



## Alnylam Pharmaceuticals Reports Second Quarter 2019 Financial Results and Highlights Recent Period Activity

August 6, 2019

– Achieved Second Quarter 2019 ONPATTRO® Global Net Product Revenues of \$38.2 Million with More Than 500 Patients on Commercial Product Worldwide –

– Reported Positive Results from ENVISION Phase 3 Study of Givosiran, Submitted Regulatory Filings in the U.S. and EU, and Received Priority Review from FDA –

– Completed Enrollment in ILLUMINATE-A Phase 3 Study of Lumasiran and Advanced Multiple Additional Late-Stage Pipeline Programs –

– Formed Broad Collaboration with Regeneron to Discover, Develop and Commercialize RNAi Therapeutics Focused on Ocular and Central Nervous System (CNS) Diseases –

– Strengthened Balance Sheet and Ended Second Quarter with Approximately \$2.0 Billion in Cash; Lowers 2019 Non-GAAP R&D and Non-GAAP SG&A Expense Guidance –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 6, 2019-- [Alnylam Pharmaceuticals, Inc.](https://www.alnylam.com) (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the second quarter 2019 and reviewed recent business highlights.

“Over the past quarter, we’re pleased with the continued strong progress in the global launch of ONPATTRO. We believe that continued commercial execution with ONPATTRO and expected upcoming launches of other products puts us on a path toward attaining self-sustainability in our business, delivering on the promise of RNAi therapeutics for patients around the world,” said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. “During this period we also achieved several key milestones with our late and earlier stage pipeline, including positive Phase 3 results with givosiran. We expect this track record of commercial and R&D execution to continue well into the future. Specifically, as we turn to the second half of 2019, we look forward to pivotal data readouts from two programs – inclisiran and lumasiran – and additional Phase 3 initiations, namely APOLLO-B with patisiran, HELIOS-B with vutrisiran, and ILLUMINATE-C with lumasiran. Each of these planned milestones will bring us closer to achieving our *Alnylam 2020* vision of building a multi-product, global biopharmaceutical company that includes a deep clinical pipeline to fuel continued growth and a robust product engine for sustainable and organic innovation for the future, a profile rarely achieved in the biopharmaceutical industry.”

“As we approach the one year anniversary of the ONPATTRO approval, we couldn’t be more proud of our commercial execution. We finished the quarter with over 500 patients on commercial therapy, and we expect continued and steady growth in the years to come driven by new patient finding, global expansion, and additional evidence generation in our ATTR amyloidosis franchise,” said Barry Greene, President of Alnylam. “We are committed to decreasing the time to diagnosis and treatment for the benefit of patients with hATTR amyloidosis with polyneuropathy, and we see increased utilization of diagnostic programs such as Alnylam Act. With recent approvals in Japan and Canada, and multiple pricing or reimbursement approvals enabling commercial sales in over ten countries across the CEMEA region, we are expanding our global footprint, bringing ONPATTRO to patients internationally and laying the groundwork for planned future launches of other RNAi therapeutics. Finally, we believe that evidence generation for ONPATTRO will continue to demonstrate the potential benefits and differentiated profile of ONPATTRO.”

### Second Quarter 2019 and Recent Significant Corporate Highlights

#### Commercial Performance in Second Quarter 2019

- Achieved global ONPATTRO net product revenues for the second quarter of 2019 of \$38.2 million.
- Attained more than 500 patients worldwide on commercial ONPATTRO treatment as of June 30, 2019.
- Received marketing authorization approvals for ONPATTRO in Japan and Canada.
- Continued progress with market access efforts across the CEMEA region (Canada, Europe, Middle East, and Africa).
  - Achieved recent reimbursement approvals and favorable ratings from health technology assessment agencies in England, Scotland, Germany, France, Canada, and Sweden, with significant progress in several additional markets.

#### 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, with plans to initiate the APOLLO-B Phase 3 study in ATTR amyloidosis with cardiomyopathy in mid-2019.
  - [Presented](#) positive 12-month data from the Global Open-Label Extension (OLE) study, as well as new analyses from the APOLLO Phase 3 study in patients previously treated with tafamidis and results from an indirect treatment comparison of patisiran versus inotersen in hATTR amyloidosis patients with polyneuropathy.
- Advanced vutrisiran (ALN-TTRsc02), a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
  - Continued enrollment in the HELIOS-A Phase 3 study of vutrisiran in hereditary ATTR amyloidosis patients with polyneuropathy.
  - The Company announces today that it has obtained regulatory alignment on the design of HELIOS-B – a Phase 3

study of vutrisiran in patients with both hereditary and wild-type ATTR amyloidosis cardiomyopathy – which it expects to start in late 2019.

- Advanced givosiran, an investigational RNAi therapeutic in development for the treatment of acute hepatic porphyria (AHP).
  - Presented [positive results](#) from the ENVISION Phase 3 study.
  - Completed submissions of a New Drug Application (NDA) with the U.S. Food and Drug Administration (FDA) and a Marketing Authorisation Application (MAA) with the European Medicines Agency (EMA); both agencies have accepted the applications for filing. The FDA also granted the Company's request for Priority Review and has set an action date of February 4, 2020, under the Prescription Drug User Fee Act (PDUFA). At this time, the FDA is not planning to hold an advisory committee meeting to discuss this application.
  - Alnylam announces today that it has initiated an Expanded Access Program for givosiran to support requests by Health Care Providers for pre-approval access for AHP patients.
- Advanced lumasiran, an investigational RNAi therapeutic in development for the treatment of primary hyperoxaluria type 1 (PH1).
  - Completed enrollment in the ILLUMINATE-A Phase 3 study of lumasiran in PH1 patients six years of age or older with mild-to-moderate renal impairment, and remain on track to report results by year-end 2019.
  - [Presented](#) complete positive results from the Phase 1/2 clinical study and [positive results](#) from the ongoing Phase 2 open-label extension (OLE) study of lumasiran.
  - Initiated ILLUMINATE-B, a global Phase 3 pediatric study of lumasiran in PH1 patients under six years of age.
- Alnylam's partner, The Medicines Company, reported new results for inclisiran, an investigational RNAi therapeutic in development for the treatment of hypercholesterolemia.
  - New data included [interim results](#) from the ongoing ORION-3 OLE study in patients with atherosclerotic cardiovascular disease (ASCVD) or ASCVD-risk equivalents and [results](#) from the ORION-2 and -7 studies in patients with homozygous familial hypercholesterolemia (HoFH) and in patients with renal impairment, respectively.
  - In addition, The Medicines Company announced that the Independent Data Monitoring Committee for ongoing inclisiran Phase 3 clinical trials (ORION 9, 10, and 11) completed its seventh 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 3,500 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, reported new results from the Phase 2 OLE study of fitusiran, an investigational RNAi therapeutic in development for the treatment of hemophilia.
- Advanced early- and mid-stage RNAi clinical pipeline.
  - Initiated a Phase 1 study of ALN-AGT, an investigational RNAi therapeutic targeting angiotensinogen (AGT) for the treatment of hypertension in high unmet need populations, including patients with resistant or refractory hypertension, chronic kidney disease or heart failure.
- Announced new platform advances, including [preclinical results](#) demonstrating oral delivery of GalNAc-conjugated small interfering RNAs (siRNAs) directed to a liver target. Oral delivery could broaden the clinical and commercial opportunities for RNAi therapeutics, which are currently administered with intravenous or subcutaneous dose administration.

#### **Additional Business Highlights**

- Formed a broad collaboration with Regeneron Pharmaceuticals, Inc. (Regeneron) to discover, develop, and commercialize RNAi therapeutics focused on ocular and CNS diseases.
- Concluded the research and option phase of the Company's 2014 collaboration with Sanofi focused on advancing RNAi therapeutics for rare genetic diseases.
- Entered into a collaboration with 23andMe to support the addition of a new Genetic Health Risk report for Hereditary Amyloidosis (TTR-related). Read more about the report [here](#).
- Announced senior leadership changes, including the appointment of Kelley Boucher as the Company's Senior Vice President, Chief Human Resources Officer; and Jeff Poulton as Executive Vice President, Chief Financial Officer, effective August 13.

#### **Upcoming Events**

In the second half of 2019, Alnylam intends to:

- Initiate the APOLLO-B Phase 3 study of patisiran in ATTR amyloidosis patients with cardiomyopathy in mid-2019.
- Launch ONPATTRO in Japan, England, Switzerland, and multiple other countries.
- Initiate the HELIOS-B Phase 3 study of vutrisiran in ATTR amyloidosis patients with cardiomyopathy.
- Initiate the ILLUMINATE-C Phase 3 study of lumasiran in PH1 patients with severe renal impairment.
- Report topline results from the ILLUMINATE-A Phase 3 study of lumasiran in PH1 patients six years of age or older.

In addition, The Medicines Company intends to report initial topline results from the ORION-9, 10, and 11 Phase 3 studies of inclisiran, and assuming

positive results, to file an NDA.

## **Financial Results for the Quarter Ended June 30, 2019**

"Alnylam had strong financial performance in the second quarter. We ended with cash and cash equivalents on our balance sheet of approximately \$2.0 billion, bolstered by robust ONPATTRO sales as well as \$800 million in additional cash received from our recently announced collaboration with Regeneron," said Manmeet Soni, outgoing Chief Financial Officer of Alnylam. "I have thoroughly enjoyed my time as part of the Alnylam team and am confident that the foundation created over the past few years will serve the Company well."

"I am thrilled to be joining an organization with great near- and long-term growth prospects driven by advancing innovative therapies with the potential to transform patients' lives," said Jeff Poulton, recently appointed Executive Vice President, Chief Financial Officer of Alnylam, effective August 13. "Having supported the profitable globalization of Shire's business during my tenure there, I look forward to partnering with the business team at Alnylam to develop a roadmap toward financial self-sustainability."

### *Cash and Investments*

At June 30, 2019, Alnylam had cash, cash equivalents and marketable debt securities, and restricted investments, excluding equity securities, of \$1.97 billion, as compared to \$1.13 billion at December 31, 2018.

In May 2019, Alnylam received an upfront collaboration payment from Regeneron of \$400 million. In addition, Regeneron purchased \$400 million of Alnylam equity at a price per share of \$90.00 (4.44 million common shares).

### *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 2019 was \$219.5 million, or \$2.02 per share on both a basic and diluted basis, as compared to a net loss of \$163.6 million, or \$1.63 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 2019 was \$198.3 million, or \$1.83 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of \$161.9 million, or \$1.61 per share on both a basic and diluted basis for the same period in the previous year.

Reconciling items between GAAP and non-GAAP net loss include stock-based compensation expense, a gain on the change in fair value of a liability obligation related to the sale of common stock to Regeneron, and a gain on a 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 \$38.2 million in the second quarter of 2019.

### *Net Revenues from Collaborators*

Net revenues from collaborators were \$6.5 million in the second quarter of 2019 as compared to \$29.9 million in the second quarter of 2018.

### *GAAP and Non-GAAP Research and Development Expenses*

GAAP research and development (R&D) expenses were \$163.9 million in the second quarter of 2019 as compared to \$137.6 million in the second quarter of 2018.

Non-GAAP R&D expenses were \$148.6 million in the second quarter of 2019 as compared to \$126.0 million in the second quarter of 2018. 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 \$112.8 million in the second quarter of 2019 as compared to \$84.7 million in the second quarter of 2018.

Non-GAAP SG&A expenses were \$97.4 million in the second quarter of 2019 as compared to \$74.1 million in the second quarter of 2018. 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 Updated Financial Guidance**

Alnylam is updating its 2019 annual non-GAAP R&D expenses to be in the range of \$550 to \$575 million (previously \$550 to \$590 million) and non-GAAP SG&A expenses to be in the range of \$390 to \$400 million (previously \$390 million to \$410 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 multiple 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, a gain on the change in fair value of a liability obligation, and a 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 a gain on the change in fair value of liability obligation and the gain on litigation settlement because the Company believes these items are one-time events 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 impact the recognition, timing of expense and fair value of these awards. In addition, the Company believes such reconciliations for its 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 second quarter 2019 results as well as expectations for the future via conference call on Tuesday, August 6, 2019 at 8:00 am ET. To access the call, please dial 800-263-0877 (domestic) or 646-828-8143 (international) five minutes prior to the start time and refer to conference ID 9566932. A replay of the call will be available beginning at 11:00 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 9566932.

#### **About ONPATTRO<sup>®</sup> (patisiran)**

ONPATTRO is an RNAi therapeutic that is approved by the U.S. Food and Drug Administration

(FDA) for the treatment of the polyneuropathy of hATTR amyloidosis in adults. ONPATTRO is

also approved in the European Union for the treatment of hATTR amyloidosis in adults with Stage 1 or Stage 2 polyneuropathy, and in Japan for the treatment of hATTR amyloidosis with polyneuropathy by the Japanese Ministry of Health, Labour and Welfare (MHLW). Based on Nobel Prize-winning science, ONPATTRO 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. ONPATTRO 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.

#### **Important Safety Information**

ONPATTRO is a medicine that treats the polyneuropathy caused by an illness called hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis). ONPATTRO is used in adults only.

#### *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 infections (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 whole new class of innovative medicines with the potential to transform 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. Alnylam's first commercial RNAi therapeutic is ONPATTRO<sup>®</sup> (patisiran), approved in the U.S., EU, Canada, and Japan. Alnylam has a deep

pipeline of investigational medicines, including five 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 1,200 people worldwide and is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit [www.alnylam.com](http://www.alnylam.com) and engage with us on Twitter at [@Alnylam](https://twitter.com/Alnylam) or on [LinkedIn](https://www.linkedin.com/company/alnylam).

#### Alnylam Forward Looking Statements

Various statements in this release concerning Alnylam's future expectations, plans and prospects, including, without limitation, Alnylam's views with respect to the potential for RNAi therapeutics, including patisiran, vutrisiran, givosiran, lumasiran, inclisiran, fitusiran and ALN-AGT, its expectations with respect to broadened clinical and commercial opportunities enabled by oral delivery of RNAi therapeutics based on preclinical results, its plans for additional evidence generation, global regulatory filings and product launches for ONPATTRO, its expectations regarding 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 late 2019, its expectations regarding the regulatory review of givosiran, including the PDUFA date set by the FDA, and expectations regarding the FDA's current views on not requiring an advisory committee meeting to discuss the givosiran application, its expectations regarding the timing of topline results from its ILLUMINATE-A Phase 3 study of lumasiran, its plans to initiate the ILLUMINATE-C Phase 3 study of lumasiran, its expectations regarding the timing of topline results to be reported by The Medicines Company from the ORION-11, ORION-9 and ORION-10 studies of inclisiran and the potential filing of an NDA, 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, plans to develop a roadmap toward financial self-sustainability, 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, including Regeneron, 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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
<b>Revenues:</b>				
Product revenues, net	\$ 38,231	\$ —	\$ 64,522	\$ —

Net revenues from collaborators	6,483	29,907	13,486	51,806
<b>Total revenues</b>	<b>44,714</b>	<b>29,907</b>	<b>78,008</b>	<b>51,806</b>
<b>Cost and expenses:</b>				
Cost of goods sold	\$ 4,326	\$ —	7,673	\$ —
Research and development	163,890	137,582	293,017	234,439
Selling, general and administrative	112,769	84,679	202,377	157,126
Total costs and expenses	280,985	222,261	503,067	391,565
Loss from operations	(236,271)	(192,354)	(425,059)	(339,759)
<b>Other income (expense):</b>				
Interest income	8,781	6,101	16,306	11,895
Other (expense) income	(453)	2,208	(410)	2,543
Change in fair value of liability obligation	9,422	—	9,422	—
Gain on litigation settlement	—	20,564	—	20,564
Total other income	17,750	28,873	25,318	35,002
Loss before income taxes	(218,521)	(163,481)	(399,741)	(304,757)
Provision for income taxes	(960)	(79)	(1,655)	(17)
Net loss	\$ (219,481)	\$ (163,560)	\$ (401,396)	\$ (304,774)
Net loss per common share - basic and diluted	\$ (2.02)	\$ (1.63)	\$ (3.75)	\$ (3.04)
Weighted-average common shares used to compute basic and diluted net loss per common share	108,576	100,519	106,997	100,251
<b>Comprehensive loss:</b>				
Net loss	\$ (219,481)	\$ (163,560)	\$ (401,396)	\$ (304,774)
Unrealized gain on marketable securities, net of tax	462	1,046	822	626
Foreign currency translation	842	—	842	—
Unrealized loss on pension obligation	(4,282)	—	(4,282)	—

Comprehensive loss	\$ (222,459)	\$ (162,514)	\$ (404,014)	\$ (304,148)
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**ALNYLAM PHARMACEUTICALS, INC.**

**RECONCILIATION OF SELECTED GAAP MEASURES TO NON-GAAP MEASURES**

(In thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
<b>Reconciliation of GAAP to Non-GAAP Research and development:</b>				
GAAP Research and development	\$ 163,890	\$ 137,582	\$ 293,017	\$ 234,439
Less: Stock-based compensation expenses	(15,282)	(11,616)	(31,407)	(21,753)
Non-GAAP Research and development	\$ 148,608	\$ 125,966	\$ 261,610	\$ 212,686
<b>Reconciliation of GAAP to Non-GAAP Selling, general and administrative:</b>				
GAAP Selling, general and administrative	\$ 112,769	\$ 84,679	\$ 202,377	\$ 157,126
Less: Stock-based compensation expenses	(15,321)	(10,625)	(31,228)	(20,072)
Non-GAAP Selling, general and administrative	\$ 97,448	\$ 74,054	\$ 171,149	\$ 137,054
<b>Reconciliation of GAAP to Non-GAAP Operating expenses:</b>				
GAAP Operating expenses	\$ 280,985	\$ 222,261	\$ 503,067	\$ 391,565
Less: Stock-based compensation expenses	(30,603)	(22,241)	(62,635)	(41,825)
Non-GAAP Operating expenses	\$ 250,382	\$ 200,020	\$ 440,432	\$ 349,740
<b>Reconciliation of GAAP to Non-GAAP Net loss:</b>				
GAAP Net loss	\$ (219,481)	\$ (163,560)	\$ (401,396)	\$ (304,774)
Add: Stock-based compensation expenses	30,603	22,241	62,635	41,825

Less: Change in fair value of liability obligation	(9,422)	—	(9,422)	—
Less: Gain on litigation settlement	—	(20,564)	—	(20,564)
Non-GAAP Net loss	\$ (198,300)	\$ (161,883)	\$ (348,183)	\$ (283,513)

**Reconciliation of GAAP to Non-GAAP Net loss per common share-basic and diluted:**

GAAP Net loss per common share - basic and diluted	\$ (2.02)	\$ (1.63)	\$ (3.75)	\$ (3.04)
Add: Stock-based compensation expenses	0.28	0.22	0.59	0.42
Less: Change in fair value of liability obligation	(0.09)	—	(0.09)	—
Less: Gain on litigation settlement	—	(0.20)	—	(0.21)
Non-GAAP Net loss per common share - basic and diluted	\$ (1.83)	\$ (1.61)	\$ (3.25)	\$ (2.83)

**ALNYLAM PHARMACEUTICALS, INC.**

**UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS**

**(In thousands, except share amounts)**

	<b>June 30,</b>	<b>December</b>
	<b>2019</b>	<b>31,</b>
	<b>2018</b>	<b>2018</b>
Cash, cash equivalents and marketable debt securities	\$ 1,926,831	\$ 1,082,949
Restricted investments	44,825	44,825
Accounts receivable, net	30,739	18,760
Inventory	40,587	24,068
Prepaid expenses and other assets	75,303	83,542
Property, plant and equipment, net	371,769	320,658
Operating lease right-of-use lease assets	226,357	—
<b>Total assets</b>	<b>\$ 2,716,411</b>	<b>\$ 1,574,802</b>

Accounts payable, accrued expenses and other liabilities	\$ 219,529	\$ 177,392
Total deferred revenue	403,129	3,954
Total deferred rent	—	61,491
Operating lease liability	306,558	—
Long-term debt	30,000	30,000
Total stockholders' equity (111.1 million shares issued and outstanding at June 30, 2019; 101.2 million shares issued and outstanding at December 31, 2018)	1,757,195	1,301,965
<b>Total liabilities and stockholders' equity</b>	<b>\$ 2,716,411</b>	<b>\$ 1,574,802</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, 2018.

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