



August 9, 2017

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

- *Advanced Industry-Leading RNAi Therapeutics Pipeline, Including Four Programs in Late-Stage Development* -

- *On Track to Report APOLLO Phase 3 Top-Line Results for Patisiran in Near Term, and Initiated ATLAS Phase 3 Program for Fitusiran* -

- *Maintained Strong Balance Sheet with \$1.25 Billion in Cash and Plans to End 2017 with Greater than \$1.0 Billion in Cash* -

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

"2017 is shaping up to be a pivotal year for Alnylam. With patisiran, we expect to report top-line APOLLO Phase 3 study results in the coming weeks. If positive, these study findings will support our first NDA filing, planned by year-end, and our commercial transition in mid-2018 assuming regulatory approval. In parallel, we continue to advance our late-stage pipeline of investigational RNAi therapeutics, with the recently announced initiation of our ATLAS Phase 3 program for fitusiran in hemophilia and expected Phase 3 starts by year-end for givosiran in acute hepatic porphyrias and, with The Medicines Company, inclisiran in hypercholesterolemia," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "We believe that these are all important and defining milestones that position us to fulfill our 'Alnylam 2020' vision of becoming a multi-product, commercial-stage company with a deep and sustainable clinical development pipeline by the end of 2020."

Second Quarter 2017 and Recent Significant Corporate Highlights

- | Advanced patisiran, an investigational RNAi therapeutic for the treatment of patients with hereditary ATTR (hATTR) amyloidosis with polyneuropathy, 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 APOLLO Phase 3 data expected in mid-2017.
- | Advanced fitusiran, an investigational RNAi therapeutic for the treatment of hemophilia and rare bleeding disorders, with [positive new data](#) from the Phase 2 OLE study presented at the International Society on Thrombosis and Haemostasis 2017 Congress.
 - | Results from the Phase 1 study were published in [The New England Journal of Medicine](#) in a paper titled, "Targeting of Antithrombin in Hemophilia A or B with RNAi Therapy."
 - | Alnylam and partner Sanofi Genzyme announced the initiation of the ATLAS Phase 3 program, a global, multicenter clinical program designed to evaluate the safety and efficacy of fitusiran in patients with hemophilia A and B with or without inhibitors.
- | Advanced givosiran, an investigational RNAi therapeutic for the treatment of acute hepatic porphyrias (AHPs), with [positive new data](#) presented at the 2017 International Congress on Porphyrins and Porphyrias (ICPP) from the ongoing randomized, double-blind, placebo-controlled Phase 1 study in recurrent attack porphyria patients, as well as positive initial results from the ongoing Phase 1 OLE study.
 - | In addition, received Breakthrough Therapy designation from the U.S. Food and Drug Administration (FDA) for givosiran for the prophylaxis of attacks in patients with acute hepatic porphyria.
- | Alnylam and partner The Medicines Company announced agreement with the FDA on a Phase 3 clinical program for inclisiran, an investigational RNAi therapeutic for the treatment of hypercholesterolemia, with LDL-C lowering as the primary endpoint for the initial pivotal trial program, which is expected to initiate in late 2017.
- | Completed successful public offering of common stock, with concurrent private placement from Sanofi Genzyme, totaling \$376.5 million in net proceeds.

Upcoming Events

- | Alnylam will continue to host its 4th Annual RNAi Roundtable Series, which kicked off last week. This series consists 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 can be found [here](#).

- | Alnylam plans to report top-line results from the patisiran APOLLO Phase 3 study in mid-2017. Full results are expected to be presented in late 2017 at the 1st European ATTR Amyloidosis Meeting for Patients and Doctors, being held November 2-3, 2017 in Paris, France.
 - | If the APOLLO Phase 3 data are positive, Alnylam expects to file its first New Drug Application (NDA) in late 2017 and Marketing Authorisation Application (MAA) shortly thereafter.
- | Alnylam plans to initiate a Phase 3 study of givosiran in late 2017, pending successful alignment on trial design with global regulatory authorities.
- | The Medicines Company has announced its intention to initiate a Phase 3 study of inclisiran in patients with atherosclerotic cardiovascular disease (ASCVD) in late 2017.

Financial results for the quarter ended June 30, 2017

"Alnylam continues to maintain a strong balance sheet," said Manmeet Soni, Chief Financial Officer of Alnylam. "Our financial strength allows us to continue to invest in a broad pipeline of investigational RNAi therapeutics and prepare to transition towards a commercial-stage company, aligned with our 'Alnylam 2020' goals and strategy."

Cash and Investments

At June 30, 2017, Alnylam had cash, cash equivalents and fixed income marketable securities, and restricted investments of \$1.25 billion, as compared to \$1.09 billion at December 31, 2016.

In May 2017, Alnylam sold an aggregate of 5,000,000 shares of its common stock through an underwritten public offering at a price to the public of \$71.87 per share. As a result of the offering, Alnylam received aggregate net proceeds of \$355.2 million.

In addition, Sanofi Genzyme exercised its right to purchase, in a concurrent private placement, 297,501 shares of common stock, at the public offering price of \$71.87 per share, resulting in proceeds to Alnylam of \$21.4 million.

GAAP and Non-GAAP Net Loss

The net loss according to accounting principles generally accepted in the U.S. (GAAP) for the second quarter of 2017 was \$118.4 million, or \$1.34 per share on both a basic and diluted basis, as compared to a net loss of \$90.1 million, or \$1.05 per share on both a basic and diluted basis, for the same period in the previous year.

The non-GAAP net loss for the second quarter of 2017 was \$94.4 million, or \$1.07 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of \$74.3 million, or \$0.87 per share on both a basic and diluted basis for the same period in the previous year.

The non-GAAP net loss excludes stock-based compensation expense. See "Use of Non-GAAP Financial Measures" below for a description of non-GAAP financial measures and a reconciliation between GAAP and non-GAAP net loss appearing later in this press release.

Revenues

Revenues were \$15.9 million in the second quarter of 2017, as compared to \$8.7 million in the second quarter of 2016. Revenues for the second quarter of 2017 included \$14.4 million from the company's alliance with Sanofi Genzyme and \$1.5 million from the company's alliance with The Medicines Company.

Research and Development Expenses

Research and development (R&D) expenses were \$90.6 million in the second quarter of 2017, which included \$13.3 million of stock-based compensation, as compared to \$83.2 million in the second quarter of 2016, which included \$9.3 million of stock-based compensation.

General and Administrative Expenses

General and administrative (G&A) expenses were \$45.8 million in the second quarter of 2017, which included \$10.8 million of stock-based compensation, as compared to \$18.0 million in the second quarter of 2016, which included \$6.5 million of stock-based compensation.

Financial Guidance

Alnylam remains on track to end 2017 with greater than \$1.0 billion in cash, cash equivalents and fixed marketable securities including \$150.0 million in restricted investments.

Conference Call Information

Management will provide an update on the company and discuss second quarter 2017 results as well as expectations for the future via conference call on Wednesday, August 9, 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 59171216. A replay of the call will be available beginning at 7:30 p.m. ET on the day of the call. To access the replay, please dial 855-859-2056 (domestic) or 404-537-3406 (international), and refer to conference ID 59171216.

Alnylam - Sanofi Genzyme Alliance

In January 2014, Alnylam and Sanofi Genzyme, the specialty care global business unit of Sanofi, formed an alliance to accelerate the advancement of RNAi therapeutics as a potential new class of innovative medicines for patients around the world with rare genetic diseases. The alliance enables Sanofi Genzyme to expand its rare disease pipeline with Alnylam's novel RNAi technology and provides access to Alnylam's R&D engine, while Alnylam benefits from Sanofi Genzyme's proven global capabilities to advance late-stage development and, upon commercialization, accelerate market access for these promising genetic medicine 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 rest of the world. In November 2016, Sanofi Genzyme elected to co-develop (through Sanofi R&D) and co-commercialize fitusiran in the United States, Canada and Western Europe, in addition to commercializing fitusiran in its rest of world territories.

About RNAi

RNAi (RNA interference) is a revolution in biology, representing a breakthrough in understanding protein synthesis 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, with the goal of preventing disease-causing proteins from being made.

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.

Use of Non-GAAP Financial Measures

This press release contains non-GAAP financial measures, including net loss adjusted to exclude certain non-cash expenses. These measures are not in accordance with, or an alternative to, GAAP, and may be different from non-GAAP financial measures used by other companies.

The item included in GAAP presentations but excluded for purposes of determining non-GAAP financial measures for the periods presented in this press release is stock-based compensation expense. The company has excluded the impact of stock-based compensation expense, which may fluctuate from period to period based on factors including the variability associated with performance-based grants for stock options and restricted stock units and changes in the company's stock price, which impacts the fair value of these awards.

The company believes the presentation of non-GAAP net loss provides useful information to management and investors regarding the company's financial condition and results of operations. When GAAP financial measures are viewed in conjunction with non-GAAP financial measures, investors are provided with a more meaningful understanding of the company's ongoing operating performance. In addition, non-GAAP net loss is among those indicators the company uses as a basis for evaluating performance, allocating resources and planning and forecasting future periods. Non-GAAP financial measures are not intended to be considered in isolation or as a substitute for GAAP financial measures. A reconciliation between GAAP and non-GAAP net loss is provided later in this press release.

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 and MAA for patisiran if the APOLLO Phase 3 study is positive and its expected transition to commercial operations in mid-2018 if regulators approve patisiran, its expected cash, cash equivalents, fixed marketable securities and restricted investments position as of December 31, 2017, 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 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 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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net revenues from collaborators	\$ 15,932	\$ 8,709	\$ 34,892	\$ 16,054
Operating expenses:				
Research and development	90,627	83,172	177,611	179,445
General and administrative	45,779	17,987	84,266	39,087
Total operating expenses	136,406	101,159	261,877	218,532
Loss from operations	(120,474)	(92,450)	(226,985)	(202,478)
Other income (expense):				
Interest income	2,577	2,092	4,705	3,905
Other (expense) income	(523)	229	(3,430)	5,470
Total other income	2,054	2,321	1,275	9,375
Net loss	<u>\$ (118,420)</u>	<u>\$ (90,129)</u>	<u>\$ (225,710)</u>	<u>\$ (193,103)</u>
Net loss per common share - basic and diluted	<u>\$ (1.34)</u>	<u>\$ (1.05)</u>	<u>\$ (2.59)</u>	<u>\$ (2.26)</u>
Weighted-average common shares used to compute basic and diluted net loss per common share	<u>88,098</u>	<u>85,545</u>	<u>87,068</u>	<u>85,411</u>

Comprehensive loss:

Net loss	\$ (118,420)	\$ (90,129)	\$ (225,710)	\$ (193,103)
Unrealized loss on marketable securities, net of tax	(476)	(18,331)	(2,412)	(26,555)
Reclassification adjustment for realized loss (gain) on marketable securities included in net loss	345	(954)	1,894	(6,110)
Comprehensive loss	<u>\$ (118,551)</u>	<u>\$ (109,414)</u>	<u>\$ (226,228)</u>	<u>\$ (225,768)</u>

ALNYLAM PHARMACEUTICALS, INC.
RECONCILIATION OF GAAP NET LOSS TO NON-GAAP NET LOSS
(In thousands, except per share amounts)

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
GAAP net loss	\$(118,420)	\$(90,129)	\$(225,710)	\$(193,103)
Adjustment:				
Stock-based compensation expenses	24,030	15,816	39,747	39,296
Non-GAAP net loss	<u>\$(94,390)</u>	<u>\$(74,313)</u>	<u>\$(185,963)</u>	<u>\$(153,807)</u>
GAAP net loss per common share - basic and diluted	\$ (1.34)	\$ (1.05)	\$ (2.59)	\$ (2.26)
Adjustment (as detailed above)	0.27	0.18	0.45	0.46
Non-GAAP net loss per common share - basic and diluted	<u>\$(1.07)</u>	<u>\$(0.87)</u>	<u>\$(2.14)</u>	<u>\$(1.80)</u>

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

	<u>June 30,</u>	<u>December</u>
	<u>2017</u>	<u>31,</u>
		<u>2016</u>
Cash, cash equivalents and fixed income marketable securities	\$1,097,841	\$ 942,601
Restricted investments	150,000	150,000
Billed and unbilled collaboration receivables	15,405	23,334
Prepaid expenses and other assets	23,504	32,303
Property, plant and equipment, net	147,533	114,572
Total assets	<u>\$1,434,283</u>	<u>\$ 1,262,810</u>
Accounts payable, accrued expenses and other liabilities	\$ 69,805	\$ 99,650
Total deferred revenue	81,873	82,932
Total deferred rent	9,639	10,007
Long-term debt	150,000	150,000
Total stockholders' equity (91.7 million and 85.9 million common shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively)	1,122,966	920,221
Total liabilities and stockholders' equity	<u>\$1,434,283</u>	<u>\$ 1,262,810</u>

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.

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Anylam Pharmaceuticals, Inc.

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