May 5, 2017

Alnylam Pharmaceuticals Reports First Quarter 2017 Financial Results and Highlights Recent Period Activity

- Advanced Industry-Leading RNAi Therapeutics Pipeline with Eight Clinical Programs, Including Three Programs in Late-Stage Development -

- Maintained Strong Balance Sheet with $962 Million in Cash and Plans to End 2017 with Greater than $700 Million in Cash -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the first quarter 2017, and highlighted recent progress in advancing its pipeline.

"2017 promises to be a pivotal year for Alnylam. With our patisiran program, we look forward to the read-out of our APOLLO Phase 3 study and, if the data are positive, our first NDA filing. In addition, we expect to advance three additional programs into Phase 3 trials: fitusiran, givosiran, and - with our partners at The Medicines Company - inclisiran," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "In the first quarter and recent period, we made great progress with each of these programs, with positive clinical data reported from patisiran, fitusiran, and inclisiran, and achievement of PRIME designation for givosiran. As we look towards the rest of 2017, we believe our upcoming milestones put us on track to meet our 'Alnylam 2020' goals of becoming a multi-product, commercial-stage company with a deep and sustainable clinical development pipeline by the end of 2020."

First Quarter 2017 and Recent Significant Corporate Highlights

- Advanced patisiran, an investigational RNAi therapeutic for the treatment of polyneuropathy due to hereditary ATTR (hATTR) amyloidosis, with final 24-month data from the Phase 2 open-label extension (OLE) study presented at the American Academy of Neurology meeting and top-line results from the APOLLO Phase 3 study expected in mid-2017.
- Advanced fitusiran, an investigational RNAi therapeutic for the treatment of hemophilia and rare bleeding disorders, with positive new data presented at the 2017 European Association for Haemophilia and Allied Disorders (EAHAD) meeting.
- Advanced givosiran (ALN-AS1), an investigational RNAi therapeutic for the treatment of the acute hepatic porphyrias, with acceptance into the European Medicines Agency's PRIME program.
- Alnylam's partner, The Medicines Company, announced positive final results from the ORION-1 Phase 2 study of inclisiran, an investigational RNAi therapeutic for the treatment of hypercholesterolemia, at the American College of Cardiology's 66th Annual Scientific Session.
  - The Medicines Company initiated the ORION-2 study of inclisiran in patients with Homozygous Familial Hypercholesterolemia (HoFH) as well as the ORION-3 study, a Phase 2 open-label cross-over extension study for patients completing the ORION-1 study.
  - In addition, The Medicines Company and Alnylam announced agreement with the FDA on the Phase 3 clinical program for inclisiran.

Upcoming Events in Early and Mid-2017[1]

- Alnylam announces today that it plans to host its 4th Annual RNAi Roundtable Series during the summer. This series will consist of webinars designed to inform attendees of the latest progress and upcoming milestones for many of the company's investigational RNAi therapeutic programs. More details for the series are forthcoming.
- The Company also announces today that it plans to present additional clinical data from the Phase 1 study of givosiran at the 2017 International Congress of Porphyrins and Porphyrias (ICPP), being held June 25 - 28, 2017, in Bordeaux, France, in an oral presentation on Monday, June 26 at 11:45am Central European Time (5:45 am ET).
- In addition, the Company announces that it plans to present additional data from the Phase 2 OLE study of fitusiran at the International Society on Thrombosis and Haemostasis (ISTH) 2017 Congress, being held July 8 - 13, 2017, in

Berlin, Germany, in an oral presentation on Monday, July 10 at 2:45 pm Central European Time (8:45 am ET).

- In early 2017, Alnylam plans to initiate the ATLAS Phase 3 program for fitusiran.
- In mid-2017, Alnylam plans to report top-line results from the APOLLO Phase 3 study of patisiran.
- Also in mid-2017, The Medicines Company plans to initiate a Phase 3 study of inclisiran in patients with atherosclerotic cardiovascular disease (ASCVD).

Financials

"Alnylam continues to maintain a strong balance sheet, ending the first quarter of 2017 with approximately $962.2 million in cash, including restricted investments," said Michael Mason, Vice President, Finance and Treasurer. "Our financial strength allows us to continue to invest in a broad pipeline of investigational RNAi therapeutics, aligned with achievement of our 'Alnylam 2020' goals and strategy. As for 2017 guidance, we remain on track to end 2017 with greater than $700 million in cash, including $150.0 million in restricted investments."

Cash and Investments
At March 31, 2017, Alnylam had cash, cash equivalents and fixed income marketable securities, and restricted investments of $962.2 million, as compared to $1.09 billion at December 31, 2016.

GAAP Net Loss
The net loss according to accounting principles generally accepted in the U.S. (GAAP) for the first quarter of 2017 was $107.3 million, or $1.25 per share on both a basic and diluted basis (including $15.7 million, or $0.18 per share of non-cash stock-based compensation expense), as compared to a net loss of $103.0 million, or $1.21 per share on both a basic and diluted basis (including $23.5 million, or $0.28 per share of non-cash stock-based compensation expense), for the same period in the previous year.

Revenues
Revenues were $19.0 million in the first quarter of 2017, as compared to $7.3 million in the first quarter of 2016. Revenues for the first quarter of 2017 included $12.3 million from the company's alliance with Sanofi Genzyme, $6.4 million from the company's alliance with The Medicines Company, and $0.3 million from other sources. The increase in revenues in the quarter ended March 31, 2017 as compared to the prior year period was due primarily to higher revenue from the company's alliance with Sanofi Genzyme.

Research and Development Expenses
Research and development (R&D) expenses were $87.0 million in the first quarter of 2017, which included $8.7 million of non-cash stock-based compensation, as compared to $96.3 million in the first quarter of 2016, which included $14.4 million of non-cash stock-based compensation. The decrease in R&D expenses for the quarter ended March 31, 2017 as compared to the prior year period was due in part to a decrease in non-cash stock-based compensation expense as a result of the vesting of certain performance-based stock option awards during the first quarter of 2016 upon completion of enrollment in our APOLLO Phase 3 clinical trial for patisiran. In addition, external services expenses decreased during the quarter ended March 31, 2017 as compared to the prior year period due to reduced pre-clinical activities.

General and Administrative Expenses
General and administrative (G&A) expenses were $38.5 million in the first quarter of 2017, which included $7.0 million of non-cash stock-based compensation, as compared to $21.1 million in the first quarter of 2016, which included $9.1 million of non-cash stock-based compensation. The increase in G&A expenses for the quarter ended March 31, 2017 as compared to the prior year period was due primarily to an increase in headcount to support corporate growth and to prepare for the potential launch of our first commercial product.

Conference Call Information
Management will provide an update on the company, discuss first quarter 2017 results, and discuss expectations for the future via conference call on Friday, May 5, 2017 at 8:30 a.m. ET. To access the call, please dial 877-312-7507 (domestic) or 631-813-4828 (international) five minutes prior to the start time and refer to conference ID 13425320. A replay of the call will be available beginning at 11:30 a.m. ET on May 5, 2017. To access the replay, please dial 855-859-2056 (domestic) or 404-537-3406 (international), and refer to conference 13425320.

Sanofi Genzyme Alliance
In January 2014, Alnylam and Sanofi Genzyme, the specialty care global business unit of Sanofi, formed an alliance to accelerate and expand the development and commercialization of RNAi therapeutics across the world. The alliance is structured as a multi-product geographic alliance in the field of rare diseases. Alnylam retains product rights in the United States, Canada and Western Europe, while Sanofi Genzyme obtained the right to access certain programs in Alnylam's current and future Genetic Medicines pipeline in the rest of the world (ROW) through the end of 2019, together with certain

broader co-development/co-commercialization rights and global rights for certain products. In the case of patisiran, Alnylam will advance the product in the United States, Canada and Western Europe, while Sanofi Genzyme will advance the product in the ROW. In the case of fitusiran, Sanofi Genzyme has elected to opt in to co-develop (through Sanofi R&D) and co-commercialize fitusiran in the United States, Canada and Western Europe, in addition to developing and commercializing fitusiran in its ROW territories.

About RNAi
RNAi (RNA interference) is a revolution in biology, representing a breakthrough in understanding how genes are turned on and off in cells, and a completely new approach to drug discovery and development. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and represents one of the most promising and rapidly advancing frontiers in biology and drug discovery today which was awarded the 2006 Nobel Prize for Physiology or Medicine. RNAi is a natural process of gene silencing that occurs in organisms ranging from plants to mammals. By harnessing the natural biological process of RNAi occurring in our cells, the creation of a major new class of medicines, known as RNAi therapeutics, is on the horizon. Small interfering RNA (siRNA), the molecules that mediate RNAi and comprise Alnylam's RNAi therapeutic platform, target the cause of diseases by potently silencing specific mRNAs, thereby preventing disease-causing proteins from being made. RNAi therapeutics have the potential to treat disease and help patients in a fundamentally new way.

About LNP Technology
Alnylam has licenses to Arbutus LNP intellectual property for use in RNAi therapeutic products using LNP technology.

About Alnylam Pharmaceuticals
Alnylam (Nasdaq: ALNY) is leading the translation of RNA interference (RNAi) into a whole new class of innovative medicines with the potential to transform the lives of patients who have limited or inadequate treatment options. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of debilitating diseases. Founded in 2002, Alnylam is delivering on a bold vision to turn scientific possibility into reality, with a robust discovery platform and deep pipeline of investigational medicines, including three product candidates that are in late-stage development or will be in 2017. Looking forward, Alnylam will continue to execute on its "Alnylam 2020" strategy of building a multi-product, commercial-stage biopharmaceutical company with a sustainable pipeline of RNAi-based medicines. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam.

Alnylam Forward-Looking Statements
Various statements in this release concerning Alnylam's future expectations, plans and prospects, including without limitation, Alnylam's views with respect to the potential for RNAi therapeutics, including patisiran, fitusiran, givosiran, and inclisiran, its expectations regarding the timing of clinical studies and the presentation of clinical data, including for studies for patisiran, fitusiran, givosiran, and inclisiran, its expectations regarding the potential filing of an NDA for patisiran if the APOLLO Phase 3 study is positive, its expected cash position as of December 31, 2017, its expectations regarding its STAr pipeline growth strategy, and its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results and future plans may differ materially from those indicated by these forward-looking statements as a result of various important risks, uncertainties and other factors, including, without limitation, Alnylam's ability to discover and develop novel drug candidates and delivery approaches, successfully demonstrate the efficacy and safety of its product candidates, the pre-clinical and clinical results for its product candidates, which may not be replicated or continue to occur in other subjects or in additional studies or otherwise support further development of product candidates for a specified indication or at all, actions or advice of regulatory agencies, which may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional pre-clinical and/or clinical testing, delays, interruptions or failures in the manufacture and supply of our product candidates, obtaining, maintaining and protecting intellectual property, Alnylam's ability to enforce its intellectual property rights against third parties and defend its patent portfolio against challenges from third parties, obtaining and maintaining regulatory approval, pricing and reimbursement for products, progress in establishing a commercial and ex-United States infrastructure, competition from others using technology similar to Alnylam's and others developing products for similar uses, Alnylam's ability to manage its growth and operating expenses, obtain additional funding to support its business activities, and establish and maintain strategic business alliances and new business initiatives, Alnylam's dependence on third parties for development, manufacture and distribution of products, the outcome of litigation, the risk of government investigations, and unexpected expenditures, as well as those risks more fully discussed in the "Risk Factors" filed with Alnylam's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) and in other filings that Alnylam makes with the SEC. In addition, any forward-looking statements represent Alnylam's views only as of today and should not be relied upon as representing its views as of any subsequent date. Alnylam explicitly disclaims any obligation, except to the extent required by law, to update any forward-looking statements.

The scientific information referenced in this news release relating to Alnylam's investigational therapeutics is preliminary and investigative. None of Alnylam's investigational therapeutics, including inclisiran which is partnered with The Medicines Company, have been approved by the U.S. Food and Drug Administration, European Medicines Agency, or any other

regulatory authority and no conclusions can or should be drawn regarding the safety or effectiveness of these therapeutics.

ALNYLAM PHARMACEUTICALS, INC.
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands, except per share amounts)

	Three Months Ended March 31,	
	2017	**2016**
Net revenues from collaborators	$ 18,960	$ 7,345
Operating expenses:		
Research and development	86,984	96,273
General and administrative	38,487	21,100
Total operating expenses	125,471	117,373
Loss from operations	(106,511)	(110,028)
Other income (expense):		
Interest income	2,128	1,813
Other (expense) income	(2,907)	5,241
Total other (expense) income	(779)	7,054
Net loss	$(107,290)	$(102,974)
Net loss per common share - basic and diluted	$ (1.25)	$ (1.21)
Weighted-average common shares used to compute basic and diluted net loss per common share	86,027	85,277
Comprehensive loss:		
Net loss	$(107,290)	$(102,974)
Unrealized loss on marketable securities, net of tax	(1,936)	(8,224)
Reclassification adjustment for realized loss (gain) on marketable securities included in net loss	1,549	(5,156)
Comprehensive loss	$(107,677)	$(116,354)

ALNYLAM PHARMACEUTICALS, INC.
UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except share amounts)

	March 31, 2017	December 31, 2016
Cash, cash equivalents and fixed income marketable securities	$ 812,227	$ 942,601
Restricted investments	150,000	150,000
Billed and unbilled collaboration receivables	19,457	23,334
Prepaid expenses and other assets	22,280	32,303
Property, plant and equipment, net	129,962	114,572
Total assets	$ 1,133,926	$ 1,262,810
Accounts payable, accrued expenses and other liabilities	$ 60,397	$ 99,650
Total deferred revenue	82,804	82,932
Total deferred rent	9,824	10,007
Long-term debt	150,000	150,000
Total stockholders' equity (86.1 million and 85.9 million common shares issued and outstanding and at March 31, 2017 and December 31, 2016, respectively)	830,901	920,221
Total liabilities and stockholders' equity	$ 1,133,926	$ 1,262,810

This selected financial information should be read in conjunction with the consolidated financial statements and notes

thereto included in Alnylam's Annual Report on Form 10-K which includes the audited financial statements for the year ended December 31, 2016.

[1] Early is Q1-Q2, Mid is Q2-Q3, and Late is Q3-Q4

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Alnylam Pharmaceuticals, Inc.
Christine Regan Lindenboom, 617-682-4340
(Investors and Media)
or
Josh Brodsky, 617-551-8276
(Investors)

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