Akebia Therapeutics Announces Fourth Quarter and Full-Year 2017 Financial Results

March 12, 2018

--Company to Host Conference Call Today at 4:30 p.m. Eastern Time--

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 12, 2018-- 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 financial results for the fourth quarter and full year ended December 31, 2017.

"The past year was highly productive for Akebia as we advanced our global Phase 3 program for vadadustat and completed significant collaboration and licensing deals to provide us with R&D financing, stronger commercial presence at launch and significant validation of vadadustat," said John P. Butler, President and Chief Executive Officer of Akebia Therapeutics. "We are now approaching an important inflection point, with top-line Phase 3 results anticipated next year, subject to the accrual of MACE events, and market launch anticipated in 2020, subject to approval."

2017 Full-Year and Recent Corporate Highlights

- Expanded our collaboration with Otsuka for vadadustat, which originally covered the U.S. only, to include a collaboration for Europe, China and other territories. Across both collaboration agreements, the total committed capital upon signing was $473 million with the potential for up to $1.4 billion in milestone payments;
- Entered into an exclusive license agreement with Vifor Pharma to sell vadadustat as its only HIF product for distribution to Fresenius Medical Care in the U.S., a kidney dialysis provider serving nearly 40% of U.S. dialysis patients, subject to approval of vadadustat by the U.S. Food and Drug Administration and inclusion of vadadustat in a bundled reimbursement model;
- Announced positive top-line results from two Phase 2 studies of vadadustat in Japanese patients, one in non-dialysis dependent chronic kidney disease (NDD-CKD) and the other in dialysis dependent chronic kidney disease (DD-CKD); the data from these studies were consistent with findings from previous studies of vadadustat;
- Following a positive consultation with the Japanese regulatory authority, PMDA, collaboration partner Mitsubishi Tanabe Pharma Corporation (MTPC) initiated Phase 3 studies of vadadustat in patients with NDD-CKD and DD-CKD in Japan, which generated a $10 million milestone to Akebia. Data read-outs are expected in 2019;
- Announced that we are targeting full enrollment in our Phase 3 INNO$_2$VATE and PRO$_2$TECT studies to occur by the end of 2018, with top-line results anticipated in 2019, subject to the accrual of MACE events, and market launch anticipated in 2020, subject to approval;
- Enhanced the study designs for FO$_2$RWARD and TRILO$_2$GY, which we believe will provide additional characterization and differentiation of vadadustat and further strengthen our commercial position, subject to vadadustat's approval;
- Completed two non-clinical carcinogenicity studies, a two-year study in rats and a six-month study in mice, results of which showed no carcinogenic effect of vadadustat;
- Published positive Phase 2a study results in the *American Journal of Nephrology*, which showed that vadadustat increased mean hemoglobin levels in a dose-dependent manner and improved biomarkers of iron mobilization in non-dialysis chronic kidney disease patients;
- Prevailed in additional European patent disputes in which the Opposition Division of the European Patent Office revoked another FibroGen, Inc. HIF-related patent in Europe, and another of FibroGen's patents was significantly narrowed to cover only an indication for which Akebia is not intending to develop vadadustat; and
- Signed an exclusive agreement with Johnson & Johnson Innovation to in-license HIF-targeting product candidates, including AKB-5169 for the treatment of inflammatory bowel disease, for which we are targeting an IND submission in 2018, and access an extensive library of well-characterized HIF pathway compounds.

Financial Results

Akebia reported net income for the fourth quarter of 2017 of $12.3 million, or $0.25 per diluted share, as compared to a net loss for the fourth quarter of 2016 of ($37.9) million, or ($0.99) per share. The net income reported for the fourth quarter of 2017 was attributable to $39.7 million of collaboration revenue recognized in connection with the MTPC agreement as the criteria for revenue recognition was satisfied in the fourth quarter.

The company reported a net loss for the full year of 2017 of ($76.9) million, or ($1.77) per share, as compared to a net loss for the full year of 2016 of ($135.7) million, or ($3.60) per share.

Collaboration revenue was $87.3 million for the fourth quarter of 2017 compared to $1.5 million for the fourth quarter of 2016, and $178.0 million for

the full year of 2017 compared to $1.5 million for the full year of 2016. Collaboration revenue recognized in 2016 related only to the Otsuka U.S. agreement, which was consummated in December 2016. Collaboration revenue recognized in 2017 related to revenue recognized under both the Otsuka U.S. agreement and the Otsuka International agreement, which was consummated in April 2017, as well as revenue recognized in connection with the MTPC agreement during the fourth quarter of 2017 as described above.

Research and development expenses were $68.4 million for the fourth quarter of 2017 compared to $33.4 million for the fourth quarter of 2016, and $230.9 million for the full year of 2017 compared to $115.8 million for the full year of 2016. The increase is primarily attributable to an increase in external costs related to the continued advancement of the PRO_2TECT and $INNO_2$VATE Phase 3 program, including ongoing enrollment; the Phase 2 studies in Japan; and study commencement activities for the FO_2RWARD and $TRILO_2$GY programs, both of which have been replaced with new study designs. Research and development expenses were further increased by headcount, consulting and facility-related costs related to additional resources required to support our expanding research and development programs.

General and administrative expenses were $7.6 million for the fourth quarter of 2017 compared to $6.1 million for the fourth quarter of 2016, and $27.0 million for the full year of 2017 compared to $22.2 million for the full year of 2016. The increase is primarily attributable to an increase in costs to support the company's research and development programs, including headcount and compensation-related costs and associated facility- and patent-related costs, as well as consulting costs.

Akebia ended 2017 with cash, cash equivalents and available-for-sale securities of $317.8 million. The company also generally receives cost-share funding from its collaboration agreements with Otsuka and MTPC on a prepaid quarterly basis. Akebia expects its existing cash resources, including the prepaid quarterly committed cost-share funding from its collaborators, to fund its current operating plan into the second quarter of 2019.

Conference Call and Webcast

Akebia management will host its fourth quarter and full-year 2017 investor update conference call and webcast beginning at 4:30 p.m. Eastern Time today, Monday, March 12, 2018.

Individuals interested in participating in the call should dial (877) 458-0977 (U.S. and Canada) or (484) 653-6724 (international) using conference ID number 5847908. To access the webcast, visit the investor's section of Akebia's website at www.akebia.com at least 15 minutes prior to the start of the call to ensure adequate time for any software downloads that may be required.

The call will be available for replay via telephone for seven days following the call. To listen to the replay, dial (855) 859-2056 (U.S. and Canada) or (404) 537-3406 (international) using conference ID number 5847908. The archived webcast will be available on Akebia's website after the call has completed.

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. For more information, please visit our website at www.akebia.com, which does not form a part of this release.

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 rate and timing of enrollment of our clinical trials; the timing, availability and presentation of clinical trial data and results; the benefits, including the potential effect on commercial position, of the designs of our studies; the execution of our global Phase 3 program for vadadustat; the timing of market launch of vadadustat; the potential characterization and differentiation information we believe will result from the designs of our studies; potential and anticipated payments from our collaborators, including the timing thereof; timing of regulatory filings and approvals; and expectations regarding financial position, including the period of time our cash resources and collaboration funding will fund our current operating plan. The terms "anticipate," "approach," "believe," "continue," "expect," "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 rate of enrollment in clinical studies of 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; 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 need to be discontinued 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 timing of any additional studies initiated for vadadustat; the actual time it takes to initiate and complete preclinical and clinical studies; the success of competitors in developing product candidates for diseases for which Akebia is currently developing its product candidates; 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 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 fiscal year ended December 31, 2017, 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.

Tables Follow

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

(unaudited)

	Three Months Ended		Twelve Months Ended	
	December 31, 2017	**December 31, 2016**	**December 31, 2017**	**December 31, 2016**
Collaboration revenue	$ 87,316	$ 1,535	$ 177,984	$ 1,535
Operating expenses:				
Research and development	68,382	33,435	230,893	115,785
General and administrative	7,567	6,144	27,008	22,210
Total operating expenses	75,949	39,579	257,901	137,995
Operating income/(loss)	11,367	(38,044)	(79,917)	(136,460)
Other income, net	914	182	3,003	713
Net income/(loss)	$ 12,281	$ (37,862)	$ (76,914)	$ (135,747)
Net income/(loss) per share				
basic	$ 0.26	$ (0.99)	$ (1.77)	$ (3.60)
diluted	$ 0.25	$ (0.99)	$ (1.77)	$ (3.60)
Weighted-average number of common shares				
basic	47,353,166	38,277,100	43,500,795	37,716,949
diluted	49,715,287	38,277,100	43,500,795	37,716,949

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

	December 31, 2017	**December 31, 2016**
Cash, cash equivalents and available for sale securities	$ 317,792	$ 260,343
Working capital	214,007	182,053
Total assets	364,247	300,216
Total stockholders' equity	119,331	68,120

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Source: Akebia Therapeutics, Inc.

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