UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 5, 2020

Alnylam Pharmaceuticals, Inc.

Delaware	001-36407	77-0602661
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)
675 West Kendall Street, Henri A. Termeer Square Cambridge, Massachusetts		02142
(Address of Principal Executive Offices)		(Zip Code)

Registrant's telephone number, including area code: (617) 551-8200

Not applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	**Trading Symbol(s)**	**Name of Each Exchange on Which Registered**
Common Stock, $0.01 par value per share	ALNY	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934(§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02. Results of Operations and Financial Condition

On November 5, 2020, Alnylam Pharmaceuticals, Inc. (the "Company") announced its financial results for the quarter ended September 30, 2020. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Item 2.02 and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits

(d) Exhibits

The following exhibit relating to Item 2.02 shall be deemed to be furnished, and not filed:

99.1	Press Release dated November 5, 2020.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: November 5, 2020

ALNYLAM PHARMACEUTICALS, INC.

By: /s/ Jeffrey V. Poulton
Jeffrey V. Poulton
Executive Vice President, Chief Financial Officer

Exhibit 99.1

Alnylam Pharmaceuticals Reports Third Quarter 2020 Financial Results and Highlights Recent Period Activity

− Achieved Third Quarter 2020 ONPATTRO® Global Net Product Revenue of $82.5 Million, Including Over 20% Quarterly U.S. Growth, with More Than 1,150 Patients on Commercial Product Worldwide –

− Achieved Third Quarter 2020 GIVLAARI® Global Net Product Revenue of $16.7 Million, with More Than 150 Patients on Commercial Product Worldwide –

– Received Positive Opinions for OXLUMO™ (lumasiran) and LEQVIO® (inclisiran) from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), Positioning the Company for Potentially Four Approved RNAi Therapeutic Medicines by Year-End 2020 –

– Increased 2020 Guidance Range for ONPATTRO Revenue from $280-$300 Million to $295-$310 Million –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--November 5, 2020--Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the third quarter 2020 and reviewed recent business highlights.

“We are extremely pleased with the performance of ONPATTRO and GIVLAARI in the third quarter, reflecting strong commercial execution and improving market conditions following the more challenging COVID-19 pandemic phase experienced in the second quarter. We’re also excited about the recent positive CHMP opinions for OXLUMO and LEQVIO, moving these potentially transformative investigational RNAi therapeutics closer to approval. We believe that this positions Alnylam to potentially exit 2020 with four revenue-generating products bolstering our sustained growth,” said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. “In addition, our robust late-stage pipeline of investigational medicines continued to advance in the quarter. Notably, we achieved positive results from the ILLUMINATE-B study – demonstrating efficacy and safety for our investigational RNAi therapeutic lumasiran in children under the age of six, including infants. We also achieved continued enrollment in our APOLLO-B and HELIOS-B Phase 3 studies of patisiran and vutrisiran, respectively, in development for the treatment of ATTR amyloidosis with cardiomyopathy. With our recent progress, we now expect to exceed our Alnylam 2020 vision and goals of building a multi-product, global biopharma company with a deep clinical pipeline and a robust, organic product engine to drive future sustainable innovation and value creation. Finally, we look forward to highlighting further progress and future plans at our upcoming virtual R&D Day event in December.”

Third Quarter 2020 and Recent Significant Corporate Highlights

Commercial Performance

ONPATTRO®

- Achieved global net product revenue for the third quarter of 2020 of $82.5 million, representing 24% quarterly growth globally and 21% quarterly growth in the U.S. alone.
- Attained over 1,150 patients worldwide on commercial ONPATTRO treatment as of September 30, 2020.
- Continued progress with market access efforts across the CEMEA region (**C**anada, **E**urope, **M**iddle **E**ast, and **A**frica), with a recent launch in Portugal, conclusion of price negotiations in France, and completion of initial access agreement in Canada.
- Continued global expansion with achievement of regulatory approval in Israel.
- Received the prestigious 2020 Prix Galien USA Award for Best Biotechnology Product.

GIVLAARI®

- Achieved global net product revenue for the third quarter of 2020 of $16.7 million.
- Attained over 150 patients worldwide on commercial GIVLAARI treatment as of September 30, 2020.
- Continued strong market access progress in the U.S., with 10 VBAs finalized to date with commercial payers and confirmed access for over 90% of covered U.S. lives.
- Continued progress with market access efforts across the CEMEA region, with ongoing launch in Germany, cohort Temporary Authorization for Use (ATU) supply in France, and named patient sales in other countries.
    - Received an Improvement of Medical Benefit (ASMR) score of II in France, concluding that GIVLAARI offers significant additional therapeutic value. In 2019, only two new commercial medicines received a similar ASMR score.
    - In addition, obtained a “Considerable Benefit” rating in Germany and secured a strong health technology assessment (HTA) rating in Italy.
- Continued global expansion with approval in Canada and submission of a new drug application in Japan.
- Received the NORD 2020 Industry Innovation Award.

R&D Highlights

- Advanced **patisiran** (the non-proprietary name for ONPATTRO), in development for the treatment of the cardiomyopathy of both hereditary and wild-type ATTR amyloidosis.
  - Continued enrollment in the APOLLO-B Phase 3 study in ATTR amyloidosis patients with cardiomyopathy.
  - Presented additional clinical data with patisiran – including 24-month results from the global open-label extension (OLE) study and results from an open-label study in patients with hATTR amyloidosis post-orthotopic liver transplant – and published findings from an evaluation of patisiran with concomitant or prior use of TTR stabilizers.

- Advanced **vutrisiran**, a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis.
  - Continued treating patients in the fully enrolled HELIOS-A Phase 3 study of vutrisiran in hATTR amyloidosis patients with polyneuropathy, and remain on track to report topline results in early 2021.
  - Continued enrollment in the HELIOS-B Phase 3 study in ATTR amyloidosis patients with cardiomyopathy.
  - Announced potential for a biannual dosing regimen option for vutrisiran, providing support for further product differentiation as a potential best-in-class agent.

- Presented new interim data from the Phase 1/2 open-label extension (OLE) study of **givosiran** (the non-proprietary name for GIVLAARI) in acute hepatic porphyria (AHP).

- Advanced **lumasiran**, an investigational RNAi therapeutic in development for the treatment of primary hyperoxaluria type 1 (PH1).
  - Received a positive CHMP opinion from EMA recommending approval of lumasiran for the treatment of PH1 in patients of all ages. If approved, lumasiran will be marketed in Europe under the brand name OXLUMO™.
  - Received a positive scientific opinion from the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) through the Early Access to Medicines Scheme (EAMS).
  - Presented positive complete results from ILLUMINATE-B, a global Phase 3 pediatric study of lumasiran in PH1 patients less than six years of age, including infants, with preserved renal function.
  - Continued enrollment in the ILLUMINATE-C Phase 3 study of lumasiran for the treatment of advanced PH1 in patients of all ages.

- Alnylam's partner, Novartis, continued advancing **inclisiran**, potentially the first and only RNAi therapeutic cholesterol-lowering treatment. Inclisiran is undergoing regulatory review in the U.S. and EU.
  - Received a positive CHMP opinion from EMA recommending approval of inclisiran for the treatment of adults with hypercholesterolemia or mixed dyslipidemia. If approved, inclisiran will be marketed under the brand name LEQVIO®.

- Alnylam's partner, Sanofi, continued advancement of the ATLAS Phase 3 program for **fitusiran** in patients with hemophilia A or B with and without inhibitors.

- Advanced early- and mid-stage RNAi therapeutic pipeline programs.
  - Continued dosing in the Phase 1 study of **ALN-AGT** in hypertension.
  - In collaboration with Regeneron, advanced **cemdisiran**, an investigational RNAi therapeutic for the treatment of complement-mediated diseases.
    - Continued enrollment in a Phase 2 clinical trial of cemdisiran monotherapy in patients with IgA nephropathy, with topline results expected in 2021.
  - Alnylam's partner Vir Biotechnology presented new data on VIR-2218 (**ALN-HBV02**) at the European Association for the Study of the Liver Digital International Liver Congress.
    - In addition, Vir initiated a Phase 2 combination trial of VIR-2218 with pegylated interferon-alpha (PEG-IFN-α), with initial clinical data anticipated in 2021.
  - The Company is announcing today that it has initiated dosing in a Phase 1 study of **ALN-HSD**, an investigational RNAi therapeutic targeting HSD17B13 in development for the treatment of nonalcoholic steatohepatitis (NASH). ALN-HSD is being advanced in collaboration with Regeneron.
  - The Company is announcing today that it will delay its planned filing of an IND for **ALN-COV** in order to obtain additional pre-clinical efficacy data in models of COVID-19 infection.
    - ALN-COV is part of a multi-target pre-clinical collaboration with Vir Biotechnology.
  - Continued progress with investigational RNAi therapeutics for CNS and ocular diseases, including advancement of **ALN-APP**, in development for the treatment of hereditary cerebral amyloid angiopathy (hCAA) and autosomal dominant Alzheimer's Disease (ADAD), which remains on track for a CTA filing in mid-2021.
    - Regeneron exercised its co-development/co-commercialization option on the ALN-APP program, which Alnylam will lead.

Additional Business Updates

- Barry Greene, former President of Alnylam, transitioned from Alnylam at the end of the third quarter. Yvonne Greenstreet assumed an expanded role as President and Chief Operating Officer on October 1, 2020.
- Closed $150 million R&D funding component of the previously announced $2 billion strategic financing collaboration with Blackstone to accelerate the advancement of RNAi therapeutics.
- Expanded global reach of commercialization activities with new third-party distribution agreement with taiba Middle East.
- Received recognition from *Science* magazine as a top employer.

Upcoming Events

- Alnylam announced today that it intends to present interim results from the Phase 1 trial of ALN-AGT at the American Heart Association Scientific Sessions 2020 on Friday, November 13.
- The Company also announced today that it plans to present a review of its R&D and commercial activities at its upcoming R&D Day event being held virtually December 15 and 16.

In addition, in late 2020, Alnylam intends to:

- Achieve regulatory approval for lumasiran in the EU and an FDA action for lumasiran in late 2020.
- Alnylam's partner Novartis expects regulatory approval for inclisiran in the EU and an FDA action for inclisiran in late 2020.
- Achieve regulatory approval for ONPATTRO in Taiwan.
- Alnylam's partner Regeneron plans to initiate a Phase 1 study of cemdisiran in combination with pozelimab.

Financial Results for the Quarter Ended September 30, 2020

"The third quarter was very strong, with a rebound in ONPATTRO sales growth, particularly in the U.S., and continued impressive early performance with GIVLAARI. While the pandemic headwinds haven't completely receded, we believe our teams have been very effective in meeting the needs of patients. As a result of this continued strength and our cautious optimism for the fourth quarter, we are again revising our full-year revenue guidance for ONPATTRO, increasing the range from $280-$300 million to $295-$310 million," said Jeff Poulton, Chief Financial Officer of Alnylam. "Beyond the commercial business, we also closed the $150 million R&D funding component of the Blackstone strategic financing collaboration, finalizing the last piece of a broad relationship that we believe secures our path to self-sustainability without the need for future equity offerings. Looking ahead, we believe we are well positioned to continue executing on our current commercial portfolio, with potential for two additional RNAi therapeutics coming to market, and advancing our broad pipeline of investigational programs to drive future growth."

Financial Highlights
(in thousands, except per share amounts)

	Three Months Ended September 30,	
	2020	2019
Net product revenues	$ 99,206	$ 46,066
ONPATTRO net product revenues	$ 82,516	$ 46,066
GIVLAARI net product revenues	$ 16,690	$ —
Net revenue from collaborations	$ 26,647	$ 23,995
Cost of goods sold	$ 21,797	$ 5,213
GAAP research and development expenses	$ 161,783	$ 160,796
Non-GAAP research and development expenses	$ 148,080	$ 138,059
GAAP selling, general and administrative expenses	$ 167,472	$ 120,351
Non-GAAP selling, general and administrative expenses	$ 114,498	$ 97,079
GAAP operating loss	$ (225,199)	$ (216,299)
Non-GAAP operating loss	$ (158,522)	$ (170,290)
GAAP net loss	$ (253,291)	$ (208,535)
Non-GAAP net loss	$ (183,597)	$ (162,526)
GAAP net loss per common share - basic and diluted	$ (2.18)	$ (1.92)
Non-GAAP net loss per common share - basic and diluted	$ (1.58)	$ (1.50)

	September 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,833,876	$ 1,536,162

Net Product Revenues

- Net product revenues were $99.2 million in the third quarter 2020 representing 115% growth from the third quarter 2019 primarily as a result of the addition of new patients on therapy and expansion into new markets for ONPATTRO, as well as the ongoing U.S. commercial launch and initial European launch of GIVLAARI.

Net Revenues from Collaborations

- Net revenues from collaborations were $26.6 million in the third quarter 2020, an increase from $24.0 million in the third quarter 2019, primarily due to an increase in revenue recognized from our Vir collaboration.

Research & Development (R&D) and Selling, General & Administrative (SG&A) Expenses

- R&D expenses were relatively flat on a GAAP basis and increased on a non-GAAP basis in the third quarter 2020 compared to the same period in 2019 primarily due to increased expenses associated with clinical and preclinical activities, personnel, and facilities as we continue to support our long-term strategic growth offset by decreased license fees associated with regulatory filings.
- SG&A expenses increased in the third quarter 2020 compared to the same period in 2019 on a GAAP and non-GAAP basis primarily due to increased investment in commercial and medical affairs activity to support the ongoing launches of ONPATTRO and GIVLAARI and initial launch preparation activities for lumasiran. SG&A expenses on a GAAP basis also increased due to a change in estimate of contingent liabilities related to our arbitration with Ionis.

Cash and Investments

- Cash, cash equivalents and marketable securities were $1.83 billion at the end of the third quarter 2020 compared to $1.54 billion at the end of 2019. The increase was primarily due to $600.0 million in proceeds received in the second quarter of 2020 from the sale of future royalties and issuance of common stock to Blackstone, partially offset by cash used in our operations to support overall growth.

A reconciliation of GAAP to non-GAAP results for the current quarter is included in the tables of this press release.

2020 Updated Financial Guidance

Full year 2020 financial guidance consists of the following:

Item	Provided 8/6/2020 ($ millions)	Updated 11/5/2020 ($ millions)
ONPATTRO net product revenues	$280 - $300	$295 - $310
GIVLAARI net product revenues	No guidance provided	Unchanged
Net revenues from collaborations	$100 - $150	Unchanged
GAAP R&D and SG&A expenses	$1,130 - $1,225	$1,160 - $1,255
Non-GAAP R&D and SG&A expenses*	$1,000 - $1,075	Unchanged

*Excludes $160-$180 million of expenses primarily related to stock-based compensation and a change in estimate of contingent liabilities from projected GAAP R&D and SG&A expenses.

The strategic financing collaboration with Blackstone under which Alnylam will receive up to $2 billion is expected to enable Alnylam's achievement of a self-sustainable financial profile without need for future equity financings.

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 expenses, unrealized gains on marketable equity securities, costs associated with our strategic financing collaboration, loss on contractual settlement and change in estimate of contingent liabilities. 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 unrealized gains on marketable equity securities, costs associated with our strategic financing collaboration, loss on contractual settlement and change in estimate of contingent liabilities because the Company believes these items are non-recurring transactions outside the ordinary course of the Company's business.

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

Conference Call Information

Management will provide an update on the Company and discuss third quarter 2020 results as well as expectations for the future via conference call on Thursday, November 5, 2020 at 8:30 am ET. To access the call, please dial 800-239-9838 (domestic) or +1-323-794-2551 (international) five minutes prior to the start time and refer to conference ID 6976021. 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 6976021.

A live audio webcast of the call will be available on the Investors section of the Company's website at www.alnylam.com/events. An archived webcast will be available on the Alnylam website approximately two hours after the event.

About ONPATTRO® (patisiran)

ONPATTRO is an RNAi therapeutic that was 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, Switzerland and Brazil 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. ONPATTRO is an intravenously administered RNAi therapeutic targeting transthyretin (TTR). 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 polyneuropathy associated with the disease. For more information about ONPATTRO, visit ONPATTRO.com.

ONPATTRO Important Safety Information

Infusion-Related Reactions

Infusion-related reactions (IRRs) have been observed in patients treated with ONPATTRO® (patisiran). In a controlled clinical study, 19% of ONPATTRO-treated patients experienced IRRs, compared to 9% 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%) and infusion-related reactions (19%).

For additional information about ONPATTRO, please see the full Prescribing Information.

About GIVLAARI® (givosiran)

GIVLAARI is an RNAi therapeutic targeting aminolevulinic acid synthase 1 (ALAS1) approved in the United States and Brazil for the treatment of adults with acute hepatic porphyria (AHP). GIVLAARI is also approved in the European Union for the treatment of AHP in adults and adolescents aged 12 years and older. In the pivotal study, givosiran was shown to significantly reduce the rate of porphyria attacks that required hospitalizations, urgent healthcare visits or intravenous hemin administration at home compared to placebo. GIVLAARI is Alnylam's first commercially available therapeutic based on its Enhanced Stabilization Chemistry ESC-GalNAc conjugate technology to increase potency and durability. GIVLAARI is administered via subcutaneous injection once monthly at a dose based on actual body weight and should be administered by a healthcare professional. GIVLAARI works by specifically reducing elevated levels of aminolevulinic acid synthase 1 (ALAS1) messenger RNA (mRNA), leading to reduction of toxins associated with attacks and other disease manifestations of AHP. For more information about GIVLAARI, visit GIVLAARI.com.

GIVLAARI Important Safety Information

Contraindications

GIVLAARI is contraindicated in patients with known severe hypersensitivity to givosiran. Reactions have included anaphylaxis.

Anaphylactic Reaction

Anaphylaxis has occurred with GIVLAARI treatment (<1% of patients in clinical trials). Ensure that medical support is available to appropriately manage anaphylactic reactions when administering GIVLAARI. Monitor for signs and symptoms of anaphylaxis. If anaphylaxis occurs, immediately discontinue administration of GIVLAARI and institute appropriate medical treatment.

Hepatic Toxicity

Transaminase elevations (ALT) of at least 3 times the upper limit of normal (ULN) were observed in 15% of patients receiving GIVLAARI in the placebo-controlled trial. Transaminase elevations primarily occurred between 3 to 5 months following initiation of treatment.

Measure liver function tests prior to initiating treatment with GIVLAARI, repeat every month during the first 6 months of treatment, and as clinically indicated thereafter. Interrupt or discontinue treatment with GIVLAARI for severe or clinically significant transaminase elevations. In patients who have dose interruption and subsequent improvement, reduce the dose to 1.25 mg/kg once monthly. The dose may be increased to the recommended dose of 2.5 mg/kg once monthly if there is no recurrence of severe or clinically significant transaminase elevations at the 1.25 mg/kg dose.

Renal Toxicity

Increases in serum creatinine levels and decreases in estimated glomerular filtration rate (eGFR) have been reported during treatment with GIVLAARI. In the placebo-controlled study, 15% of patients receiving GIVLAARI experienced a renally-related adverse reaction. The median increase in creatinine at Month 3 was 0.07 mg/dL. Monitor renal function during treatment with GIVLAARI as clinically indicated.

Injection Site Reactions

Injection site reactions were reported in 25% of patients receiving GIVLAARI in the placebo-controlled trial. Symptoms included erythema, pain, pruritus, rash, discoloration, or swelling around the injection site. One (2%) patient experienced a single, transient, recall reaction of erythema at a prior injection site with a subsequent dose administration.

Drug Interactions

Concomitant use of GIVLAARI increases the concentration of CYP1A2 or CYP2D6 substrates, which may increase adverse reactions of these substrates. Avoid concomitant use of GIVLAARI with CYP1A2 or CYP2D6 substrates for which minimal concentration changes may lead to serious or life-threatening toxicities. If concomitant use is unavoidable, decrease the CYP1A2 or CYP2D6 substrate dosage in accordance with approved product labeling.

Adverse Reactions

The most common adverse reactions that occurred in patients receiving GIVLAARI were nausea (27%) and injection site reactions (25%).

For additional information about GIVLAARI, please see 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 or disease pathway 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 RNAi therapeutics platform. Alnylam’s commercial RNAi therapeutic products are ONPATTRO® (patisiran), approved in the U.S., EU, Canada, Japan, Brazil, and Switzerland, and GIVLAARI® (givosiran), approved in the U.S, EU, and Brazil. Alnylam has a deep pipeline of investigational medicines, including six product candidates that are in late-stage development. Alnylam is executing 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 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 expectations, plans and prospects, including, without limitation, expectations regarding the direct or indirect effects on Alnylam's business, activities and prospects as a result of the COVID-19 pandemic, or delays or interruptions resulting therefrom and the success of Alnylam's mitigation efforts, Alnylam's views and plans with respect to the potential for RNAi therapeutics, including ONPATTRO, GIVLAARI, lumasiran, inclisiran, patisiran, vutrisiran, fitusiran, cemdisiran, ALN-AGT, ALN-HSD, ALN-COV and ALN-APP, its plans for additional global regulatory filings and the continuing product launches of ONPATTRO and GIVLAARI, expectations regarding reimbursement for ONPATTRO and GIVLAARI in various territories and the status of VBA agreements and confirmed access, the advancement of lumasiran and inclisiran through regulatory review and toward the market including the receipt of positive CHMP opinions for both drug candidates, the potential for a biannual dosing regimen option for vutrisiran, the achievement of additional pipeline milestones, including relating to ongoing clinical studies of vutrisiran, the expected timing for the presentation of interim results from the Phase 1 trial of ALN-AGT, the expected timing for filing a CTA for ALN-APP and the revised expectations regarding the filing of an IND for ALN-COV, the initiation of a Phase 1 clinical study of cemdisiran in combination with pozelimab by Regeneron, its expectations relating to continued ONPATTRO and GIVLAARI revenue growth and the further revised expected range of ONPATTRO net product revenues for 2020, the expected range for net revenues from collaborations for 2020, the revised expected range of 2020 aggregate annual non-GAAP and R&D and SG&A expenses and further revised expected range of 2020 aggregate annual GAAP R&D and SG&A expenses, Alnylam's belief that the funding provided by Blackstone should enable Alnylam to achieve a self-sustainable profile without the need for future equity financing, and expectations regarding Alnylam's ability to exceed its "Alnylam 2020" strategic plan announced in 2015 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: the direct or indirect impact of the COVID-19 global pandemic or any future pandemic, such as the scope and duration of the outbreak, government actions and restrictive measures implemented in response, material delays in diagnoses of rare diseases, initiation or continuation of treatment for diseases addressed by Alnylam products, or in patient enrollment in clinical trials, potential supply chain disruptions, and other potential impacts to Alnylam's business, the effectiveness or timeliness of steps taken by Alnylam to mitigate the impact of the pandemic, and Alnylam's ability to execute business continuity plans to address disruptions caused by the COVID-19 or any future pandemic; Alnylam's ability to discover and develop novel drug candidates and delivery approaches and successfully demonstrate the efficacy and safety of its product candidates, including vutrisiran, ALN-AGT, ALN-HSD, ALN-APP and ALN-COV; 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 or its marketed products, including ONPATTRO, GIVLAARI, inclisiran, lumasiran and vutrisiran; obtaining, maintaining and protecting intellectual property; intellectual property matters including potential patent litigation relating to its platform, products or product candidates; obtaining regulatory approval for its product candidates, including lumasiran and inclisiran, and maintaining regulatory approval and obtaining pricing and reimbursement for its products, including ONPATTRO and GIVLAARI; successfully launching, marketing and selling its approved products globally, including ONPATTRO and GIVLAARI and achieving net product revenues for ONPATTRO within its further revised expected range during 2020; 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 within the reduced ranges of guidance provided by Alnylam through the implementation of further discipline in operations to moderate spend and its ability to achieve a self-sustainable financial profile in the future without the need for future equity financing; Alnylam's ability to establish and maintain strategic business alliances and new business initiatives; Alnylam's dependence on third parties, including Regeneron, for development, manufacture and distribution of certain products, including eye and CNS products and ALN-APP, and Vir for the development of ALN-COV and other potential RNAi therapeutics targeting SARS-CoV-2 and host factors for SARS-CoV-2; 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.

This release discusses investigational RNAi therapeutics and uses of previously approved RNAi therapeutics in development and is not intended to convey conclusions about efficacy or safety as to those investigational therapeutics or uses. There is no guarantee that any investigational therapeutics or expanded uses of commercial products will successfully complete clinical development or gain health authority approval.

ALNYLAM PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except per share amounts)
(Unaudited)

	Three Months Ended		Nine Months Ended	
	September 30, 2020	September 30, 2019	September 30, 2020	September 30, 2019
Statements of Operations				
Revenues:				
Net product revenues	$ 99,206	$ 46,066	$ 248,677	$ 110,588
Net revenues from collaborations	26,647	23,995	80,614	37,481
Total revenues	125,853	70,061	329,291	148,069
Operating costs and expenses:				
Cost of goods sold	21,797	5,213	55,028	12,886
Research and development	161,783	160,796	486,350	453,813
Selling, general and administrative	167,472	120,351	422,129	322,728
Total operating costs and expenses	351,052	286,360	963,507	789,427
Loss from operations	(225,199)	(216,299)	(634,216)	(641,358)
Other (expense) income:				
Interest expense	(28,731)	—	(55,979)	—
Interest income	2,072	9,889	10,717	26,195
Other (expense) income	(594)	(2,519)	67,477	(2,929)
Change in fair value of liability obligation	—	—	—	9,422
Total other (expense) income	(27,253)	7,370	22,215	32,688
Loss before income taxes	(252,452)	(208,929)	(612,001)	(608,670)
(Provision) benefit for income taxes	(839)	394	(2,740)	(1,261)
Net loss	$ (253,291)	$ (208,535)	$ (614,741)	$ (609,931)
Net loss per common share - basic and diluted	$ (2.18)	$ (1.92)	$ (5.37)	$ (5.63)
Weighted-average common shares used to compute basic and diluted net loss per common share	115,986	108,701	114,554	108,427

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

	Three Months Ended	
	September 30, 2020	**September 30, 2019**
Reconciliation of GAAP to Non-GAAP research and development:		
GAAP Research and development	$ 161,783	$ 160,796
Less: Stock-based compensation expenses	(13,703)	(22,737)
Non-GAAP Research and development	$ 148,080	$ 138,059
Reconciliation of GAAP to Non-GAAP selling, general and administrative:		
GAAP Selling, general and administrative	$ 167,472	$ 120,351
Less: Stock-based compensation expenses	(23,561)	(23,272)
Less: Costs associated with the strategic financing collaboration	(763)	—
Less: Loss on contractual settlement	(650)	—
Less: Change in estimate of contingent liabilities	(28,000)	—
Non-GAAP Selling, general and administrative	$ 114,498	$ 97,079
Reconciliation of GAAP to Non-GAAP operating loss:		
GAAP operating loss	$ (225,199)	$ (216,299)
Add: Stock-based compensation expenses	37,264	46,009
Add: Costs associated with the strategic financing collaboration	763	—
Add: Loss on contractual settlement	650	—
Add: Change in estimate of contingent liabilities	28,000	—
Non-GAAP operating loss	$ (158,522)	$ (170,290)
Reconciliation of GAAP to Non-GAAP net loss:		
GAAP net loss	$ (253,291)	$ (208,535)
Add: Stock-based compensation expenses	37,264	46,009
Add: Costs associated with the strategic financing collaboration	763	—
Add: Loss on contractual settlement	650	—
Add: Change in estimate of contingent liabilities	28,000	—
Add: Unrealized loss on marketable equity securities	3,017	—
Non-GAAP net loss	$ (183,597)	$ (162,526)
Reconciliation of GAAP to Non-GAAP net loss per common share-basic and diluted:		
GAAP net loss per common share - basic and diluted	$ (2.18)	$ (1.92)
Add: Stock-based compensation expenses	0.32	0.42
Add: Costs associated with the strategic financing collaboration	0.01	—
Add: Loss on contractual settlement	0.01	—
Add: Change in estimate of contingent liabilities	0.24	—
Add: Unrealized gain on marketable equity securities	0.02	—
Non-GAAP net loss per common share - basic and diluted	$ (1.58)	$ (1.50)

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

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

	September 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,833,876	$ 1,536,162
Restricted investments	24,725	14,825
Accounts receivable, net	79,118	43,011
Inventory	84,040	56,348
Prepaid expenses and other assets	95,600	98,412
Property, plant and equipment, net	444,690	425,179
Operating lease right-of-use lease assets	245,234	221,197
Receivable related to the sale of future royalties	500,000	—
Total assets	$ 3,307,283	$ 2,395,134
Accounts payable, accrued expenses and other liabilities	$ 339,473	$ 256,415
Total deferred revenue	380,309	396,204
Operating lease liability	329,454	303,823
Liability related to the sale of future royalties	1,043,024	—
Total stockholders' equity (116.1 million shares issued and outstanding at September 30, 2020; 112.2 million shares issued and outstanding at December 31, 2019)	1,215,023	1,438,692
Total liabilities and stockholders' equity	$ 3,307,283	$ 2,395,134

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, 2019.

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