Alnylam Pharmaceuticals Reports Third Quarter 2018 Financial Results and Highlights Recent Period Activity

November 7, 2018

− Obtained FDA and EMA Approvals of ONPATTRO™ (patisiran) – the First-Ever RNAi Therapeutic – and Launched in U.S. and EU −

− Received 125 U.S. Patient Start Forms in First Seven Weeks of ONPATTRO Launch −

− Reported Positive Topline Interim Analysis Results from ENVISION Phase 3 Study of Givosiran in Patients with Acute Hepatic Porphyrias −

− Advanced Lumasiran into ILLUMINATE Phase 3 Program −

− Maintained Strong Balance Sheet with $1.27 Billion in Cash and Expects to End 2018 with Approximately $1.0 Billion in Cash −

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 7, 2018-- Alnylam Pharmaceuticals, Inc. (Nasdaq:ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the third quarter 2018 and reviewed recent commercial and R&D highlights.

"The third quarter and recent period were truly revolutionary for Alnylam with the approval of ONPATTRO in both the U.S. and EU, heralding the arrival of RNAi therapeutics as a whole new class of medicines. With these approvals and the subsequent launches, we have begun to realize the promise of RNAi therapeutics on a global scale," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "Our recent regulatory and commercial launch experiences will be leveraged for our entire portfolio including late-stage programs such as givosiran, where we will initiate a rolling NDA submission this year; lumasiran, which we have advanced into late-stage development with the recent initiation of the ILLUMINATE-A Phase 3 study; and ALN-TTRsc02, which will enter Phase 3 later this year in the HELIOS-A study. We believe these accomplishments put us well on our way toward achieving our *Alnylam 2020* goal of building a multi-product, global, commercial-stage company with a deep and sustainable clinical pipeline by the end of 2020."

"With the approval and launch of ONPATTRO, Alnylam is now a global commercial-stage company. With only seven weeks of results for the third quarter, we're encouraged by the number of U.S. patient start forms, and emerging prescriber base, highlighting what we believe is strong demand for ONPATTRO for adults with polyneuropathy caused by hATTR amyloidosis. Moreover, we believe our regional presence in North America, Europe, Asia, and, soon, Latin America, along with established medical affairs and supply chain capabilities, positions us to expand our efforts in markets around the world," said Barry Greene, President of Alnylam. "We look forward to continuing our work toward strong commercial execution, with a focus on raising disease awareness, improving diagnosis, and bringing ONPATTRO to patients in need."

Third Quarter 2018 and Recent Period Significant Corporate Highlights

Commercial Highlights

- Launched ONPATTRO™ (patisiran) in the U.S. and EU, initially in Germany.
- Received 125 U.S. patient Start Forms as of September 30, 2018.
- Recognized ONPATTRO revenue of $0.5 million for the quarter ended September 30, 2018.
- Announced alignment on value-based agreements with leading health insurers and launched Alnylam Assist™, a comprehensive patient support services program for ONPATTRO in the U.S.

R&D Highlights

- Achieved the first-ever regulatory approval of an RNAi therapeutic, ONPATTRO (patisiran), in the U.S. and EU.
  - Received U.S. Food and Drug Administration (FDA) approval of ONPATTRO for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults.
  - Received marketing authorization from the European Commission for ONPATTRO for the treatment of hATTR amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy.
  - Continued global efforts to bring ONPATTRO to patients with submission of a New Drug Application to Japan's Pharmaceuticals and Medical Devices Agency and receipt of a Priority Review designation in Canada.
  - Published results from the APOLLO Phase 3 study of patisiran in the July 5, 2018 issue of *The New England Journal of Medicine* and APOLLO exploratory cardiac endpoint data in the September 14, 2018 issue of *Circulation*.
- Advanced ALN-TTRsc02, a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
  - Aligned the design of HELIOS-A, a pivotal Phase 3 study of ALN-TTRsc02 in patients with hATTR amyloidosis polyneuropathy, with FDA and European Medicines Agency (EMA) feedback.
  - The Company is on track to start the HELIOS-A study in late 2018 and plans to initiate additional Phase 3 studies of ALN-TTRsc02, including in hereditary and wild-type ATTR amyloidosis cardiomyopathy, in 2019.
- Advanced givosiran, an investigational RNAi therapeutic in development for the treatment of acute hepatic porphyrias (AHPs).

    - Announced positive topline results from the interim analysis of the ENVISION Phase 3 study of givosiran.
    - Announced plans to initiate a rolling submission of a New Drug Application (NDA) and pursue full approval based on complete results – now expected in early 2019 – from the ENVISION Phase 3 study. The rolling NDA submission is expected to be initiated in 2018, with full clinical sections submitted in mid-2019, assuming positive results.
- Advanced lumasiran, an investigational RNAi therapeutic in development for the treatment of primary hyperoxaluria type 1 (PH1).
    - Announced initiation of ILLUMINATE-A, a global Phase 3 pivotal trial of lumasiran in children and adults with PH1. Alnylam expects to report topline results from ILLUMINATE-A in late 2019 and, if positive, submit filings for global regulatory approvals starting in early 2020.
    - Presented updated positive results from the Phase 1/2 study in PH1 patients at the 2018 European Society for Paediatric Nephrology and the American Society of Nephrology annual meetings.
    - Announced alignment with the FDA on the trial design for ILLUMINATE-B, a Phase 3 study of lumasiran in PH1 patients less than six years of age with preserved renal function.
    - Expanded the Alnylam Act® program to include no-charge, third-party genetic testing and counseling for adults and children who may carry a mutation in the gene encoding alanine-glyoxylate aminotransferase (*AGXT*), which is associated with PH1.
- Alnylam's partner, The Medicines Company, announced in October that the Independent Data Monitoring Committee for the ongoing inclisiran Phase 3 clinical trials (ORION 9, 10, and 11) conducted its fourth planned review of safety and efficacy data from the ORION trials and recommended that the trials continue without modification.
    - The safety database for inclisiran now provides 1,899 years of patient exposure to an RNAi therapeutic, representing the industry's most comprehensive body of safety data for an RNAi therapeutic.
- Alnylam's partner, Sanofi, continues enrollment in the fitusiran Phase 3 ATLAS program in patients with hemophilia A or B with and without inhibitors.

- Advanced early-stage RNAi pipeline.
    - Submitted a Clinical Trial Authorization (CTA) application for ALN-AAT02, an investigational RNAi therapeutic for the treatment of alpha-1 antitrypsin deficiency-associated liver disease (alpha-1 liver disease), which is based on Alnylam's Enhanced Stabilization Chemistry-Plus (ESC+) GalNAc conjugate technology.
    - The Company announces today that due to recruitment challenges, it has discontinued a Phase 2 study of cemdisiran in atypical hemolytic uremic syndrome (aHUS). Alnylam will now focus its cemdisiran clinical efforts on a Phase 2 study in IgA nephropathy.
    - Reported new platform innovations at the Oligonucleotide Therapeutics Society 2018 Annual Meeting, including pre-clinical results demonstrating CNS and ocular delivery of RNAi therapeutics in rats and non-human primates.

Additional Business Updates

- Expanded organization with key appointments and new hires.
    - Appointed Dr. Margaret Hamburg, former FDA Commissioner, to the Board of Directors, effective January 10, 2019. Concurrent with Dr. Hamburg's appointment, Mr. John Clarke is resigning from the Board after sixteen years of service.
    - Alnylam announces today the promotion of Andy Orth as Senior Vice President, Head of U.S. In this role, he is responsible for commercial execution of Alnylam programs in the U.S. market. He was previously Alnylam's Vice President of Commercial Operations, and joined the Company in 2016 from Biogen. Prior to Biogen, he held commercial and finance leadership roles at Genzyme and Amgen.
    - Alnylam also announces today the appointment of Norton Oliveira as Senior Vice President, Head of Latin America. He joins Alnylam from Gilead Sciences where he was Vice President for Latin America and the Caribbean. Prior to Gilead, Norton held commercial leadership roles at Merck/MSD and Shire.

Upcoming Events

In late 2018, Alnylam intends to:

- Initiate a rolling submission of an NDA with the FDA for givosiran, with full clinical sections to be submitted in mid-2019, assuming positive results.
- Initiate the HELIOS-A Phase 3 study for ALN-TTRsc02 in hATTR amyloidosis.
- Initiate the Phase 1/2 study for ALN-AAT02 in alpha-1 liver disease.
- File a Clinical Trial Authorization (CTA) application for ALN-HBV02 (also known as VIR-2218), in partnership with Vir Biotechnology, for the treatment of chronic hepatitis B virus infection.
- Select its first CNS-targeted development candidate (DC) program.
- Hold an R&D Day Investor Conference on December 6 in New York City.

Financial results for the quarter ended September 30, 2018

"We are pleased to have recognized initial revenue for an Alnylam product for the first time in the Company's history, following the August FDA approval of ONPATTRO," said Manmeet Soni, Chief Financial Officer of Alnylam. "With cash and investments on our balance sheet standing at $1.27 billion at September 30, and expectations to finish the year with approximately $1.0 billion, we believe Alnylam is in a strong position to continue executing on global commercial operations while advancing our pipeline programs through the clinic."

Cash and Investments
At September 30, 2018, Alnylam had cash, cash equivalents and marketable debt securities, and restricted investments, excluding equity securities, of $1.27 billion, as compared to $1.73 billion at December 31, 2017.

GAAP and Non-GAAP Net Loss
The net loss according to accounting principles generally accepted in the U.S. (GAAP) for the third quarter of 2018 was $245.3 million, or $2.43 per share on both a basic and diluted basis, as compared to a net loss of $122.9 million, or $1.34 per share on both a basic and diluted basis, for the same period in the previous year.

The non-GAAP net loss for the third quarter of 2018 was $157.3 million, or $1.56 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of $97.0 million, or $1.06 per share on both a basic and diluted basis, for the same period in the previous year.

The non-GAAP net loss for the third quarter of 2018 and 2017 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.

ONPATTRO Revenues, Net
Net product revenues from sales of ONPATTRO were $0.5 million in the third quarter of 2018 following its approval by the FDA in August 2018.

Net Revenues from Collaborators
Net revenues from collaborators were $1.6 million in the third quarter of 2018 as compared to $17.1 million in the third quarter of 2017.

GAAP and Non-GAAP Research and Development Expenses
GAAP research and development (R&D) expenses were $139.9 million in the third quarter of 2018 as compared to $95.3 million in the third quarter of 2017.

Non-GAAP R&D expenses were $94.2 million in the third quarter of 2018 as compared to $80.2 million in the third quarter of 2017. Non-GAAP R&D expenses exclude stock-based compensation expense. A reconciliation between GAAP and non-GAAP R&D expenses appears later in this press release.

GAAP and Non-GAAP Selling, General and Administrative Expenses
GAAP selling, general and administrative (SG&A) expenses were $116.5 million in the third quarter of 2018 as compared to $47.6 million in the third quarter of 2017.

Non-GAAP SG&A expenses were $74.4 million in the third quarter of 2018 as compared to $36.8 million in the third quarter of 2017. Non-GAAP SG&A expenses exclude stock-based compensation expense. A reconciliation between GAAP and non-GAAP SG&A expenses appears later in this press release.

2018 Financial Guidance
Alnylam reiterates its expectations to end 2018 with approximately $1.0 billion of cash, cash equivalents and marketable debt securities, restricted cash and restricted investments, excluding equity securities.

The Company reiterates its expectations for 2018 annual non-GAAP R&D expenses to be in the range of $420 million to $460 million and non-GAAP SG&A expenses to be in the range of $280 million to $320 million. Both non-GAAP R&D and non-GAAP SG&A expenses exclude stock-based compensation expenses.

Use of Non-GAAP Financial Measures
This press release contains non-GAAP financial measures, including expenses adjusted to exclude certain non-cash expenses and non-recurring gains outside the ordinary course of the Company's business. 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 items included in GAAP presentations but excluded for purposes of determining non-GAAP financial measures for the periods presented in the press release are stock-based compensation expense and the gain on litigation settlement. 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 has excluded the impact of the gain on litigation settlement because the Company believes this item is a one-time event occurring outside the ordinary course of the Company's business.

The Company believes the presentation of non-GAAP financial measures 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 and are better able to compare the Company's performance between periods. In addition, these non-GAAP financial measures are 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 measures is provided later in this press release.

Conference Call Information
Management will provide an update on the Company and discuss third quarter 2018 and recent period results as well as expectations for the future via conference call on Wednesday, November 7, 2018 at 8:30 am ET. To access the call, please dial 800-667-5617 (domestic) or 334-323-0509

(international) five minutes prior to the start time and refer to conference ID 7650424. A replay of the call will be available beginning at 11:30 am ET on the day of the call. To access the replay, please dial 888-203-1112 (domestic) or 719-457-0820 (international) and refer to conference ID 7650424.

Alnylam – Sanofi Genzyme Alliance
Alnylam and Sanofi Genzyme, the specialty care global business unit of Sanofi, established an alliance to accelerate the advancement of RNAi therapeutics as a potential new class of 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 medicines products.

About RNAi
RNAi (RNA interference) is a natural cellular process of gene silencing that represents one of the most promising and rapidly advancing frontiers in biology and drug development today. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and was recognized with the award of the 2006 Nobel Prize for Physiology or Medicine. By harnessing the natural biological process of RNAi occurring in our cells, a new class of medicines, known as RNAi therapeutics, is now a reality. Small interfering RNA (siRNA), the molecules that mediate RNAi and comprise Alnylam's RNAi therapeutic platform, function upstream of today's medicines by potently silencing messenger RNA (mRNA) – the genetic precursors – that encode for disease-causing proteins, thus preventing them from being made. This is a revolutionary approach with the potential to transform the care of patients with genetic and other diseases.

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

About ONPATTRO™ (patisiran)
Patisiran, based on Nobel Prize-winning science, is an intravenously administered RNAi therapeutic targeting transthyretin (TTR) for the treatment of hereditary ATTR amyloidosis. It is designed to target and silence specific messenger RNA, potentially blocking the production of TTR protein before it is made. Patisiran blocks the production of transthyretin in the liver, reducing its accumulation in the body's tissues in order to halt or slow down the progression of the disease. In August 2018, patisiran received U.S. Food and Drug Administration (FDA) approval for the treatment of the polyneuropathy of hATTR amyloidosis in adults, as well as European Medicines Agency marketing authorization for the treatment of hATTR amyloidosis in adults with Stage 1 or Stage 2 polyneuropathy.

Important Safety Information

Infusion-Related Reactions
Infusion-related reactions (IRRs) have been observed in patients treated with ONPATTRO. In a controlled clinical study, 19 percent of ONPATTRO-treated patients experienced IRRs, compared to 9 percent of placebo-treated patients. The most common symptoms of IRRs with ONPATTRO were flushing, back pain, nausea, abdominal pain, dyspnea, and headache.

To reduce the risk of IRRs, patients should receive premedication with a corticosteroid, paracetamol, and antihistamines (H1 and H2 blockers) at least 60 minutes prior to ONPATTRO infusion. Monitor patients during the infusion for signs and symptoms of IRRs. If an IRR occurs, consider slowing or interrupting the infusion and instituting medical management as clinically indicated. If the infusion is interrupted, consider resuming at a slower infusion rate only if symptoms have resolved. In the case of a serious or life-threatening IRR, the infusion should be discontinued and not resumed.

Reduced Serum Vitamin A Levels and Recommended Supplementation
ONPATTRO treatment leads to a decrease in serum vitamin A levels. Supplementation at the recommended daily allowance (RDA) of vitamin A is advised for patients taking ONPATTRO. Higher doses than the RDA should not be given to try to achieve normal serum vitamin A levels during treatment with ONPATTRO, as serum levels do not reflect the total vitamin A in the body.

Patients should be referred to an ophthalmologist if they develop ocular symptoms suggestive of vitamin A deficiency (e.g. night blindness).

Adverse Reactions
The most common adverse reactions that occurred in patients treated with ONPATTRO were respiratory-tract infection (29 percent) and infusion-related reactions (19 percent).

About Alnylam Pharmaceuticals
Alnylam (Nasdaq:ALNY) is leading the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to improve the lives of people afflicted with rare genetic, cardio-metabolic, hepatic infectious, and central nervous system (CNS) diseases. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of severe and debilitating diseases. Founded in 2002, Alnylam is delivering on a bold vision to turn scientific possibility into reality, with a robust discovery platform. ONPATTRO ™ (patisiran) lipid complex injection, available in the U.S. for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults, is Alnylam's first U.S. FDA-approved RNAi therapeutic. In the EU, ONPATTRO is approved for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy. Alnylam has a deep pipeline of investigational medicines, including three product candidates that are in late-stage development. 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 to address the needs of patients who have limited or inadequate treatment options. Alnylam employs over 1,000 people worldwide and is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam or on LinkedIn.

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 givosiran, ALN-TTRsc02, lumasiran, inclisiran, ALN-AAT02 and cemdisiran, its plans for additional regulatory filings and product launches for ONPATTRO, including in Japan and Latin America, its expectations regarding the timing for initiation of a rolling NDA submission with the FDA for givosiran and the reporting of complete topline results, its expectations regarding the potential timing of ILLUMINATE-A Phase 3 study results for lumasiran in late 2019 and a possible filing of an NDA in early 2020 if such results are positive, its plans to initiate the ILLUMINATE-B Phase 3 study of lumasiran, its plans to initiate a Phase 3 study for ALN-TTRsc02 in 2018 in hATTR amyloidosis and its plans to initiate additional Phase 3 studies of ALN-TTRsc02, including in hereditary and wild-type ATTR amyloidosis cardiomyopathy, in 2019,

its plan to initiate the Phase 1/2 study for ALN-AAT02 in alpha-1 liver disease and to file a CTA application for ALN-HBV02 in partnership with Vir Biotechnology, its plans to select its first CNS-targeted DC program, its expected cash, cash equivalents and marketable debt securities, restricted cash and restricted investments balance, excluding equity securities, as of December 31, 2018, its expected range of 2018 annual non-GAAP R&D expenses and non-GAAP SG&A expenses, and expectations regarding 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 its 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, successfully launching, marketing and selling its approved products globally, Alnylam's ability to successfully expand the indication for ONPATTRO in the future, 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.

With the exception of ONPATTRO (patisiran), 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 such investigational therapeutics.

ALNYLAM PHARMACEUTICALS, INC.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Revenues:				
Product revenues, net	$ 460	$ —	$ 460	$ —
Net revenues from collaborators	1,609	17,096	53,415	51,988
Total revenues	$ 2,069	$ 17,096	$ 53,875	$ 51,988
Cost and expenses:				
Cost of goods sold	137	—	137	—
Research and development	139,945	95,252	374,384	272,863
Selling, general and administrative	116,545	47,644	273,671	131,910
Total costs and expenses	256,627	142,896	648,192	404,773
Loss from operations	(254,558)	(125,800)	(594,317)	(352,785)
Other income (expense):				
Interest income	6,796	3,296	18,691	8,001
Other income (expense)	2,925	(433)	5,468	(3,863)
Gain on litigation settlement	—	—	20,564	—
Total other income	9,721	2,863	44,723	4,138
Loss before income taxes	(244,837)	(122,937)	(549,594)	(348,647)
Provision for income taxes	(445)	—	(462)	—
Net loss	$ (245,282)	$ (122,937)	$ (550,056)	$ (348,647)
Net loss per common share - basic and diluted	$ (2.43)	$ (1.34)	$ (5.48)	$ (3.93)
Weighted-average common shares used to compute basic and diluted net loss per common share	100,783	91,828	100,430	88,672
Comprehensive loss:				
Net loss	$ (245,282)	$ (122,937)	$ (550,056)	$ (348,647)
Unrealized gain (loss) on marketable securities, net of tax	415	218	1,041	(2,194)
Reclassification adjustment for realized loss on marketable securities included in net loss	—	—	—	1,894

Comprehensive loss	$ (244,867)	$ (122,719)	$ (549,015)	$ (348,947)

ALNYLAM PHARMACEUTICALS, INC.

RECONCILIATION OF SELECTED GAAP MEASURES TO NON-GAAP MEASURES

(In thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Reconciliation of GAAP to Non-GAAP Research and development:				
GAAP Research and development	$ 139,945	$ 95,252	$ 374,384	$ 272,863
Less: Stock-based compensation expenses	(45,784)	(15,090)	(67,537)	(37,035)
Non-GAAP Research and development	$ 94,161	$ 80,162	$ 306,847	$ 235,828
Reconciliation of GAAP to Non-GAAP Selling, general and administrative:				
GAAP Selling, general and administrative	$ 116,545	$ 47,644	$ 273,671	$ 131,910
Less: Stock-based compensation expenses	(42,170)	(10,865)	(62,242)	(28,667)
Non-GAAP Selling, general and administrative	$ 74,375	$ 36,779	$ 211,429	$ 103,243
Reconciliation of GAAP to Non-GAAP Operating costs and expenses:				
GAAP Operating costs and expenses	$ 256,627	$ 142,896	$ 648,192	$ 404,773
Less: Stock-based compensation expenses	(87,954)	(25,955)	(129,779)	(65,702)
Non-GAAP Operating costs and expenses	$ 168,673	$ 116,941	$ 518,413	$ 339,071
Reconciliation of GAAP to Non-GAAP Net loss:				
GAAP Net loss	$ (245,282)	$ (122,937)	$ (550,056)	$ (348,647)
Add: Stock-based compensation expenses	87,954	25,955	129,779	65,702
Less: Gain on litigation settlement	—	—	(20,564)	—
Non-GAAP Net loss	$ (157,328)	$ (96,982)	$ (440,841)	$ (282,945)
Reconciliation of GAAP to Non-GAAP Net loss per common share-basic and diluted:				
GAAP Net loss per common share - basic and diluted	$ (2.43)	$ (1.34)	$ (5.48)	$ (3.93)
Add: Stock-based compensation expenses	0.87	0.28	1.29	0.74
Less: Gain on litigation settlement	—	—	(0.20)	—
Non-GAAP Net loss per common share - basic and diluted	$ (1.56)	$ (1.06)	$ (4.39)	$ (3.19)

ALNYLAM PHARMACEUTICALS, INC.

UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	September 30, 2018	**December 31, 2017**
Cash, cash equivalents and marketable debt securities	$ 1,221,830	$ 1,704,537
Restricted investments	44,825	30,000
Accounts receivable, net	3,362	34,002
Inventory	11,081	—
Prepaid expenses and other assets	121,904	44,291
Property, plant and equipment, net	272,652	181,900
Total assets	$ 1,675,654	$ 1,994,730
Accounts payable, accrued expenses and other liabilities	$ 119,671	$ 104,905
Total deferred revenue	5,067	84,780
Total deferred rent	42,797	8,614

Long-term debt	30,000	30,000
Total stockholders' equity (101.0 million and 99.7 million common shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively)	1,478,119	1,766,431
Total liabilities and stockholders' equity	$ 1,675,654	$ 1,994,730

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, 2017.

View source version on businesswire.com: https://www.businesswire.com/news/home/20181107005464/en/

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