



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

October 31, 2019

– Achieved Third Quarter 2019 ONPATTRO® Global Net Product Revenues of \$46.1 Million, Including Initial Sales in Japan, with Over 600 Patients on Commercial Product Worldwide –

– Initiated APOLLO-B Phase 3 Study of Patisiran for the Treatment of Hereditary and Wild-Type ATTR Amyloidosis with Cardiomyopathy –

– Advanced Additional Late-Stage Clinical Programs: On Track to Report Topline ILLUMINATE-A Phase 3 Results with Lumasiran by Year-End –

– Alnylam's Partner, The Medicines Company, Announced Positive Phase 3 Results with Inclisiran, in Development for the Treatment of Hypercholesterolemia –

– Maintained Strong Balance Sheet with \$1.74 Billion in Cash and Investments –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 31, 2019-- [Alnylam Pharmaceuticals, Inc.](#) (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the third quarter 2019 and reviewed recent business highlights.

"In the third quarter of 2019 and recent period we saw strong execution on the global commercialization of ONPATTRO. For the rest of the year and beyond, we expect steady and continued growth in patients on ONPATTRO therapy through improved disease awareness, new patient finding, expansion in global markets – such as our recent launch in Japan and NDA filing in Brazil – and the potential for future label expansion in hereditary and wild-type ATTR cardiomyopathy through our recently initiated APOLLO-B Phase 3 study. We're also pleased to have received a Priority Review and Accelerated Assessment for givosiran from the FDA and EMA, respectively, and we are preparing for the potential launch of our second RNAi therapeutic in the coming months, assuming positive regulatory reviews," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "While executing on our commercial objectives, we also made excellent progress on our late stage pipeline, including positive Phase 3 results for inclisiran announced by our partner, The Medicines Company, representing what we believe is a landmark event for Alnylam and for the entire field of RNAi therapeutics. In the final months of 2019, we aim to extend this encouraging track record with pivotal data from our lumasiran program, continued enrollment in our HELIOS-A Phase 3 trial and initiation of the HELIOS-B Phase 3 study with vutrisiran. Each of these anticipated milestones will bring us closer to achieving our Alnylam 2020 goals of building a multi-product, global biopharma company with a deep clinical pipeline to fuel future growth and a robust product engine for sustainable and organic innovation, a profile rarely achieved in our industry."

### Third Quarter 2019 and Recent Significant Corporate Highlights

#### Commercial Performance in Third Quarter 2019

- Achieved global net product revenues for the third quarter of 2019 of \$46.1 million for ONPATTRO.
- Attained over 600 patients worldwide on commercial ONPATTRO treatment since launch.
- Continued global expansion with receipt of regulatory approval for ONPATTRO in Switzerland, and initiation of commercial launches in Japan and Canada.
- Continued progress with market access efforts across the CEMEA region (**C**anada, **E**urope, **M**iddle **E**ast, and **A**frica).
  - Following favorable ratings from health technology assessment agencies, achieved reimbursement approvals in the United Kingdom, Belgium, and Germany.
- Received recognition for ONPATTRO as an innovative biotechnology medicine through award of the prestigious Prix Galien in The Netherlands and Italy and nominations for the Prix Galien in additional countries, including France, Germany, and the U.S.

#### Late Stage R&D Highlights

- Advanced patisiran (the non-proprietary name for ONPATTRO), an intravenously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
  - Initiated the APOLLO-B Phase 3 study in ATTR amyloidosis patients with cardiomyopathy.
  - Filed a marketing authorization application with the Brazilian Health Regulatory Agency (ANVISA) for the treatment of hereditary ATTR amyloidosis with polyneuropathy.
- Advanced vutrisiran, a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
  - Continued enrollment in the HELIOS-A Phase 3 study in hereditary ATTR amyloidosis patients with polyneuropathy.
  - Aligned with regulatory agencies on the design of the HELIOS-B Phase 3 study in patients with hereditary or wild-type ATTR amyloidosis with cardiomyopathy, and are on track to initiate the study in late 2019.
- Advanced givosiran, an investigational RNAi therapeutic in development for the treatment of acute hepatic porphyria (AHP).

- Received Priority Review from the U.S. Food and Drug Administration (FDA) for the givosiran New Drug Application (NDA). The FDA set an action date of February 4, 2020 under the Prescription Drug User Fee Act (PDUFA), and the agency has indicated that it is not currently planning an advisory committee meeting as part of the NDA review.
  - Completed submission of a Marketing Authorization Application (MAA) under an Accelerated Assessment to the European Medicines Agency (EMA).
  - Presented [new clinical results](#) at the 2019 International Congress on Porphyrins and Porphyrrias.
- Advanced lumasiran, an investigational RNAi therapeutic in development for the treatment of primary hyperoxaluria type 1 (PH1).
  - The Company remains on track to report topline results from the ILLUMINATE-A Phase 3 study in late 2019.
  - Continued enrollment in ILLUMINATE-B, a global Phase 3 pediatric study of lumasiran in PH1 patients under six years of age.
  - Presented [new clinical results](#) on the pediatric cohort of patients from the Phase 1/2 study at the International Pediatric Nephrology Association (IPNA) 2019 Annual Meeting.
- Alnylam's partner, The Medicines Company, reported positive results from Phase 3 studies with inclisiran, an investigational RNAi therapeutic in development for the treatment of hypercholesterolemia, including:
  - Positive complete results from the ORION-11 Phase 3 study in patients with atherosclerotic cardiovascular disease (ASCVD) (ex-U.S.), presented at the European Society of Cardiology's ESC Congress 2019.
  - Positive topline results from the ORION-9 Phase 3 study in patients with heterozygous familial hypercholesterolemia (HeFH).
  - Positive topline results from the ORION-10 Phase 3 study in patients with ASCVD (U.S.-based).

#### **Additional Business Updates**

- Received recognition as the world's #1 biopharma employer from *Science* magazine based on more than 7,500 responses to its annual survey of the biotech and pharmaceutical industry.
- Entered into a U.S. gastroenterologist disease education and post-approval promotional agreement with Ironwood Pharmaceuticals for the investigational RNAi therapeutic givosiran, to augment Alnylam's broader education and commercial activities.
- Announced the +myFamily program as part of the existing collaboration with 23andMe to offer free 23andMe Health + Ancestry kits to first-degree family members of 23andMe customers with a detected TTR variant.
- Announced the appointment of Jeff Poulton as Executive Vice President, Chief Financial Officer.

#### **Upcoming Events**

In late 2019, Alnylam intends to:

- Continue global expansion of ONPATTRO.
- Prepare for the potential launch of givosiran in the U.S. and Europe, assuming regulatory approvals.
- Initiate the HELIOS-B Phase 3 study of vutrisiran in hereditary and wild-type ATTR amyloidosis patients with cardiomyopathy.
- Report topline results from the ILLUMINATE-A Phase 3 study of lumasiran.
- Initiate the ILLUMINATE-C Phase 3 study of lumasiran in PH1 patients with severe renal impairment.
- Present a review of its R&D and commercial activities at the Company's R&D Day on November 22 in New York City.

In addition, The Medicines Company plans to report complete results from the ORION-9 and 10 Phase 3 studies of inclisiran at the American Heart Association Scientific Sessions 2019, taking place November 16 – 18 in Philadelphia. The Medicines Company also plans to file an NDA for inclisiran by year-end 2019.

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

"Having recently joined Alnylam as CFO, I have been very impressed by the strong commercial execution of the organization, building on the Company's heritage of scientific excellence and R&D success. Notably, we finished the third quarter with over 600 patients on commercial ONPATTRO and generated \$46.1 million in global net product revenues with strong growth contributions from both our U.S. and international markets," said Jeff Poulton, Chief Financial Officer of Alnylam. "As we look towards 2020 and beyond, our balance sheet remains strong, supporting further investment in both commercial and R&D programs to drive near- and long-term growth, which we believe will bring us closer to achieving a self-sustainable financial profile for the future."

#### *Cash and Investments*

At September 30, 2019, Alnylam had cash, cash equivalents and marketable debt securities, and restricted investments, excluding equity securities, of \$1.74 billion, as compared to \$1.13 billion at December 31, 2018.

#### *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 2019 was \$208.5 million, or \$1.92 per share on both a basic and diluted basis, as compared to a net loss of \$245.3 million, or \$2.43 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 2019 was \$162.5 million, or \$1.50 per share on both a basic and diluted basis, as compared to a non-GAAP net loss of \$157.3 million, or \$1.56 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 for the third quarter of 2019 and 2018 include 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 \$46.1 million in the third quarter of 2019, as compared to net product revenues of \$0.5 million for the same period in the previous year.

#### *Net Revenues from Collaborators*

Net revenues from collaborators were \$24.0 million in the third quarter of 2019, primarily related to \$15.3 million in revenue from the Regeneron collaboration, as compared to \$1.6 million in the third quarter of 2018.

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

GAAP research and development (R&D) expenses were \$160.8 million in the third quarter of 2019, as compared to \$139.9 million in the third quarter of 2018.

Non-GAAP R&D expenses were \$138.1 million in the third quarter of 2019, as compared to \$94.2 million in the third 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 \$120.4 million in the third quarter of 2019, as compared to \$116.5 million in the third quarter of 2018.

Non-GAAP SG&A expenses were \$97.1 million in the third quarter of 2019, as compared to \$74.4 million in the third 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 Financial Guidance**

Alnylam reiterates its expectations for 2019 non-GAAP R&D expenses to be in the range of \$550 to \$575 million and non-GAAP SG&A expenses to be in the range of \$390 to \$400 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 a 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 third quarter 2019 results as well as expectations for the future via conference call on Thursday, October 31, 2019 at 8:30 am ET. To access the call, please dial 866-548-4713 (domestic) or +1-323-794-2093 (international) five minutes prior to the start time and refer to conference ID 2198008. 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 +1-719-457-0820 (international) and refer to conference ID 2198008.

### **About ONPATTRO® (patisiran)**

ONPATTRO is an RNAi therapeutic that is approved in the United States and Canada for the treatment of the polyneuropathy of hATTR amyloidosis in adults. ONPATTRO is also approved in the European Union and Switzerland 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. 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.

## **ONPATTRO 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, acetaminophen, 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 upper respiratory-tract infections (29 percent) and infusion-related reactions (19 percent).

For additional information about ONPATTRO, please see the full [Prescribing Information](#).

## **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, Switzerland 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](#).

## **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, and inclisiran, its plans for additional global regulatory filings and product launches for ONPATTRO, as well as an additional clinical study to support potential label expansion for 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 reporting of results by The Medicines Company from the ORION-9 and ORION-10 studies of inclisiran and the potential filing of an NDA, its expectations relating to ONPATTRO growth and the 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 and achieve a self-sustainable financial profile in the future, 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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<b>Revenues:</b>				
Product revenues, net	\$ 46,066	\$ 460	\$ 110,588	\$ 460
Net revenue from collaborators	23,995	1,609	37,481	53,415
<b>Total revenues</b>	<b>70,061</b>	<b>2,069</b>	<b>148,069</b>	<b>53,875</b>
<b>Costs and expenses:</b>				
Cost of goods sold	\$ 5,213	\$ 137	\$ 12,886	\$ 137
Research and development	160,796	139,945	453,813	374,384
Selling, general and administrative	120,351	116,545	322,728	273,671
Total costs and expenses	286,360	256,627	789,427	648,192
Loss from operations	(216,299)	(254,558)	(641,358)	(594,317)
<b>Other income (expense):</b>				
Interest income	9,889	6,796	26,195	18,691
Other (expense) income	(2,519)	2,925	(2,929)	5,468

Change in fair value of liability obligation	—	—	9,422	—
Gain on litigation settlement	—	—	—	20,564
Total other income	7,370	9,721	32,688	44,723
Loss before income taxes	(208,929)	(244,837)	(608,670)	(549,594)
Benefit (provision) for income taxes	394	(445)	(1,261)	(462)
Net loss	\$ (208,535)	\$ (245,282)	\$ (609,931)	\$ (550,056)
Net loss per common share - basic and diluted	\$ (1.92)	\$ (2.43)	\$ (5.63)	\$ (5.48)
Weighted-average common shares used to compute basic and diluted net loss per common share	108,701	100,783	108,427	100,430

**Comprehensive loss:**

Net loss	\$ (208,535)	\$ (245,282)	\$ (609,931)	\$ (550,056)
Unrealized (loss) gain on marketable securities, net of tax	(50)	415	772	1,041
Foreign currency translation	1,439	—	2,281	—
Defined benefit pension plans	71	—	(4,211)	—
Comprehensive loss	\$ (207,075)	\$ (244,867)	\$ (611,089)	\$ (549,015)

**ALNYLAM PHARMACEUTICALS, INC.  
RECONCILIATION OF SELECTED GAAP MEASURES TO NON-GAAP MEASURES  
(In thousands, except per share amounts)**

	<b>Three Months Ended</b>		<b>Nine Months Ended</b>	
	<b>September 30,</b>		<b>September 30,</b>	
	<b>2019</b>	<b>2018</b>	<b>2019</b>	<b>2018</b>
<b>Reconciliation of GAAP to Non-GAAP Research and development:</b>				
GAAP Research and development	\$ 160,796	\$ 139,945	\$ 453,813	\$ 374,384
Less: Stock-based compensation expenses	(22,737)	(45,784)	(54,144)	(67,537)
Non-GAAP Research and development	\$ 138,059	\$ 94,161	\$ 399,669	\$ 306,847

**Reconciliation of GAAP to Non-GAAP Selling, general and administrative:**

GAAP Selling, general and administrative	\$ 120,351	\$ 116,545	\$ 322,728	\$ 273,671
Less: Stock-based compensation expenses	(23,272)	(42,170)	(54,500)	(62,242)
Non-GAAP Selling, general and administrative	\$ 97,079	\$ 74,375	\$ 268,228	\$ 211,429

**Reconciliation of GAAP to Non-GAAP Operating expenses:**

GAAP Operating expenses	\$ 286,360	\$ 256,627	\$ 789,427	\$ 648,192
Less: Stock-based compensation expenses	(46,009)	(87,954)	(108,644)	(129,779)
Non-GAAP Operating expenses	\$ 240,351	\$ 168,673	\$ 680,783	\$ 518,413

**Reconciliation of GAAP to Non-GAAP Net loss:**

GAAP Net loss	\$ (208,535)	\$ (245,282)	\$ (609,931)	\$ (550,056)
Add: Stock-based compensation expenses	46,009	87,954	108,644	129,779
Less: Change in fair value of liability obligation	—	—	(9,422)	—
Less: Gain on litigation settlement	—	—	—	(20,564)
Non-GAAP Net loss	\$ (162,526)	\$ (157,328)	\$ (510,709)	\$ (440,841)

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

GAAP Net loss per common share - basic and diluted	\$ (1.92)	\$ (2.43)	\$ (5.63)	\$ (5.48)
Add: Stock-based compensation expenses	0.42	0.87	1.01	1.29
Less: Change in fair value of liability obligation	—	—	(0.09)	—
Less: Gain on litigation settlement	—	—	—	(0.20)
Non-GAAP Net loss per common share - basic and diluted	\$ (1.50)	\$ (1.56)	\$ (4.71)	\$ (4.39)

*Please note that the figures presented above may not sum exactly due to rounding*

	<b>September 30,</b>	<b>December 31,</b>
	<b>2019</b>	<b>2018</b>
Cash, cash equivalents and marketable debt securities	\$ 1,721,058	\$ 1,082,949
Restricted investments	14,825	44,825
Accounts receivable, net	48,109	18,760
Inventory	54,562	24,068
Prepaid expenses and other assets	83,168	83,542
Property, plant and equipment, net	396,456	320,658
Operating lease right-of-use lease assets	223,444	—
<b>Total assets</b>	<b>\$ 2,541,622</b>	<b>\$ 1,574,802</b>
Accounts payable, accrued expenses and other liabilities	\$ 226,376	\$ 177,392
Total deferred revenue	403,538	3,954
Total deferred rent	—	61,491
Operating lease liability	304,621	—
Long-term debt	—	30,000
Total stockholders' equity (111.3 million shares issued and outstanding at September 30, 2019; 101.2 million shares issued and outstanding at December 31, 2018)	1,607,087	1,301,965
<b>Total liabilities and stockholders' equity</b>	<b>\$ 2,541,622</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.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20191031005466/en/>

Source: Alnylam Pharmaceuticals, Inc.

**Alnylam Pharmaceuticals, Inc.**

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