Operations
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended		Six Months Ended	
	June 30, 2020	June 30, 2019	June 30, 2020	June 30, 2019
Revenues:				
Product revenue, net	$ 30,696	$ 29,089	$ 59,905	$ 52,200
License, collaboration and other revenue	59,446	71,714	118,715	121,269
Total revenues	90,142	100,803	178,620	173,469
Cost of goods sold:				
Product	49,988	28,569	68,601	50,726
Amortization of intangibles	9,101	9,100	18,201	18,200
Impairment of intangible asset	115,527	—	115,527	—
Total cost of goods sold	174,616	37,669	202,329	68,926
Operating expenses:				
Research and development	52,819	85,694	134,050	168,045
Selling, general and administrative	35,482	36,068	73,465	70,359
License expense	1,044	895	1,720	1,631
Total operating expenses	89,345	122,657	209,235	240,035
Operating loss	(173,819)	(59,523)	(232,944)	(135,492)

Other income (expense), net	(1,932)	508	(3,554)	1,299
Net loss before income taxes	(175,751)	(59,015)	(236,498)	(134,193)
Benefit from income taxes	—	(845)	—	(3,602)
Net loss	$ (175,751)	$ (58,170)	$ (236,498)	$ (130,591)
Net loss per share - basic and diluted	$ (1.28)	$ (0.49)	$ (1.78)	$ (1.11)
Weighted-average number of common shares - basic and diluted	136,906,968	118,268,832	132,651,066	117,669,422

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

	June 30, 2020	December 31, 2019
Cash, cash equivalents and available for sale securities	$295,349	$147,694
Working capital	253,328	101,415
Total assets	745,174	771,201
Total stockholders' equity	370,260	394,757

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