

## Akcea Reports Financial Results and Highlights for Second Quarter 2018

### **TEGSEDI™ (inotersen) Approved in European Union**

### **\$382 million to fund the company through key milestones in 2019**

### **Conference Call Webcast Monday, August 6, 4:30 p.m. ET at [www.akceatx.com](http://www.akceatx.com)**

CAMBRIDGE, Mass., Aug. 06, 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 second quarter ended June 30, 2018. The company reported a net loss for the three and six months ended June 30, 2018 on a GAAP basis of \$62 million and \$92 million, respectively, and on a pro forma basis of \$50 million and \$73 million, respectively. Akcea had \$382 million of cash, cash equivalents and short-term investments as of June 30, 2018.

“This is an incredibly exciting time for Akcea with TEGSEDI now approved in Europe and upcoming PDUFA dates in the US for WAYLIVRA™ (volanesorsen) and TEGSEDI. We are ready to launch TEGSEDI first in Germany post the summer holidays, and in the US, we are prepared to launch both products quickly upon approval. We are poised to deliver these drugs to two underserved, rare disease patient communities in our three initial key regions. We’ve also accelerated global access to TEGSEDI and WAYLIVRA in Latin America through our partnership with PTC Therapeutics,” said Paula Soteropoulos, chief executive officer of Akcea. “In addition, with the upcoming top line results from the AKCEA-APO(a)-LRx Phase 2 study, we have the potential to make a significant step forward for patients with cardiovascular disease caused by elevated levels of lipoprotein a, or Lp(a). With the largest efficacy, safety and treatment duration database to date, these results could be a game changer for the ligand conjugated antisense, or LICA, technology platform and we look forward to sharing them later this year.”

“Our financial results reflect the investments we have made as we prepare to launch TEGSEDI and WAYLIVRA in multiple markets this year,” said Michael MacLean, chief financial officer of Akcea. “Our operating expenses are in line with our expectations and, with over \$380 million in cash, we are well positioned to execute on all of our strategic priorities through 2019.”

#### Upcoming Events

- Launch of TEGSEDI in Europe, starting with Germany.
- Potential approval and launch of WAYLIVRA in the US, EU and Canada.
- Potential approval and launch of TEGSEDI in the US and Canada.
- Report top-line results in the second half of 2018 from a Phase 2 trial of AKCEA-APO(a)-LRx in patients with cardiovascular disease caused by elevated lipoprotein(a), or Lp(a).
- Initiate Phase 1 clinical studies of AKCEA-TTR-LRx.

#### Recent Key Highlights

- Approval of TEGSEDI in the European Union for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR.
- Vote in favor of supporting approval of WAYLIVRA for treatment of Familial Chylomicronemia Syndrome, or FCS, by the FDA Advisory Committee.
- Announcement and closing of the partnership with Ionis to commercialize the TTR franchise.
- Announcement and closing of the partnership with PTC Therapeutics to commercialize TEGSEDI and WAYLIVRA in Latin America.
- Results published from the pivotal study of TEGSEDI in the July 5th edition of the *New England Journal of Medicine*.
- Appointment of industry leader Dr. Richard Moscicki to Akcea’s Board of Directors.
- Launch of hATTR Compass, a genetic testing program for people with suspected hATTR amyloidosis.
- Completion of landmark RE-FOCUS study to assess disease burden in people living with FCS.

#### 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 three and six months ended June 30, 2018 was \$18 million and \$35 million.

Akcea's revenue to date is entirely related to the Company's collaboration with Novartis, executed in 2017, and the associated amortization 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.

### *Operating Expenses*

Akcea's operating expenses for the three and six months ended June 30, 2018 on a GAAP basis were \$82 million and \$129 million, respectively, and on a pro forma basis were \$70 million and \$111 million, respectively. These amounts compare to GAAP operating expenses of \$25 million and \$95 million and pro forma operating expenses of \$21 million and \$88 million for the same periods in 2017. These increases primarily relate to development costs, including AKCEA-TTR-LRx and commercialization costs for TEGSEDI and WAYLIVRA in the second quarter ended June 30, 2018 compared to the same period in 2017.

### *Net Loss*

Akcea reported a net loss of \$62 million and \$92 million on a GAAP basis for the three and six months ended June 30, 2018, respectively, compared to \$20 million and \$84 million for the same periods in 2017. Akcea reported a pro forma net loss of \$50 million and \$73 million for the three and six months ended June 30, 2018, respectively, compared to \$16 million and \$77 million for the same periods in 2017. This increase in pro forma net loss was primarily due to increased operating expenses related to development and commercialization costs for TEGSEDI and WAYLIVRA. For the three and six months ended June 30, 2018, basic and diluted net loss per share of common stock [owned by Ionis] was \$0.72 and \$1.19, respectively. For the three and six months ended June 30, 2018, basic and diluted net loss per share of common stock [owned by others] was \$0.85 and \$1.33, respectively.

### *Balance Sheet*

As of June 30, 2018 Akcea had cash, cash equivalents and short-term investments of \$382 million compared to \$260 million at December 31, 2017 which is sufficient to execute on key milestones through 2019.

### Conference Call

At 4:30 p.m. Eastern Time today, August 6, 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 9969807 or access the webcast at [www.akceatx.com](http://www.akceatx.com). A webcast replay will be available for a limited time at the same address.

### ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics, Inc., 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)-LRx, AKCEA-ANGPTL3-LRx, AKCEA-APOCIII-LRx, and AKCEA-TTR-LRx, 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 approved in the E.U. for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) and is currently under regulatory review in the US and Canada. WAYLIVRA is under regulatory review in the U.S., E.U. and Canada for the treatment of familial chylomicronemia syndrome, or FCS, and is currently in Phase 3 clinical development for the treatment of people with 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](http://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.

Ionis Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics™ TEGSEDI™ and WAYLIVRA™ are trademarks of Akcea Therapeutics, Inc.

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

	Three months ended		Six months ended	
	June 30,		June 30,	
	2018	2017	2018	2017
	(unaudited)		(unaudited)	
R&D Revenue	18,321	5,713	35,429	11,807
Expenses:				
Research and development	39,457	18,487	67,427	83,282
General and administrative	42,287	6,915	61,752	11,590
Total operating expenses	81,744	25,402	129,179	94,872
Loss from operations	(63,423 )	(19,689 )	(93,750 )	(83,065 )
Other income (expense):				
Investment income	1,546	245	2,414	308
Interest expense	-	(965 )	-	(1,507 )
Other income (expense)	45	50	(123 )	50
Loss before income tax expense	(61,832 )	(20,359 )	(91,459 )	(84,214 )
Income tax expense	(214 )	-	(214 )	-
Net loss	(62,046 )	(20,359 )	(91,673 )	(84,214 )
Net loss per share of preferred stock, basic and diluted	-	(0.70 )	-	(2.92 )
Weighted-average shares of preferred stock outstanding, basic and diluted	-	28,884,540	-	28,884,540
Net loss per share of common stock owned by Ionis, basic and diluted	(0.72 )	-	(1.19 )	-
Weighted-average shares of common stock outstanding owned by Ionis, basic and diluted	60,832,494	-	53,182,685	-
Net loss per share of common stock owned by others, basic and diluted	(0.85 )	-	(1.33 )	-
Weighted-average shares of common stock outstanding owned by others, basic and diluted	21,492,157	-	21,332,650	-

Reconciliation of GAAP to Pro Forma Basis:

Condensed Consolidated Operating Expenses, Loss from Operations, and Net Loss

(In Thousands)

	Three months ended		Six months ended	
	June 30,		June 30,	
	2018	2017	2018	2017
	(unaudited)		(unaudited)	
As reported operating expenses according to GAAP	81,744	25,402	129,179	94,872
Excluding compensation expense related to equity awards	(12,126 )	(3,942 )	(18,509 )	(7,122 )
Pro forma operating expenses	69,618	21,460	110,670	87,750
As reported loss from operations according to GAAP	(63,423 )	(19,689 )	(93,750 )	(83,065 )
Excluding compensation expense related to equity awards	(12,126 )	(3,942 )	(18,509 )	(7,122 )
Pro forma loss from operations	(51,297 )	(15,747 )	(75,241 )	(75,943 )
As reported net loss according to GAAP	(62,046 )	(20,359 )	(91,673 )	(84,214 )
Excluding compensation expense related to equity awards	(12,126 )	(3,942 )	(18,509 )	(7,122 )
Pro forma net loss	(49,920 )	(16,417 )	(73,164 )	(77,092 )

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)

	June 30,	December 31,
	2018	2017
	(unaudited)	
Assets:		
Cash and cash equivalents	216,840	58,367
Short-term investments	165,011	201,763
Contract receivable	3,295	5,413
Other current assets	3,575	1,302
Property, plant and equipment, net	1,523	77
Licenses, net	1,714	1,221
Deposits and other assets	3,047	661
Total assets	395,005	268,804
Liabilities and stockholders' equity (deficit)		

Accounts payable	2,706	2,381
Payable to Ionis Pharmaceuticals, Inc.	27,137	14,365
Accrued compensation	4,548	4,083
Accrued liabilities	25,969	7,570
Current portion of deferred revenue	35,713	58,192
Other current liabilities	1,138	1,875
Long-term portion of deferred rent	1,551	12
Long-term portion of deferred revenue	5,839	12,501
Stockholders' equity	290,404	167,825
Total liabilities and stockholders' equity	395,005	268,804

Media and Investor Contact:

Kathleen Gallagher  
Vice President of Corporate Communications and Investor Relations  
617.207.8509  
[kgallagher@akceatx.com](mailto:kgallagher@akceatx.com)

---

<https://ir.akceatx.com/2018-08-06-Akcea-Reports-Financial-Results-and-Highlights-for-Second-Quarter-2018>