



February 8, 2017

## Alnylam Pharmaceuticals Reports Fourth Quarter and Full Year 2016 Financial Results and Highlights Recent Period Activity

- *Advanced Industry-Leading RNAi Therapeutics Pipeline with Eight Clinical Programs, Including Three Programs in Late-Stage Development; Discontinued Revusiran Development -*
- *Maintained Strong Balance Sheet with \$1.1 Billion in Cash and Expects 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 fourth quarter and full year 2016, and highlighted recent progress in advancing its pipeline.

"We continue to advance a broad pipeline of investigational RNAi therapeutics - including 8 programs in clinical development - across a wide range of disease indications with high unmet need. In the fourth quarter of 2016 and recent period, we reported positive clinical results with fitusiran for hemophilia, givosiran for porphyria, and ALN-CC5 for complement-mediated diseases. In addition, our partners at The Medicines Company, reported positive interim clinical data with inclisiran for hypercholesterolemia. On the other hand, we were disappointed to announce the discontinuation of our revusiran program due to safety findings, although our investigation to date indicates the findings are unique to this program and the clinical setting," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "2017 promises to be a pivotal year for Alnylam, with our first Phase 3 data read out - APOLLO for patisiran - and, if positive, our first NDA filing. In addition, we plan to advance three additional programs into Phase 3 trials. We believe all of these important 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."

### Fourth Quarter 2016 and Recent Significant Corporate Highlights

- Advanced patisiran for the treatment of polyneuropathy due to hereditary ATTR (hATTR) amyloidosis, with APOLLO Phase 3 data expected in mid-2017.
- Announced decision to discontinue development of revusiran, an investigational RNAi therapeutic that was being developed for the treatment of cardiomyopathy due to hATTR amyloidosis.
- Advanced fitusiran for the treatment of hemophilia and rare bleeding disorders (RBD), with positive [new data](#) presented at the 2016 American Society of Hematology (ASH) meeting and [additional data](#) presented at the 2017 European Association for Haemophilia and Allied Disorders (EAHAD) meeting.
  - Alnylam and partner Sanofi Genzyme announced that Sanofi Genzyme elected to exercise its right to co-develop and co-promote fitusiran with Alnylam in the United States, Canada and Western Europe. Sanofi Genzyme retains commercial rights for rest of world.
- Advanced givosiran (ALN-AS1) for the treatment of acute hepatic porphyrias with positive [initial clinical activity results](#) reported at the 2016 ASH meeting from Phase 1 study in acute intermittent porphyria (AIP) patients with recurring porphyria attacks.
  - Alnylam and partner Sanofi Genzyme announced that Sanofi Genzyme has elected not to opt in to develop and commercialize givosiran, providing Alnylam with full global control of the program for further development and potential commercialization.
- Alnylam's partner, The Medicines Company, announced positive results from the Day 90 interim analysis at the 2016 American Heart Association meeting and top-line Day 180 results for all 501 patients in the ongoing ORION-1 Phase 2 study of inclisiran (also known as ALN-PCSsc and PCSK9si), an investigational RNAi therapeutic for the treatment of hypercholesterolemia.
  - 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.
- Reported [updated clinical results](#) at the 2016 ASH meeting from Phase 1/2 study of ALN-CC5 in patients with paroxysmal nocturnal hemoglobinuria (PNH).
  - Alnylam and partner Sanofi Genzyme announced that Sanofi Genzyme has elected not to opt in to develop and

commercialize ALN-CC5, providing Alnylam with full global control of the program for further development and potential commercialization.

- | Reported positive [initial clinical results](#) from Phase 1 study of ALN-TTRsc02, an ESC-GalNAc conjugate targeting TTR for hATTR amyloidosis.

## Upcoming Events in Early and Mid-2017

- | Alnylam announces today that it plans to present complete 24-month data and additional analyses from the Phase 2 OLE study of patisiran at the American Academy of Neurology (AAN) 69<sup>th</sup> Annual Meeting, being held April 22 - 28, 2017 in Boston, Massachusetts, in an oral presentation on Wednesday, April 26 at 8:24 am ET.
- | In addition, The Medicines Company plans to present full safety and efficacy data from the ORION-1 Phase 2 study of inclisiran, with six- to nine-month follow-up for all patients in the study, at the American College of Cardiology's 66<sup>th</sup> Annual Scientific Session, to be held March 17 - 19, 2017, in Washington, D.C., in a late-breaker oral presentation on Friday, March 17, at 1:30 pm ET.
- | Also 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;
  - | Present additional data from the Phase 2 OLE study of fitusiran, likely at the International Society on Thrombosis and Haemostasis (ISTH) 2017 Congress, being held July 8 - 13, 2017, in Berlin, Germany;
  - | Present additional data from Part C of the Phase 1 study of givosiran, likely at the 2017 International Congress of Porphyrins and Porphyrrias (ICPP), being held June 25 - 28, 2017, in Bordeaux, France; and,
  - | The Medicines Company plans to initiate the Phase 3 program for inclisiran.

## Financials

"Alnylam continues to maintain a strong balance sheet, ending 2016 with approximately \$1.1 billion 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. As for 2017 guidance, we expect to end 2017 with greater than \$700 million in cash, including \$150.0 million in restricted investments."

### *Cash and Investments*

At December 31, 2016, Alnylam had cash, cash equivalents and marketable securities, and restricted investments of \$1.09 billion, as compared to cash, cash equivalents and marketable securities of \$1.28 billion at December 31, 2015.

### *GAAP Net Loss*

The net loss according to accounting principles generally accepted in the U.S. (GAAP) for the fourth quarter of 2016 was \$112.9 million, or \$1.32 per share on both a basic and diluted basis (including \$20.7 million, or \$0.24 per share of non-cash stock-based compensation expense), as compared to a net loss of \$90.7 million, or \$1.07 per share on both a basic and diluted basis (including \$15.5 million, or \$0.18 per share of non-cash stock-based compensation expense), for the same period in the previous year. For the year ended December 31, 2016, the net loss was \$410.1 million, or \$4.79 per share on both a basic and diluted basis (including \$75.5 million, or \$0.88 per share of non-cash stock-based compensation expense), as compared to a net loss of \$290.1 million, or \$3.45 per share on both a basic and diluted basis (including \$45.8 million, or \$0.55 per share of non-cash stock-based compensation expense), for prior year.

### *Revenues*

Revenues were \$17.5 million in the fourth quarter of 2016, as compared to \$7.6 million in the fourth quarter of 2015. Revenues for the fourth quarter of 2016 included \$14.8 million from the company's alliance with Sanofi Genzyme, \$2.6 million from the company's alliance with The Medicines Company, and \$0.1 million from other sources. Revenues were \$47.2 million for the year ended December 31, 2016, as compared to \$41.1 million for the prior year. Revenues for year ended December 31, 2016 included \$32.0 million from the company's alliance with Sanofi Genzyme, \$11.2 million from the company's alliance with The Medicines Company, and \$4.0 million from other sources. The increase in revenues in the quarter and year ended December 31, 2016 as compared to the prior year periods was due primarily to higher revenue from the company's agreement with Sanofi Genzyme.

### *Research and Development Expenses*

Research and development (R&D) expenses were \$105.0 million in the fourth quarter of 2016, which included \$10.0 million of non-cash stock-based compensation, as compared to \$82.8 million in the fourth quarter of 2015, which included \$9.3

million of non-cash stock-based compensation. R&D expenses were \$382.4 million for the year ended December 31, 2016, which included \$42.9 million of non-cash stock-based compensation, as compared to \$276.5 million for the prior year, which included \$27.1 million of non-cash stock-based compensation. The increase in R&D expenses for the quarter and year ended December 31, 2016 as compared to the prior year periods was due primarily to additional clinical trial and manufacturing and external services expenses resulting from the advancement of the company's Genetic Medicine pipeline. In addition, compensation and related expenses and non-cash stock-based compensation expenses increased during the quarter and year ended December 31, 2016 as compared to the prior year periods as a result of an increase in headcount during the period as the company expands its pipeline into later-stage development.

#### *General and Administrative Expenses*

General and administrative (G&A) expenses were \$27.9 million in the fourth quarter of 2016, which included \$10.7 million of non-cash stock-based compensation, as compared to \$17.2 million in the fourth quarter of 2015, which included \$6.3 million of non-cash stock-based compensation. General and administrative (G&A) expenses were \$89.4 million for the year ended December 31, 2016, which included \$32.6 million of non-cash stock-based compensation, as compared to \$60.6 million for the prior year, which included \$18.7 million of non-cash stock-based compensation. The increase in G&A expenses for the quarter and year ended December 31, 2016 as compared to the prior year periods was due primarily to an increase in compensation and related expenses and non-cash stock-based compensation expenses. In addition, consulting and professional services expense increased during the quarter and year ended December 31, 2016 as compared to the prior year periods as a result of increased general business activities.

#### *2017 Financial Guidance*

Alnylam expects that its cash, cash equivalents and marketable securities, and restricted investments balance will be greater than \$700 million at December 31, 2017.

#### **Conference Call Information**

Management will provide an update on the company, discuss fourth quarter 2016 results, and discuss expectations for the future via conference call on Wednesday, February 8, 2017 at 4:30 p.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 57758793. A replay of the call will be available beginning at 7:30 p.m. ET on February 8, 2017. To access the replay, please dial 855-859-2056 (domestic) or 404-537-3406 (international), and refer to conference 57758793.

#### **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 is a biopharmaceutical company developing novel therapeutics based on RNA interference, or RNAi. The company is leading the translation of RNAi as a new class of innovative medicines. Alnylam's pipeline of investigational RNAi therapeutics is focused in 3 Strategic Therapeutic Areas (STAr): Genetic Medicines, with a broad pipeline of RNAi therapeutics for the treatment of rare diseases; Cardio-Metabolic Disease, with a pipeline of RNAi therapeutics toward genetically validated, liver-expressed disease targets for unmet needs in cardiovascular and metabolic diseases; and

Hepatic Infectious Disease, with a pipeline of RNAi therapeutics that address the major global health challenges of hepatic infectious diseases. In early 2015, Alnylam launched its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics as a whole new class of innovative medicines. Specifically, by the end of 2020, Alnylam expects to achieve a company profile with 3 marketed products, 10 RNAi therapeutic clinical programs - including 4 in late stages of development - across its 3 STArS. The company's demonstrated commitment to RNAi therapeutics has enabled it to form major alliances with leading companies including Ionis, Novartis, Roche, Takeda, Merck, Monsanto, The Medicines Company, and Sanofi Genzyme. In addition, Alnylam holds an equity position in Regulus Therapeutics Inc., a company focused on discovery, development, and commercialization of microRNA therapeutics. Alnylam scientists and collaborators have published their research on RNAi therapeutics in over 200 peer-reviewed papers, including many in the world's top scientific journals such as *Nature*, *Nature Medicine*, *Nature Biotechnology*, *Cell*, *New England Journal of Medicine*, and *The Lancet*. Founded in 2002, Alnylam maintains headquarters in Cambridge, Massachusetts. For more information about Alnylam's pipeline of investigational RNAi therapeutics, please visit [www.alnylam.com](http://www.alnylam.com).

## 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, inclisiran, and ALN-TTRsc02, 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 ongoing, comprehensive evaluation of the revusiran safety findings and any indications or tentative conclusions based on the evaluation to date, its expected cash position as of December 31, 2017, its expectations regarding its STAr pipeline growth strategy, its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics, and its plans regarding the pursuit of pre-clinical programs 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 Quarterly Report on Form 10-Q 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 discussed in this news release relating to Alnylam's investigational therapeutics is preliminary and investigative. None of Alnylam's investigational therapeutics 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 December 31,		Year Ended December 31,	
	2016	2015	2016	2015
<b>Net revenues from collaborators</b>	\$ 17,454	\$ 7,551	\$ 47,159	\$ 41,097
<b>Operating expenses:</b>				

Research and development	105,011	82,835	382,392	276,495
General and administrative	27,876	17,228	89,354	60,610
Total operating expenses	132,887	100,063	471,746	337,105
Loss from operations	(115,433)	(92,512)	(424,587)	(296,008)
<b>Other income:</b>				
Interest income	2,199	1,616	8,308	5,859
Other income	300	175	6,171	76
Total other income	2,499	1,791	14,479	5,935
Net loss	\$(112,934)	\$(90,721)	\$(410,108)	\$(290,073)
Net loss per common share - basic and diluted	\$ (1.32)	\$ (1.07)	\$ (4.79)	\$ (3.45)
Weighted-average common shares used to compute basic and diluted net loss per common share	85,843	84,871	85,596	83,992
<b>Comprehensive loss:</b>				
Net loss	\$(112,934)	\$(90,721)	\$(410,108)	\$(290,073)
Unrealized (loss) gain on marketable securities, net of tax	(5,502)	11,588	(30,833)	(44,394)
Reclassification adjustment for realized gain on marketable securities included in net loss	(161)	—	(6,977)	—
Comprehensive loss	\$(118,597)	\$(79,133)	\$(447,918)	\$(334,467)

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

	December 31, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	\$ 942,601	\$ 1,280,951
Restricted investments	150,000	—
Billed and unbilled collaboration receivables	23,334	8,298
Prepaid expenses and other assets	23,306	18,030
Property and equipment, net	114,572	27,812
Investment in equity securities of Regulus Therapeutics Inc.	8,997	51,419
<b>Total assets</b>	<b>\$ 1,262,810</b>	<b>\$ 1,386,510</b>
Accounts payable, accrued expenses and other liabilities	\$ 99,650	\$ 46,886
Total deferred revenue	82,932	68,317
Total deferred rent	10,007	6,593
Long-term debt	150,000	—
Total stockholders' equity (85.9 million and 85.1 million common shares issued and outstanding and at December 31, 2016 and December 31, 2015, respectively)	920,221	1,264,714
<b>Total liabilities and stockholders' equity</b>	<b>\$ 1,262,810</b>	<b>\$ 1,386,510</b>

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, 2015.

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