Alnylam Pharmaceuticals Reports Fourth Quarter and Full Year 2018 Financial Results and Highlights Recent Period Activity

February 7, 2019

− Achieved Fourth Quarter 2018 ONPATTRO® Global Net Product Revenues of $12.1 Million, with Over 200 Patients on Commercial Product in U.S. and EU as of Year-End 2018 –

− Obtained Regulatory Alignment on APOLLO-B Phase 3 Study of Patisiran in ATTR Amyloidosis with Cardiomyopathy and Initiated HELIOS-A Phase 3 Study to Advance Vutrisiran to Market, Supporting Plan for Sustained and Continuous Growth of ATTR Franchise –

− Advanced Additional Phase 3 Programs, Including Givosiran, with Rolling Submission of New Drug Application (NDA) Initiated and Topline ENVISION Phase 3 Results Expected in March, and Lumasiran, with ILLUMINATE-A Phase 3 Study Initiated –

− Maintained Strong Balance Sheet with $1.13 Billion in Cash as of Year-End 2018 –

− Provides 2019 Non-GAAP R&D and SG&A Expense Guidance –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 7, 2019-- Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the fourth quarter and full year ended December 31, 2018 and reviewed recent commercial and R&D highlights.

“In 2018, we saw the approval and launch of ONPATTRO, the world’s first RNAi therapeutic, heralding the arrival of a whole new class of medicines. With our planned APOLLO-B Phase 3 study to support potential expansion of ONPATTRO to ATTR amyloidosis patients with cardiomyopathy and advancement of subcutaneously administered vutrisiran in the HELIOS-A Phase 3 study, we are committing further efforts to help ATTR amyloidosis patients and to support our plans for the sustained and continued growth in our ATTR amyloidosis franchise for years to come,” said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. “While executing on our efforts to bring ONPATTRO to patients around the world, we also made great strides advancing our pipeline of investigational RNAi therapeutics. In 2019, we intend to execute on six Phase 3 programs – of which two are being advanced together with partners – with Phase 3 data expected from three of these programs, and expect two NDA submissions, assuming positive results. In the meanwhile, we’ll continue to benefit from a sustainable RNAi research engine that we expect will fuel future innovation and deliver important medicines for patients.”

“We are pleased with our commercial progress to date, as we strive to make ONPATTRO available to patients around the world. Specifically, we are encouraged by our fourth quarter 2018 results, with over 200 patients receiving treatment with commercial ONPATTRO in the U.S. and EU since launch, reflecting what we believe to be strong patient and physician demand and excellent performance by our customer-facing field teams,” said Barry Greene, President of Alnylam. “In 2019, we’re focused on continued execution of our ONPATTRO launch in the U.S. and EU, while expanding our global commercial presence in Asia and Latin America. Longer term, we’re excited by the opportunity for positive impact for patients, patient families and caregivers, and meaningful growth potential for our ATTR amyloidosis franchise.”

Fourth Quarter 2018 and Recent Significant Corporate Highlights

Commercial Performance at Year-End 2018

- Achieved global net product revenues for the fourth quarter of 2018 of $12.1 million for ONPATTRO, and $12.5 million for the full year ended December 31, 2018.
- Attained over 200 patients in the U.S. and EU on commercial ONPATTRO treatment.
    - Including patients on commercial drug and patients in clinical studies and in the Company’s global Expanded Access Program (EAP), approximately 550 total patients worldwide were being treated with patisiran.
- Received a total of 250 Start Forms in the U.S., with approximately 50 percent from patients not previously treated in the ONPATTRO EAP.
    - Start Forms came from a diverse range of prescribing physician specialties, including 44 percent from neurologists, 35 percent from cardiologists, and 21 percent from other specialties.
    - For Start Forms received, 62 percent of patients were covered by Medicare, 32 percent were covered by commercial insurers, and 6 percent were covered by other government insurers.
- Continued significant progress with value-based agreements (VBAs) with commercial payers in the U.S. and with market access efforts in the EU.
    - Since launch, Alnylam has completed definitive VBAs with Harvard Pilgrim Healthcare, Humana, and another top five U.S. payer. Additional VBAs are under negotiation with over 15 other commercial payers with the potential to cover over 90 percent of commercial lives in the U.S.
    - The Company announces today that it has advanced pricing & reimbursement procedures with authorities in 15 EU countries – representing the vast majority of the hATTR amyloidosis opportunity in Europe – with positive feedback from several EU payers. Recent examples include positive technology assessment reports from authorities in Germany and Sweden, the special innovation designation of ONPATTRO by the Italian authorities, and favorable alignment with authorities in The Netherlands, among others.

R&D Highlights

- Advanced patisiran (the non-branded name for ONPATTRO), an intravenously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
    - Continued global efforts to bring ONPATTRO to patients with filing of a New Drug Submission (NDS) in Canada and a Marketing Authorisation Application (MAA) in Switzerland, which has now been accepted.
    - Obtained alignment with the U.S. Food and Drug Administration (FDA) on the design of APOLLO-B, a randomized, double-blind, placebo-controlled Phase 3 study of patisiran in hereditary and wild-type ATTR amyloidosis patients with cardiomyopathy, with the goal of starting the trial in mid-2019.
- Advanced vutrisiran (ALN-TTRsc02), a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
    - Initiated HELIOS-A Phase 3 study in hereditary ATTR amyloidosis with polyneuropathy.
    - Announced plans to initiate an additional Phase 3 study, HELIOS-B, of vutrisiran in hereditary and wild-type ATTR amyloidosis with cardiomyopathy in late 2019.
- Advanced givosiran, an investigational RNAi therapeutic in development for the treatment of acute hepatic porphyria (AHP).
    - Initiated rolling submission of a New Drug Application (NDA) with plans to pursue full approval based on complete results – expected in March 2019 – from the ENVISION Phase 3 study. Assuming positive results, the Company expects to submit full clinical sections to the FDA in mid-2019.
    - Published data from Phase 1 study of givosiran in *The New England Journal of Medicine.*
    - Presented updated positive Phase 1/2 open-label extension (OLE) results at The Liver Meeting® 2018 of the American Association for the Study of Liver Diseases (AASLD).
- Advanced lumasiran, an investigational RNAi therapeutic in development for the treatment of primary hyperoxaluria type 1 (PH1).
    - Initiated ILLUMINATE-A, a global Phase 3 pivotal trial of lumasiran in children and adult PH1 patients with preserved renal function. 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 (ESPN) and the American Society of Nephrology (ASN) 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.
- Alnylam's partner, The Medicines Company, announced in January 2019 that the Independent Data Monitoring Committee for the ongoing inclisiran Phase 3 clinical trials (ORION 9, 10, and 11) conducted its fifth 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 more than 2,450 patient-years of exposure to an RNAi therapeutic, representing the industry's most comprehensive body of safety data for an RNA 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 programs.
    - Discontinued a Phase 2 study of cemdisiran in atypical hemolytic uremic syndrome (aHUS) due to recruitment challenges. Alnylam announces today that it has received regulatory approval to initiate a Phase 2 study of cemdisiran in IgA nephropathy.
    - 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). Alnylam announces today that it has initiated a Phase 1 study of ALN-AAT02, with initial results expected in 2019.
    - With Vir Biotechnology, initiated a Phase 1/2 study of ALN-HBV02 (also known as VIR-2218), with initial results expected in 2019.
    - Reported new platform innovations at the Oligonucleotide Therapeutics Society 2018 Annual Meeting, including pre-clinical results demonstrating potent, wide-spread, and highly durable CNS and ocular delivery of RNAi therapeutics in rats and non-human primates.
    - Selected its first CNS-targeted development candidate, ALN-APP, an investigational RNAi therapeutic targeting amyloid precursor protein (APP) for the treatment of cerebral amyloid angiopathy (CAA).

Additional Business Highlights

- Entered into an exclusive distribution agreement with Medison Pharma for the commercialization of certain RNAi therapeutics in Israel.
- Resolved all litigation worldwide with Silence Therapeutics.

Upcoming Events in Early 2019

- Alnylam expects to report topline results from the ENVISION Phase 3 study of givosiran in March 2019, and announces today that it plans to present full study results in an oral presentation at the The International Liver Congress™ 2019 of the European Association for the Study of the Liver (EASL) on Saturday, April 13, 2019 in Vienna, Austria.

Financial Results for the Quarter and Year Ended December 31, 2018

"We ended 2018 with cash and investments on our balance sheet of $1.13 billion, exceeding our 2018 guidance, and we're pleased to have recently strengthened our balance sheet further with our public offering in January resulting in net proceeds of approximately $382 million," said Manmeet Soni, Chief Financial Officer of Alnylam. "Taken together with our growing product revenues, we believe Alnylam is in a strong position to continue executing on global commercialization plans while advancing our pipeline of late- and early-stage programs."

Cash and Investments

At December 31, 2018, Alnylam had cash, cash equivalents and marketable debt securities and restricted investments, excluding equity securities, of $1.13 billion, as compared to $1.73 billion at December 31, 2017.

In January 2019, 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 $77.50 per share. As a result of the offering, Alnylam received aggregate net proceeds of approximately $382 million.

GAAP and Non-GAAP Net Loss

The net loss according to accounting principles generally accepted in the U.S. (GAAP) for the fourth quarter of 2018 was $211.4 million, or $2.09 per share on both a basic and diluted basis, as compared to a net loss of $142.2 million, or $1.48 per share on both a basic and diluted basis, for the same period in the previous year. For the year ended December 31, 2018, the net loss was $761.5 million, or $7.57 per share on both a basic and diluted basis, as compared to a net loss of $490.9 million, or $5.42 per share on both a basic and diluted basis, for the prior year.

The non-GAAP net loss for the fourth quarter of 2018 was $183.5 million, or $1.82 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of $115.1 million, or $1.20 per share on both a basic and diluted basis for the same period in the previous year. The non-GAAP net loss for the year ended December 31, 2018 was $624.3 million, or $6.21 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of $398.1 million, or $4.40 per share on both a basic and diluted basis, for the prior year.

The non-GAAP net loss excludes stock-based compensation expense and gain on litigation settlement. 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 $12.1 million in the fourth quarter of 2018. Net product revenues from sales of ONPATTRO were $12.5 million in the year ended December 31, 2018.

Net Revenues from Collaborators

Net revenues from collaborators were $9.0 million in the fourth quarter of 2018, as compared to $37.9 million in the fourth quarter of 2017. Net revenues from collaborators were $62.4 million in the year ended December 31, 2018, as compared to $89.9 million in the year ended December 31, 2017.

GAAP and Non-GAAP Research and Development Expenses

GAAP research and development (R&D) expenses were $131.0 million in the fourth quarter of 2018 as compared to $117.8 million in the fourth quarter of 2017. GAAP R&D expenses were $505.4 million in the year ended December 31, 2018 as compared to $390.6 million for the prior year.

Non-GAAP R&D expenses were $118.1 million in the fourth quarter of 2018 as compared to $102.9 million in the fourth quarter of 2017. Non-GAAP R&D expenses were $424.9 million in the year ended December 31, 2018 as compared to $338.8 million for the prior year. 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 $108.7 million in the fourth quarter of 2018 as compared to $67.5 million in the fourth quarter of 2017. GAAP SG&A expenses were $382.4 million in the year ended December 31, 2018 as compared to $199.4 million for the prior year.

Non-GAAP SG&A expenses were $93.7 million in the fourth quarter of 2018 as compared to $55.2 million in the fourth quarter of 2017. Non-GAAP SG&A expenses were $305.1 million in the year ended December 31, 2018 as compared to $158.4 million for the prior year. 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.

2019 Financial Guidance

Alnylam expects its 2019 annual non-GAAP R&D expenses to be in the range of $520 to $560 million and non-GAAP SG&A expenses to be in the range of $390 to $420 million. Both non-GAAP R&D and non-GAAP SG&A expenses exclude stock-based compensation expenses.

The Company expects its current cash, cash equivalents, and marketable debt securities will support company operations for approximately two years based upon its current operating plan.

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.

The Company does not provide in this press release a reconciliation of its estimated 2019 non-GAAP R&D and non-GAAP SG&A expense guidance to the comparable GAAP measures because it is not able to estimate 2019 stock-based compensation expense without unreasonable efforts. The Company's stock-based compensation expense is subject to significant fluctuations from period to period due to variability in the probability of performance-based vesting events for stock options and restricted stock units and changes in the Company's stock price which materially impacts the recognition, timing of expense and fair value of these awards. In addition, we believe such reconciliations for our 2019 financial guidance would imply a degree of precision that would be confusing or misleading to investors.

Conference Call Information

Management will provide an update on the Company and discuss fourth quarter and year end 2018 results as well as expectations for the future via conference call on Thursday, February 7, 2019 at 4:30 pm 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 4263166. A replay of the call will be available beginning at 7:30 pm 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 4263166.

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 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 TTR messenger RNA, thereby blocking the production of TTR protein before it is made. Patisiran blocks the production of TTR 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 LNP Technology

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

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 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)/ocular 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 patisiran, vutrisiran, givosiran, lumasiran, inclisiran, fitusiran, ALN-AAT02, cemdisiran, ALN-HBV02 and ALN-APP, its plans for additional global regulatory filings and product launches for ONPATTRO, its expectations regarding the completion of additional VBAs with U.S. commercial payers and the status of pricing and reimbursement procedures outside the U.S., its expectations regarding the timing for the initiation of its APOLLO-B Phase 3 study of patisiran, its plans to initiate the HELIOS-B Phase 3 study for vutrisiran in 2019, its expectations regarding the timing and reporting of complete topline results from the ENVISION Phase 3 study of givosiran and, if positive, for the submission of full clinical sections of an NDA with the FDA, its expectations regarding the timing of topline results from its ILLUMINATE-A Phase 3 study of lumasiran and a possible filing of global regulatory submissions starting 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 2 study of cemdisiran in IgA nephropathy, its expectations regarding the timing of initial results from its Phase 1/2 study for ALN-AAT02 and Vir’s Phase 1/2 study of ALN-HBV02, its expected range of 2019 annual non-GAAP R&D expenses and non-GAAP SG&A expenses, its expectations regarding the length of time its current cash, cash equivalents and marketable debt securities will support company operations based on its current operating plan, 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 December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Revenues:				
Product revenues, net	$ 12,075	$ —	$ 12,535	$ —
Net revenues from collaborators	8,958	37,924	62,373	89,912
Total revenues	21,033	37,924	74,908	89,912
Cost and expenses:				
Cost of goods sold	1,665	—	1,802	—
Research and development	131,036	117,772	505,420	390,635
Selling, general and administrative	108,688	67,455	382,359	199,365
Total costs and expenses	241,389	185,227	889,581	590,000
Loss from operations	(220,356)	(147,303)	(814,673)	(500,088)
Other income (expense):				
Interest income	10,571	4,235	29,262	12,236
Other (expense) income	(1,295)	841	4,173	(3,022)
Gain on litigation settlement	—	—	20,564	—
Total other income	9,276	5,076	53,999	9,214
Loss before income taxes	(211,080)	(142,227)	(760,674)	(490,874)
Provision for income taxes	(361)	—	(823)	—
Net loss	$ (211,441)	$ (142,227)	$ (761,497)	$ (490,874)
Net loss per common share - basic and diluted	$ (2.09)	$ (1.48)	$ (7.57)	$ (5.42)
Weighted-average common shares used to compute basic and diluted net loss per common share	101,066	96,139	100,590	90,554
Comprehensive loss:				
Net loss	$ (211,441)	$ (142,227)	$ (761,497)	$ (490,874)
Unrealized gain (loss) on marketable securities, net of tax	179	(692)	1,220	(2,886)
Reclassification adjustment for realized loss on marketable securities included in net loss	—	—	—	1,894
Comprehensive loss	$ (211,262)	$ (142,919)	$ (760,277)	$ (491,866)

ALNYLAM PHARMACEUTICALS, INC.

RECONCILIATION OF SELECTED GAAP MEASURES TO NON-GAAP MEASURES

(In thousands, except per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Reconciliation of GAAP to Non-GAAP Research and development:				
GAAP Research and development	$ 131,036	$ 117,772	$ 505,420	$ 390,635
Less: Stock-based compensation expenses	(12,972)	(14,837)	(80,509)	(51,872)
Non-GAAP Research and development	$ 118,064	$ 102,935	$ 424,911	$ 338,763
Reconciliation of GAAP to Non-GAAP Selling, general and administrative:				
GAAP Selling, general and administrative	$ 108,688	$ 67,455	$ 382,359	$ 199,365
Less: Stock-based compensation expenses	(15,001)	(12,280)	(77,243)	(40,947)
Non-GAAP Selling, general and administrative	$ 93,687	$ 55,175	$ 305,116	$ 158,418
Reconciliation of GAAP to Non-GAAP Operating costs and expenses:				
GAAP Operating costs and expenses	$ 241,389	$ 185,227	$ 889,581	$ 590,000
Less: Stock-based compensation expenses	(27,973)	(27,117)	(157,752)	(92,819)
Non-GAAP Operating costs and expenses	$ 213,416	$ 158,110	$ 731,829	$ 497,181

Reconciliation of GAAP to Non-GAAP Net loss:				
GAAP Net loss	$ (211,441)	$ (142,227)	$ (761,497)	$ (490,874)
Add: Stock-based compensation expenses	27,973	27,117	157,752	92,819
Less: Gain on litigation settlement	—	—	(20,564)	—
Non-GAAP Net loss	$ (183,468)	$ (115,110)	$ (624,309)	$ (398,055)
Reconciliation of GAAP to Non-GAAP Net loss per common share-basic and diluted:				
GAAP Net loss per common share - basic and diluted	$ (2.09)	$ (1.48)	$ (7.57)	$ (5.42)
Add: Stock-based compensation expenses	0.27	0.28	1.57	1.02
Less: Gain on litigation settlement	—	—	(0.21)	—
Non-GAAP Net loss per common share - basic and diluted	$ (1.82)	$ (1.20)	$ (6.21)	$ (4.40)

ALNYLAM PHARMACEUTICALS, INC.

UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	December 31, 2018	**December 31, 2017**
Cash, cash equivalents and marketable debt securities	$ 1,082,949	$ 1,704,537
Restricted investments	44,825	30,000
Accounts receivable, net	18,760	34,002
Inventory	24,068	—
Prepaid expenses and other assets	83,542	44,291
Property, plant and equipment, net	320,658	181,900
Total assets	$ 1,574,802	$ 1,994,730
Accounts payable, accrued expenses and other liabilities	$ 177,392	$ 104,905
Total deferred revenue	3,954	84,780
Total deferred rent	61,491	8,614
Long-term debt	30,000	30,000
Total stockholders' equity (101.2 million and 99.7 million common shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively)	1,301,965	1,766,431
Total liabilities and stockholders' equity	$ 1,574,802	$ 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.

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Source: Alnylam Pharmaceuticals, Inc.

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