Akcea Reports Financial Results and Highlights for First Quarter 2018

May 3, 2018

- ***TTR licensing transaction positions Akcea for two rare disease drug launches in 2018***
- ***FDA extended TEGSEDI™ (inotersen) PDUFA date to October 6, 2018***
- ***$445 million in pro forma cash to fund the company through key milestones in 2019***
- ***Conference Call Webcast Thursday, May 3, 4:30 p.m. ET at www.akceatx.com***

CAMBRIDGE, Mass., May 03, 2018 (GLOBE NEWSWIRE) -- Akcea Therapeutics, Inc. (NASDAQ:AKCA), an affiliate of Ionis Pharmaceuticals, Inc., focused on developing and commercializing drugs to treat patients with serious and rare diseases, today reported financial results for the first quarter ended March 31, 2018. The company reported a net loss for the first quarter ended March 31, 2018 of $30 million on a GAAP basis and $23 million on a pro forma basis. Akcea had $245 million of cash, cash equivalents and short-term investments as of March 31, 2018. In April, Akcea received an additional $200 million of cash for the issuance of 10.7 million shares to Ionis, providing additional funds to launch two drugs in 2018 and advance the Company's pipeline, including AKCEA-TTR-L $_{Rx}$.

"With our acquisition of the TTR franchise from Ionis, we accelerated our growth and are now in a position to potentially launch two rare disease drugs this year. The addition of the TTR franchise allows us to efficiently scale our business and provide a strong foundation for future growth," said Paula Soteropoulos, chief executive officer of Akcea. "This past quarter we have been focused on completing the build out of the commercial teams, to launch both inotersen, which we plan to market under the brand name TEGSEDI and volanesorsen, which we plan to market under the brand name WAYLIVRA™. We are also supporting the FCS and hATTR patient communities through early access programs. As we look toward approval of both drugs, we are confident that we are prepared to successfully launch and deliver these drugs to two underserved, rare disease patient communities."

"The FDA has extended the review period for TEGSEDI and has assigned a new PDUFA goal date of October 6, 2018. The FDA determined that they need additional time to review our responses to their standard information requests and we are working closely with them to advance the review of our filing as quickly as possible," said Sarah Boyce, president of Akcea. "Additionally, our discussions with the EU and Canada continue to progress well. We are moving forward at full speed to bring TEGSEDI to people with this devastating and fatal disease as quickly as possible after approval."

"The addition of the TTR franchise has created meaningful value for our shareholders. We are now preparing to realize commercial revenue from two products this year. With the $200 million cash infusion from Ionis, we have approximately $445 million of cash on a pro forma basis, which we believe is sufficient to execute on our key milestones through 2019," said Michael MacLean, chief financial officer of Akcea.

2018 Upcoming Events

- Potential approval and launch of TEGSEDI and WAYLIVRA this year in the US, EU and Canada.
- Report top-line results in the second half of 2018 from a Phase 2b trial of AKCEA-APO(a)-L$_{Rx}$ in patients with high lipoprotein(a), also known as Lp(a).
- Initiate Phase 1 clinical studies of AKCEA-TTR-L$_{Rx}$.

Recent Key Highlights

- Announcement and closing of the partnership with Ionis to commercialize the TTR franchise.
- Addition of Sarah Boyce to Akcea as president and a member of Akcea's board of directors.
- Initiation of the WAYLIVRA global Early Access Program including initiation of the Early Access to Medicines Scheme (EAMS) in the UK for the treatment of people with FCS.
- Presentation of multiple data sets at the International Symposium on Amyloidosis (ISA) including the NEURO-TTR and Open Label Extension Studies for TEGSEDI and the preclinical data set for AKCEA-TTR-L$_{Rx}$.
- Presentation of AKCEA-APOCIII-L$_{Rx}$ Phase 1/2 results at the American College of Cardiology (ACC) Annual Scientific Session and Expo.
- Convening of the first FCS global connection summit where the patient leaders named the first Friday in November as FCS Awareness Day.

Financial Results

All pro forma amounts referred to in this press release exclude non-cash compensation expense related to equity awards. Please refer to the reconciliation of pro forma and GAAP measures, which is provided later in this release.

Revenue
Akcea's revenue for the first quarter ended March 31, 2018 was $17 million. Akcea's revenue to date is entirely related to the Company's collaboration with Novartis comprised of the $75 million upfront payment and the purchase of Ionis stock by Novartis at a premium of $33 million. On January 1, 2018, Akcea adopted ASC 606, *Revenue from Contracts with Customers*, and recorded a cumulative effect adjustment to equity of approximately $12 million as of that date primarily related to the change in measuring progress of the Phase 2 trials for AKCEA-APO(a)-L$_{Rx}$ and AKCEA-APOCIII-L$_{Rx}$.

Operating Expenses
Akcea's operating expenses for the first quarter ended March 31, 2018 on a GAAP basis were $47 million and $41 million on a pro forma basis. These amounts compare to GAAP operating expenses of $69 million and pro forma operating expenses of $66 million for the same period in 2017. Akcea's

pro forma operating expenses decreased for the quarter ended March 31, 2018 compared to the same period in 2017. This decrease is primarily due to the one-time sub-license expense of $48 million paid to Ionis in the first quarter of 2017 related to Akcea's collaboration with Novartis. However, this decrease is reduced by a $23 million increase in Akcea's pro forma operating expenses related to development and commercialization activities in the first quarter ended March 31, 2018 compared to the same period in 2017.

Net Loss

Akcea reported a net loss of $30 million on a GAAP basis for the first quarter ended March 31, 2018 compared to $64 million for the same period in 2017. Akcea reported a pro forma net loss of $23 million for the first quarter ended March 31, 2018 compared to $61 million for the same period in 2017. This is primarily due to the one-time sub-license expense of $48 million paid to Ionis in the first quarter of 2017 related to Akcea's collaboration with Novartis. For the first quarter of 2018, basic and diluted net loss per share of common stock was $0.44.

Balance Sheet

As of March 31, 2018 Akcea had cash, cash equivalents and short-term investments of $245 million compared to $260 million at December 31, 2017. With the $200 million cash infusion from Ionis in April of 2018, Akcea has approximately $445 million on a pro forma basis, which is sufficient to execute on key milestones through 2019.

Conference Call

At 4:30 p.m. Eastern Time today, May 3, 2018, Akcea will conduct a live webcast conference call to discuss this earnings release and related activities. Interested parties may listen to the call by dialing (855) 237-2439, passcode 1497015 or access the webcast at www.akceatx.com. A webcast replay will be available for a limited time at the same address.

ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics, an affiliate of Ionis Pharmaceuticals, Inc. (NASDAQ:IONS), is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea is advancing a mature pipeline of six novel drugs, including TEGSEDI™ (inotersen), WAYLIVRA™ (volanesorsen), AKCEA-APO(a)-L_{Rx}, AKCEA-ANGPTL3-L_{Rx}, AKCEA-APOCIII-L_{Rx}, and AKCEA-TTR-L_{Rx}, all with the potential to treat multiple diseases. All six drugs were discovered by and are being co-developed with Ionis, a leader in antisense therapeutics, and are based on Ionis' proprietary antisense technology. TEGSEDI is under regulatory review in the U.S., EU and Canada for the treatment of hereditary transthyretin amyloidosis (hATTR). WAYLIVRA is under regulatory review in the U.S., EU and Canada for the treatment of familial chylomicronemia syndrome, or FCS, and is currently in Phase 3 clinical development for the treatment of familial partial lipodystrophy, or FPL. Akcea is building the infrastructure to commercialize its drugs globally. Akcea is a global company headquartered in Cambridge, Massachusetts. Additional information about Akcea is available at www.akceatx.com.

FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the business of Akcea Therapeutics, Inc. and the therapeutic and commercial potential of TEGSEDI (inotersen), WAYLIVRA (volanesorsen) and other products in development. Any statement describing Akcea's goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. Akcea's forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Akcea's forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Akcea. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Akcea's programs are described in additional detail in Akcea's annual report on Form 10-K, which is on file with the SEC. Copies of this and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis", "Akcea," "Company," "Companies," "we," "our," and "us" refers to Ionis Pharmaceuticals and/or Akcea Therapeutics.

AKCEA THERAPEUTICS, INC.
SELECTED FINANCIAL INFORMATION
Consolidated Statements of Operations
(In Thousands, Except Per Share Data)

	Three months ended, March 31,	
	2018	2017
R&D Revenue	$17,108	$6,094
Expenses:		
Research and development	27,970	64,794
General and administrative	19,465	4,676
Total operating expenses	47,435	69,470
Loss from operations	(30,327)	(63,376)
Other income (expense):		
Investment income	868	61

Interest expense	-	(541)
Other expense	(168)	-
Loss before income tax expense	(29,627)	(63,856)
Income tax expense	-	-
Net loss	$(29,627)	$(63,856)
Net loss per share of preferred stock, basic and diluted	$ -	$(2.21)
Weighted-average shares of preferred stock outstanding, basic and diluted	-	28,884,540
Net loss per share of common stock, basic and diluted	$(0.44)	$ -
Weighted-average shares of common stock outstanding, basic and diluted	66,616,337	-

AKCEA THERAPEUTICS, INC.
Reconciliation of GAAP to Pro Forma Basis:
Condensed Consolidated Operating Expenses, Loss From Operations, and Net Loss
(In Thousands)

	Three months ended, March 31,	
	2018	2017
	(unaudited)	
As reported operating expenses according to GAAP	$47,435	$69,470
Excluding compensation expense related to equity awards	(6,384)	(3,180)
Pro forma operating expenses	$41,051	$66,290
As reported loss from operations according to GAAP	$(30,327)	$(63,376)
Excluding compensation expense related to equity awards	(6,384)	(3,180)
Pro forma loss from operations	$(23,943)	$(60,196)
As reported net loss according to GAAP	$(29,627)	$(63,856)
Excluding compensation expense related to equity awards	(6,384)	(3,180)
Pro forma net loss	$(23,243)	$(60,676)

Reconciliation of GAAP to Pro Forma Basis
As illustrated in the Selected Financial Information in this press release, pro forma operating expenses, pro forma loss from operations, and pro forma net loss were adjusted from GAAP to exclude compensation expense related to equity awards, which are non-cash expenses. Akcea has regularly reported non-GAAP measures for operating results as pro forma results. These measures are provided as supplementary information and are not a substitute for financial measures calculated in accordance with GAAP. Akcea reports these pro forma results to better enable financial statement users to assess and compare its historical performance and project its future operating results and cash flows. Further, the presentation of Akcea's pro forma results is consistent with how Akcea's management internally evaluates the performance of its operations.

AKCEA THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(In Thousands)

	March 31, 2018	December 31, 2017
Assets:		
Cash and cash equivalents	$69,907	$58,367
Short-term investments	175,028	201,763
Contract receivable	---	5,413
Other current assets	5,628	1,302
Licenses, net	1,191	1,221
Other assets	711	738
Total assets	$252,465	$268,804
Liabilities and stockholders' equity:		
Accounts payable	$2,578	$2,381
Payable to Ionis Pharmaceuticals, Inc.	27,737	14,365
Accrued compensation	3,773	4,083
Accrued liabilities	13,413	7,570
Current portion of deferred revenue	48,866	58,192
Other current liabilities	1,929	1,875
Long-term portion of deferred rent	10	12
Long-term deferred revenue	7,859	12,501
Stockholders' equity	146,300	167,825
Total liabilities and stockholders' equity	$252,465	$268,804

Media and Investor Contact:
Kathleen Gallagher
Head of Corporate Communications and Investor Relations
617.207.8509
kgallagher@akceatx.com

Primary Logo

Source: Akcea Therapeutics, Inc.