Akebia Reports Fourth Quarter and Full-Year 2020 Financial Results and Provides Business Updates

February 25, 2021

- Full-year 2020 net product revenue increases to $128.9 million, up 16% from 2019
- Company on track to submit vadadustat NDA by mid-second quarter 2021
- LeAnne M. Zumwalt, dialysis industry leader, joins Board of Directors
- Strengthens balance sheet and reinforces cash runway with $60 million non-dilutive transaction with HealthCare Royalty

CAMBRIDGE, Mass., Feb. 25, 2021 /PRNewswire/ -- 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 fourth quarter and full-year ended December 31, 2020 and provided business updates. The Company will host a conference call today, Thursday, February 25, 2021, at 9:00 a.m. Eastern Time.

Akebia also announced a $60 million non-dilutive transaction with an entity managed by HealthCare Royalty Management, LLC (HCR), to monetize the Company's rights to receive royalties and sales milestones on vadadustat net sales under its collaboration agreement with Mitsubishi Tanabe Pharma Corporation (MTPC). MTPC has the exclusive rights to commercialize vadadustat in Japan, where it is currently marketed under the trade name Vafseo ™ (vadadustat), and certain other Asian countries. Under the terms of the agreement with HCR, Akebia receives an upfront cash payment of $45 million and is eligible to receive an additional $15 million if certain sales milestones are achieved. In exchange, HCR has the right to receive Vafseo royalties and sales milestones due to the Company under its collaboration agreement with MTPC, subject to an annual cap of $13 million and an aggregate cap of $150 million. After the annual cap is met in a given calendar year, Akebia will recognize 85 percent of Vafseo royalties and sales milestones from MTPC for that year. After the aggregate cap is met, Akebia will recognize 100 percent of Vafseo royalties and sales milestones until this revenue stream ends under the terms of the Company's collaboration agreement with MTPC. The transaction does not include potential future regulatory milestones to be paid by MTPC.

"2020 was a year of focused execution for Akebia as we advanced vadadustat, our investigational oral hypoxia-inducible factor prolyl hydroxylase inhibitor (HIF-PHI), and executed on our commercial priorities. Importantly, we achieved this while continuing to provide patients with access to our therapies and keeping with our goal of maintaining a strong balance sheet," stated John P. Butler, Chief Executive Officer of Akebia. "With this forward momentum, we have line of sight to significant milestones in 2021, including our NDA submission for vadadustat for the treatment of anemia due to chronic kidney disease (CKD) in both adult patients on dialysis and not on dialysis expected by the middle of the second quarter of this year."

Butler continued, "As we advance vadadustat toward commercialization, subject to approval, we continue to execute on key commercial, development, and financial priorities. As dialysis is a primary focus, we're thrilled to have recently added LeAnne Zumwalt to our Board of Directors. LeAnne's perspective and extensive operating experience with one of the largest dialysis operators in the U.S. will help ensure that our market and commercial strategies are well aligned with the needs of dialysis providers and their patients. We're also pleased to have announced a $60 million royalty monetization transaction with HCR that we believe strengthens our balance sheet and helps preserve both our strategic and financial flexibility while we continue investing for the successful launch of vadadustat, upon approval."

Akebia plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for vadadustat by the middle of the second quarter of this year for the treatment of anemia due to CKD in both adult patients on dialysis and adult patients not on dialysis. In addition, Akebia and its collaborator, Otsuka Pharmaceutical Co. Ltd. (Otsuka), are working in close collaboration to prepare a Marketing Authorization Application (MAA) for submission to the European Medicines Agency (EMA), expected this year.

Recent Business Highlights:

- In February, the Company announced that LeAnne M. Zumwalt joined Akebia's Board of Directors. Ms. Zumwalt recently served as Group Vice President, Government Affairs at DaVita Inc.
- In February, Akebia launched its *Medical Engagement Hub*, an online resource dedicated to scientific education and connecting U.S. healthcare professionals with Akebia Medical Affairs.
- In January, the University of Texas Health Science Center at Houston (UTHealth) in Houston, Texas, announced that it had received $5.1 million in government funding for its study evaluating the use of vadadustat as a potential therapy to prevent and lessen the severity of acute respiratory distress syndrome (ARDS), a complication of COVID-19. This investigator-sponsored research study is currently underway and actively enrolling patients.
- In November, the study design and methodology for Akebia's global Phase 3 INNO$_2$VATE program was published in *Nephrology Dialysis Transplantation (NDT)*.
- In October, the study design and methodology for Akebia's global Phase 3 PRO$_2$TECT program was published in *American Heart Journal*.
- In October, Akebia completed a pre-NDA meeting with the FDA for vadadustat.

- In October, top-line data from both INNO$_2$VATE and PRO$_2$TECT were presented at American Society of Nephrology Kidney Week 2020 Reimagined.
- In October, Akebia and Otsuka launched *Balancing Anemia Due to CKD*, a campaign and website designed to increase awareness and education of anemia due to CKD among healthcare providers with the goal of improving the management of this disease for patients.

Fourth Quarter and Full-Year 2020 Financial Results

- **Revenues:** Total revenue was $56.7 million for the fourth quarter of 2020 compared to $69.6 million for the fourth quarter of 2019, and $295.3 million for the full-year 2020 compared to $335.0 million for the full-year 2019. The decline in both periods was driven by lower collaboration revenue consistent with the Company successfully completing its global Phase 3 clinical development program for vadadustat, which consisted of two programs that evaluated the efficacy and safety of vadadustat versus darbepoetin alfa for the treatment of anemia due to CKD in adult patients on dialysis (INNO$_2$VATE) and not on dialysis (PRO$_2$TECT).
    - Collaboration revenue was $22.1 million for the fourth quarter of 2020 compared to $40.6 million for the fourth quarter of 2019, and $166.4 million for the full-year 2020 compared with $223.9 million for the full-year 2019. The change in both periods is due to the timing of when vadadustat development expenses are incurred and the associated revenue is recognized on a percentage-of-completion basis. Collaboration revenue for the full-year 2020 includes $0.4 million in royalty revenue related to the commercial sale of Vafseo in Japan from MTPC, which launched in August of 2020.
    - Net product revenue was $34.6 million for the fourth quarter of 2020 compared with $28.9 million for the fourth quarter of 2019, an increase of 20 percent. Net product revenue was $128.9 million for the full-year 2020 compared to $111.1 million for the full-year 2019, an increase of 16 percent. While Akebia did not experience a significant impact from COVID-19 on revenues during the first nine months of 2020, the Company believes its revenue growth was negatively impacted during the fourth quarter of 2020 primarily as the kidney patient populations that it serves continue to experience both higher hospitalization and mortality rates due to COVID-19.
- **COGS:** Cost of goods sold was $63.2 million for the fourth quarter of 2020 compared to $38.1 million for the fourth quarter of 2019. Excluding non-cash purchase accounting adjustments as a result of the merger with Keryx, the increase in the fourth quarter of 2020 was primarily driven by a $14.8 million non-cash charge related to excess purchase commitments. Cost of goods sold was $295.9 million for the full-year 2020, compared with $145.3 million for the full-year 2019. Cost of goods sold for 2020 includes a $115.5 million non-cash charge related to the impairment of the Auryxia intangible asset in the second quarter of 2020, a $25.1 million non-cash charge related to excess purchase commitments, and a $20.1 million non-cash charge for inventory write-downs largely related to a previously disclosed manufacturing quality issue related to Auryxia.
- **R&D Expenses:** Research and development expenses were $37.6 million for the fourth quarter of 2020 compared to $80.4 million for the fourth quarter of 2019, and $218.5 million for the full-year 2020 compared to $323.0 million for the full-year 2019. The decline in both periods was primarily driven by a decrease in costs consistent with the Company completing the INNO$_2$VATE and PRO$_2$TECT studies.
- **SG&A Expenses:** Selling, general and administrative expenses were $40.3 million for the fourth quarter of 2020 compared to $44.9 million for the fourth quarter of 2019, and $153.9 million for the full-year 2020 compared to $149.5 million for the full-year 2019.
- **Net Loss:** Net loss was $87.0 million for the fourth quarter of 2020 compared to $94.5 million for the fourth quarter of 2019, and $383.5 million for the full-year 2020 compared to $279.7 million for the full-year 2019. The increase in net loss for the full-year 2020 compared to the prior year period was due primarily to lower collaboration revenue and higher cost of goods sold, partially offset by lower operating expenses.
- **Cash Position:** Cash, cash equivalents and available-for-sale securities as of December 31, 2020 were $268.7 million. The Company expects its cash resources to fund its current operating plan beyond the expected U.S. launch of vadadustat, assuming timely regulatory approval and the receipt of associated regulatory milestones.

"As COVID-19 continues to adversely and disproportionately impact our patient population with higher hospitalization and mortality rates, we expect this will have a negative impact on future Auryxia revenue growth. While we are unable to fully quantify the impact of the COVID-19 pandemic on future revenues and revenue growth, we continue to work to position the Company to navigate these challenges. As such, our financial priorities remain focused on improving our cost structure and maintaining a strong balance sheet, as evidenced by our recent $60 million non-dilutive royalty transaction with HCR," stated David A. Spellman, Chief Financial Officer of Akebia Therapeutics.

Conference Call

Akebia will host a conference call at 9:00 a.m. Eastern Time today, Thursday, February 25th, to discuss its fourth quarter and full-year 2020 financial results and recent business highlights. To listen to the conference call, please dial (877) 458-0977 (domestic) or (484) 653-6724 (international) using conference ID number 5455117. The call will also be webcast LIVE and can be accessed via the Investors section of the Company's website at http://ir.akebia.com.

A replay of the conference call will be available two hours after the completion of the call through March 3, 2021. To access the replay, dial (855) 859-2056 (domestic) or (404) 537-3406 (international) and reference conference ID number 5455117. 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 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 recently completed its global Phase 3 development program for the treatment of anemia due to CKD. Vadadustat 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 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: the timing of submission of an NDA to the FDA for vadadustat for the treatment of anemia due to chronic kidney disease in both adult patients on dialysis and not on dialysis; the timing of submission of a MAA for submission to the EMA; the potential for obtaining approval of vadadustat in dialysis and non-dialysis indications; statements regarding Akebia's marketing and commercial strategies and Ms. Zumwalt's ability to ensure alignment of such strategies with the needs of dialysis providers and their patients; Akebia's achievement of certain sales milestones and the receipt of cash payments tied thereto in connection with the non-dilutive royalty transaction with HCR; the results that the royalty monetization agreement between Akebia and HCR has on Akebia's balance sheet and strategic and financial flexibility; the negative impact that higher hospitalization and mortality rates due to COVID-19 in the kidney patient populations that the Company serves had on the Company's revenue growth during the fourth quarter of 2020 and may continue to have in the future; and the timing and expectations for the Company's cash runway in relation to the expected timing of the U.S. launch of vadadustat. The terms "believe," "confident," "expect," "plan," "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, but not limited to: the timing related to regulatory filings and approvals, such as the anticipated timing of filing the NDA to the FDA and MAA

to the EMA for vadadustat and our outlook related thereto; the direct or indirect impact of the COVID-19 pandemic on Akebia's business, operations, and the markets and communities in which the Company and its partners, collaborators, vendors and customers operate; the potential therapeutic benefits, safety profile and effectiveness of Akebia's product candidates, including vadadustat; the timing and content of advice given and decisions made by health authorities, including approval and labeling decisions; the potential indications, demand and market potential and acceptance of Akebia's product and product candidates, including estimates regarding the potential market opportunity for the Company's product, vadadustat or any other product candidates and the size of eligible patient populations; enrollment in clinical and preclinical studies; manufacturing, supply and quality risks, and any recalls, write-downs, impairments or other related consequences or potential consequences; risks associated with hiring, training, management and retention and key personnel changes and transitional periods; the actual funding required to continue to commercialize Akebia's commercial product, to develop and commercialize vadadustat, and to operate the Company; market acceptance and coverage and reimbursement of the Company's commercial product and vadadustat, if approved; the risks associated with potential generic entrants for Akebia's 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 competitive landscape for Akebia's commercial product and vadadustat, if approved; 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 collaborations partners and vendors; expected reliance on third parties, including with respect to the development, manufacturing, supply or commercialization of Akebia's product and product candidates; the Company's expectations, projections and estimates regarding its capital requirements, need for additional capital, financing our future cash needs, costs, expenses, revenues, capital resources, cash flows, financial performance, profitability, tax obligations, liquidity, growth, contractual obligations, our internal control over financial reporting and disclosure controls and procedures, and remediation of any material weakness or deficiencies identified in our internal controls and procedures; and Akebia's intellectual property position, including its 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 September 30, 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, except as required by law, 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		Twelve Months Ended	
	December 31, 2020	December 31, 2019	December 31, 2020	December 31, 2019
Revenues:				
Product revenue, net	$ 34,604	$ 28,915	$ 128,901	$ 111,119
License, collaboration and other revenue	22,095	40,640	166,406	223,882
Total revenues	56,699	69,555	295,307	335,001
Cost of goods sold:				
Product	56,026	29,047	148,866	108,935
Amortization of intangibles	7,208	9,100	31,515	36,401
Impairment of intangible asset	—	—	115,527	—
Total cost of goods sold	63,234	38,147	295,908	145,336
Operating expenses:				
Research and development	37,578	80,412	218,485	322,969
Selling, general and administrative	40,311	44,918	153,947	149,455
License expense	979	969	3,409	3,529
Total operating expenses	78,868	126,299	375,841	475,953
Operating loss	(85,403)	(94,891)	(376,442)	(286,288)
Other expense, net	(1,597)	(1,344)	(7,015)	(2)
Net loss before income taxes	(87,000)	(96,235)	(383,457)	(286,290)
Benefit from income taxes	—	(1,752)	—	(6,631)
Net loss	$ (87,000)	$ (94,483)	$ (383,457)	$ (279,659)
Net loss per share - basic and diluted	$ (0.60)	$ (0.79)	$ (2.77)	$ (2.36)
Weighted-average number of common shares - basic and diluted	145,111,415	119,358,081	138,463,152	118,395,919

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and available for sale securities	$268,690	$147,694
Working capital	184,291	101,415
Total assets	644,139	771,201
Total stockholders' equity	247,618	394,757

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