Alnylam Pharmaceuticals Reports Third Quarter 2021 Financial Results and Highlights Recent Period Activity

Oct 28, 2021

– Achieved Third Quarter 2021 Combined Net Product Revenues of $167 Million for ONPATTRO®, GIVLAARI®, and OXLUMO® –

– Reported Positive Topline 18-Month Results from HELIOS-A Phase 3 Study of Vutrisiran in hATTR Amyloidosis Patients with Polyneuropathy –

– Completed Enrollment Ahead of Schedule in HELIOS-B Phase 3 Study of Vutrisiran in ATTR Amyloidosis Patients with Cardiomyopathy –

– Reiterated 2021 Financial Guidance, Including Combined Net Product Revenues of $640-$665 Million –

– Founding Alnylam CEO John Maraganore to Transition CEO Leadership to Alnylam President Yvonne Greenstreet at Year End –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 28, 2021-- Alnylam Pharmaceuticals, Inc. (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=Alnylam+Pharmaceuticals%2C+Inc.&index=1&md5=694ba12e8b6e848da9537410404a24c1) (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the third quarter ended September 30, 2021 and reviewed recent business highlights.

"Our third quarter commercial performance was highlighted by strength from ONPATTRO, and continued execution on the on-going launches of GIVLAARI and OXLUMO. Another notable achievement was our announcement of positive topline 18-month results from the HELIOS-A Phase 3 study of vutrisiran in patients with hATTR amyloidosis with polyneuropathy, including improvements in exploratory and cardiac amyloid endpoints, which we believe are encouraging hypothesis-generating data ahead of upcoming readouts from the APOLLO-B and HELIOS-B Phase 3 studies of patisiran and vutrisiran, respectively," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "We also made significant progress with many of our other programs, and we remain focused on executing on our '*Alnylam P^{5}x25*' vision, through which we intend to deliver transformative medicines to patients around the world for rare and prevalent diseases, advancing a robust and high-yielding pipeline of first and/or best-in-class product candidates from our organic product engine, while delivering exceptional financial performance. Lastly, with the announcement this morning of my planned transition from Alnylam at year end, I'd like to emphasize how thrilled I am about the future of Alnylam under Yvonne's upcoming stewardship. I have immense confidence in her ability to continue to deliver on the promise of RNAi therapeutics for patients around the world."

Third Quarter 2021 and Recent Significant Corporate Highlights

Commercial Performance

ONPATTRO® (patisiran)

- Achieved global net product revenues for the third quarter of 2021 of $120 million, representing 6% growth compared to Q2 2021.
- Attained over 1,875 hATTR amyloidosis patients with polyneuropathy worldwide on commercial ONPATTRO treatment as of September 30, 2021.

GIVLAARI® (givosiran)

- Achieved global net product revenues for the third quarter of 2021 of $32 million, representing 4% growth compared to Q2 2021.

- Attained over 300 patients worldwide on commercial GIVLAARI treatment as of September 30, 2021.
- Continued geographic expansion for GIVLAARI with pricing and reimbursement approvals in Spain, the United Kingdom, France and Japan.

OXLUMO® (lumasiran)

- Achieved global net product revenues for the third quarter of 2021 of $15 million, representing a 9% decrease compared to Q2 2021, reflecting the transition of the initial bolus of commercial patients from monthly loading dose to quarterly maintenance dose regimens.
- Attained over 120 patients worldwide on commercial OXLUMO treatment as of September 30, 2021.
- Continued planned geographic expansion for OXLUMO with ongoing pricing and reimbursement discussions in many European countries.

Leqvio® (inclisiran)

- Alnylam earned royalty revenues of $0.5 million from Novartis based on Leqvio global net product revenues in the third quarter of 2021.
- Novartis announced a commercial agreement with the NHS in England as part of a collaboration to pioneer a first-of-its-kind population health management approach to address elevated LDL-C in eligible patients with ASCVD across England.
- Leqvio is now approved in more than 45 countries, with most awaiting reimbursement, and remains on track for U.S. launch with FDA action date of January 1, 2022.

R&D Highlights

Vutrisiran, a subcutaneously administered investigational RNAi therapeutic in development for the treatment of ATTR amyloidosis

- Reported positive topline results for 18-month endpoints and safety from the HELIOS-A Phase 3 study in hATTR amyloidosis patients with polyneuropathy.
- Completed enrollment in the HELIOS-B Phase 3 study in ATTR amyloidosis patients with cardiomyopathy, ahead of schedule.
- Submitted a Marketing Authorization Application to the European Medicines Agency, ahead of schedule, and a New Drug Application to ANVISA in Brazil for the treatment of in hATTR amyloidosis in adult patients with polyneuropathy.

Lumasiran (the non-proprietary name for OXLUMO), for the treatment of primary hyperoxaluria type 1 (PH1)

- Reported positive topline results from the ILLUMINATE-C Phase 3 study in patients with advanced PH1.

Cemdisiran, an investigational RNAi therapeutic in development for the treatment of complement-mediated diseases

- The Company announces today that its topline results from a Phase 2 study of cemdisiran in IgA nephropathy are now expected in early 2022.

Fitusiran, an investigational RNAi therapeutic in development for the treatment of hemophilia A or B with and without inhibitors, in collaboration with Sanofi

- Sanofi announced that a potential filing date for fitusiran has been moved to 2024 due to the introduction of a lower dose cohort in the ongoing Phase 3 studies.

Early- and mid-stage investigational RNAi therapeutic pipeline programs and RNAi platform

- Alnylam's partner Vir Biotechnology initiated a Phase 2 clinical trial evaluating the combination of **ALN-HBV02 (VIR-2218)** and VIR-3434 as a functional cure regimen for chronic hepatitis B virus infection.
- Advanced **ALN-HSD** into Part B of the ongoing Phase 1 study in patients with non-alcoholic steatohepatitis (NASH).
- Revealed hexadecyl (C16) lipophilic conjugate for potent and effective delivery of siRNAs in the CNS.
- Presented pre-clinical data with IKARIA platform and **ALN-TTRsc04** demonstrating potential to achieve over 90% target mRNA knockdown with an annual dosing regimen.

Additional Business Updates

- Entered into a strategic collaboration with PeptiDream Inc. to discover and develop peptide-siRNA conjugates for targeted delivery of investigational RNAi therapeutics to tissues outside the liver.
- Received second $500 million payment from Blackstone related to the partial monetization of the inclisiran royalty.
- Announced the planned transition of founding CEO John Maraganore, Ph.D., to Yvonne Greenstreet, MBChB, at year-end 2021. Dr. Maraganore will continue to contribute to Alnylam's success as a member of the Company's Scientific Advisory Board. Effective immediately, Dr. Greenstreet has been appointed to the Alnylam Board of Directors as part of the planned succession.

Upcoming Events

Alnylam announces today the upcoming presentations of clinical data at medical congresses:

- Full results from the ILLUMINATE-C Phase 3 study of lumasiran at the American Society of Nephrology (ASN) Kidney Week 2021, being held November 2-7, 2021 in San Diego, California.
- Additional clinical results from the Phase 1 study of **zilebesiran**, an investigational RNAi therapeutic in development for the treatment of hypertension, at the American Heart

Association (AHA) Scientific Sessions, being held November 13-15, 2021 in Boston, Massachusetts.
- Full 18-month endpoint and safety results from the HELIOS-A Phase 3 study of vutrisiran in early 2022.

In addition, in late 2021, Alnylam intends to:

- Present a review of its pipeline and platform activities at its upcoming R&D Day being held virtually on Friday, November 19, 2021
- Initiate KARDIA-2 Phase 2 combination therapy study of zilebesiran
- Report initial clinical results in healthy volunteers from the Phase 1 study of ALN-HSD at the Company's upcoming R&D Day
- Alnylam's partner Regeneron plans to initiate a Phase 3 study of cemdisiran and pozelimab combination in myasthenia gravis.
- Initiate Phase 2 study of lumasiran in patients with recurrent renal stones
- File CTA for ALN-APP, in development for the treatment of Alzheimer's Disease and CAA
- File CTA for ALN-XDH, in development for the treatment of gout
- File a JNDA in Japan for vutrisiran for the treatment of hATTR amyloidosis with polyneuropathy
- Submit supplemental regulatory filings with the FDA and EMA for lumasiran based on results from the ILLUMINATE-C Phase 3 study in patients with advanced PH1.

Financial Results for the Quarter Ended September 30, 2021

"We are pleased with the increase in patients across our commercial product portfolio in the third quarter of 2021, as our three wholly owned products continue to serve the unmet needs of patients globally. We also further strengthened our balance sheet with receipt of the second $500 million payment from Blackstone related to the partial monetization of the inclisiran royalty," said Jeff Poulton, Chief Financial Officer of Alnylam. "We are reiterating our 2021 financial guidance, which includes our expectation to achieve between $640 million and $665 million in combined net product revenues across our three wholly owned commercial brands for the full year 2021. We look forward to continued strong topline growth balanced with disciplined investment in our operations, which we believe will transition us toward a self-sustainable financial profile, aligned with our *Alnylam P^{5}x25* strategy."

Financial Highlights
(in thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020

ONPATTRO net product revenues	$120,317	$82,516	$336,107	$215,715
GIVLAARI net product revenues	31,833	16,690	87,136	$32,962
OXLUMO net product revenues	14,894	—	40,381	$—
Total net product revenues	$167,044	$99,206	$463,624	$248,677
Net revenue from collaborations	$20,136	$26,647	$121,328	80,614
Royalty revenue	$453	$—	$800	—
GAAP operating loss	$(181,677)	$(225,199)	$(514,091)	$(634,216)
Non-GAAP operating loss	$(138,310)	$(158,522)	$(382,956)	$(498,886)
GAAP net loss	$(204,514)	$(253,291)	$(594,364)	$(614,741)
Non-GAAP net loss	$(179,838)	$(183,597)	$(524,502)	$(546,679)
GAAP net loss per common share - basic and diluted	$(1.72)	$(2.18)	$(5.04)	$(5.37)
Non-GAAP net loss per common share - basic and diluted	$(1.51)	$(1.58)	$(4.44)	$(4.77)

Net Product Revenues

- Net product revenues increased 68% and 86% during the three and nine months ended September 30, 2021, respectively, as compared to the same periods in 2020, primarily due to increased ONPATTRO and GIVLAARI demand in the U.S. and Europe, as well as the ongoing launch of OXLUMO since the first quarter of 2021.

Net Revenues from Collaborations

- Net revenues from collaborations decreased 24% during the three months ended September 30, 2021, as compared to the same period in 2020, primarily due to a decrease in revenue from our collaboration with Vir.
- Net revenues from collaborations increased 51% during the nine months ended September 30, 2021, as compared to the same period in 2020, primarily due to an increase in revenue from our collaboration with Regeneron.

Royalty Revenue

- Royalty revenue is recognized on net global sales of Leqvio by our partner, Novartis.

Third Quarter and Year-to-Date 2021 Expenses

	Three Months Ended September 30,	**Nine Months Ended September 30,**

	2021	2020	2021	2020
GAAP research and development expenses	$ 194,572	$ 161,783	$ 563,106	$ 486,350
Non-GAAP research and development expenses	$ 172,155	$ 148,080	$ 503,228	$ 440,808
GAAP selling, general and administrative expenses	$ 142,075	$ 167,472	$ 434,257	$ 422,129
Non-GAAP selling, general and administrative expenses	$ 121,125	$ 114,498	$ 363,000	$ 332,341

Research & Development (R&D) Expenses

- GAAP and Non-GAAP R&D expenses increased during the three and nine months ended September 30, 2021, as compared to the same periods in 2020, primarily due to continued investment in our early- and late-stage clinical programs.

Selling, General & Administrative (SG&A) Expenses

- GAAP SG&A expenses decreased during the three months ended September 30, 2021, as compared to the same period in 2020, primarily due to a change in an estimate of contingent liabilities related to our arbitration with Ionis Pharmaceuticals, Inc. in 2020.
- GAAP SG&A expenses increased during the nine months ended September 30, 2021, as compared to the same period in 2020, primarily due to increased investment to support the global growth of our three commercialized products and increased stock-based compensation expense primarily due to the accounting for certain performance-based stock awards.
- Non-GAAP SG&A expenses increased during the three and nine months ended September 30, 2021, as compared to the same periods in 2020, primarily due to increased investment to support the global growth of our three commercialized products.

Other Financial Highlights

Other (Expense) Income

- For the three months ended September 30, 2021, interest expense was $40.3 million, which included $30.3 million associated with the sale of future royalties and $10 million associated with the drawdown of our credit facility beginning in December 2020.
- For the nine months ended September 30, 2021, interest expense was $106.2 million, which included $87.3 million associated with the sale of future royalties and $18.9 million associated with the drawdown of our credit facility beginning in December 2020. In addition, we recorded a loss of $19.7 million as a result of a change in fair value of the development derivative liability.

Cash and Investments

- Cash, cash equivalents and marketable securities were $2.33 billion as of September 30, 2021 compared to $1.87 billion as of December 31, 2020 with the increase primarily due to receipt in September 2021 of the second $500 million payment from Blackstone from the partial sale of future inclisiran royalties, $250 million in gross proceeds from the second drawdown on our credit facility and approximately $200 million in proceeds from the exercise of employee equity awards, offset by cash used in our operations to support overall growth.

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

2021 Financial Guidance

Full year 2021 financial guidance is reiterated and consists of the following:

Combined net product revenues for ONPATTRO, GIVLAARI and OXLUMO	$640 million - $665 million
Net revenues from collaborations and royalties	$150 million - $200 million
GAAP R&D and SG&A expenses	$1,335 million - $1,455 million
Non-GAAP R&D and SG&A expenses*	$1,175 million - $1,275 million

*Primarily excludes $160-$180 million of stock-based compensation expenses from estimated GAAP R&D and SG&A expenses.

Use of Non-GAAP Financial Measures

This press release contains non-GAAP financial measures, including expenses adjusted to exclude certain non-cash expenses and non-recurring gains outside the ordinary course of the Company's business. These measures are not in accordance with, or an alternative to, GAAP, and may be different from non-GAAP financial measures used by other companies.

The items included in GAAP presentations but excluded for purposes of determining non-GAAP financial measures for the periods presented in this press release are stock-based compensation expenses, unrealized (gains) losses on marketable equity securities, costs associated with our strategic financing collaboration, upfront payment on license and collaboration agreement, change in estimate of contingent liabilities and loss on contractual settlement. The Company has excluded the impact of stock-based compensation expense, which may fluctuate from period to period based on factors including the variability associated with performance-based grants for stock options and restricted stock units and changes in the Company's stock price, which impacts the fair value of these awards. The Company has excluded the impact of the unrealized (gains) losses on marketable equity securities because the Company does not believe these adjustments accurately reflect the performance of the Company's ongoing operations for the period in which such gains or losses are reported, as

their sole purpose is to adjust amounts on the balance sheet. The Company has excluded the impact of the costs associated with our strategic financing collaboration, upfront payment on license and collaboration agreement, change in estimate of contingent liabilities and loss on contractual settlement 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 2021 results as well as expectations for the future via conference call on Thursday, October 28, 2021 at 8:30 am ET. To access the call, please dial 877-312-7507 (domestic) or +1-631-813-4828 (international) five minutes prior to the start time and refer to conference ID 1428538. 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 855-859-2056 (domestic) or +1-404-537-3406 (international) and refer to conference ID 1428538.

A live audio webcast of the call will be available on the Investors section of the Company's website at www.alnylam.com/events (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Finvestors.alnylam.com%2Fevents&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=www.alnylam.com%2Fevents&index=2&md5=54f77c82f346e27ed97727786c8578a0). 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) and should be administered via a healthcare professional. 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, including please see the full US Prescribing Information (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2Fwp-content%2Fuploads%2Fpdfs%2FONPATTRO-Prescribing-Information.pdf&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=Prescribing+Information&index=3&md5=5a8941fd01fa913ef6b8067cb6a88e7e), visit ONPATTRO.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=http%3A%2F%2Fwww.onpattro.com%2F&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=ONPATTRO.com&index=4&md5=a841472f39f40b7402a3667f8d5a079d).

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, including the full U.S. Prescribing Information (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2FGIVLAARI-Prescribing-Information&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=Prescribing+Information&index=5&md5=b367cc1dd605061feff46cd5e60d59fe). visit GIVLAARI.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=http%3A%2F%2Fwww.givlaari.com%2F&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=GIVLAARI.com&index=6&md5=c09c552f5227c3581a1d3459283bcbae).

About OXLUMO® (lumasiran)

OXLUMO is an RNAi therapeutic targeting hydroxyacid oxidase 1 (HAO1) for the treatment of primary hyperoxaluria type 1 (PH1) to lower urinary oxalate levels in pediatric and adult patients. HAO1 encodes glycolate oxidase (GO), an enzyme upstream of the disease-causing defect in PH1. OXLUMO works by degrading HAO1 messenger RNA and reducing the synthesis

of GO, which inhibits hepatic production of oxalate – the toxic metabolite responsible for the clinical manifestations of PH1. In the pivotal ILLUMINATE-A study, OXLUMO was shown to significantly reduce levels of urinary oxalate relative to placebo, with the majority of patients reaching normal or near-normal levels. Injection site reactions (ISRs) were the most common drug-related adverse reaction. In the ILLUMINATE-B pediatric Phase 3 study, OXLUMO demonstrated an efficacy and safety profile consistent to that observed in ILLUMINATE-A. OXLUMO utilizes Alnylam's Enhanced Stabilization Chemistry (ESC)-GalNAc conjugate technology designed to increase potency and durability. OXLUMO is administered via subcutaneous injection once monthly for three months, then once quarterly thereafter at a dose based on actual body weight. For patients who weigh less than 10 kg, ongoing dosing remains monthly. OXLUMO should be administered by a healthcare professional. For more information about OXLUMO, including the full U.S. Prescribing Information. (https://cts.businesswire.com/ct/CT?id=smartlink&url=http%3A%2F%2Fwww.alnylam.com%2Foxlumo-us-prescribing-information&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=Prescribing+Information.&index=7&md5=010287a7a5d222d650142cab63e64df2) visit OXLUMO.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.oxlumo.com&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=OXLUMO.com&index=8&md5=0c08463566a31c354e682430b9c648a4).

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), GIVLAARI® (givosiran), and OXLUMO® (lumasiran), as well as Leqvio® (inclisiran), which is being developed and commercialized by Alnylam's partner Novartis. Alnylam has a deep pipeline of investigational medicines, including six product candidates that are in late-stage development. Alnylam is executing on its "Alnylam P^5x25" strategy to deliver transformative medicines in both rare and common diseases benefiting patients around the world through sustainable innovation and exceptional financial performance, resulting in a leading biotech profile. Alnylam is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit www.alnylam.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2F&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=www.alnylam.com&index=9&md5=41bfdbc456ad1a1447a549331d2d86df) and engage with us on Twitter at @Alnylam (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Ftwitter.com%2FAlnylam&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=%40Alnylam&index=10&md5=fc17318c765070f89e5ab94ac7ecf1de), on LinkedIn (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.linkedin.com%2Fcompany%2Falnylam-pharmaceuticals%2F&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=LinkedIn&index=11&md5=29e1f72964576cb705da440a97dff45f), or on Instagram (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.instagram.com%2Falnylampharma%2F&esheet=52517231&newsitemid=20211028005385&lan=en-US&anchor=Instagram&index=12&md5=ab286d4f00bb39f9bfbbc078e7902113).

Alnylam Forward Looking Statements

Various statements in this release concerning Alnylam's expectations, plans and prospects, including, without limitation, the CEO leadership transition planned for year end, the potential of the 18-month exploratory and cardiac endpoints data from the HELIOS-A Phase 3 study to represent hypothesis-generating data with respect to the upcoming APOLLO-B and HELIOS-B readouts, its plans for additional global regulatory filings and the continuing product launches of its approved products, the achievement of additional pipeline milestones and data, including relating to ongoing clinical studies of patisiran, vutrisiran, lumasiran, zilebesiran,

fitusiran and ALN-HSD, the initiation of additional clinical studies for zilebesiran, lumasiran and the combination of cemdisiran and pozelimab, the expected timing for filing a CTA for each of ALN-APP and ALN-XDH, a JNDA for vutrisiran for the treatment of hATTR amyloidosis with polyneuropathy and supplemental regulatory filings with the FDA and EMA for lumasiran, continued strong topline revenue growth for its approved products and disciplined investment in its operations, to support the transition toward a self-sustainable financial profile, the expected range of net product revenues and net revenues from collaborations and royalties for 2021, the expected range of aggregate annual GAAP and non-GAAP R&D and SG&A expenses for 2021, and its aspiration to become a leading biotech company, and the planned achievement of its "Alnylam P^5x25" strategy, 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 on Alnylam's business, results of operations and financial condition and the effectiveness or timeliness of Alnylam's efforts to mitigate the impact of the pandemic; the potential impact of the planned leadership transition at year end on Alnylam's ability to attract and retain talent and to successfully execute on its "Alnylam P^5x25" strategy; Alnylam's ability to discover and develop novel drug candidates and delivery approaches and successfully demonstrate the efficacy and safety of its product candidates; the pre-clinical and clinical results for its product candidates; actions or advice of regulatory agencies and Alnylam's ability to obtain and maintain regulatory approval for its product candidates, as well as favorable pricing and reimbursement; successfully launching, marketing and selling its approved products globally; delays, interruptions or failures in the manufacture and supply of its product candidates or its marketed products; obtaining, maintaining and protecting intellectual property; Alnylam's ability to successfully expand the indication for ONPATTRO and vutrisiran in the future; Alnylam's ability to manage its growth and operating expenses through disciplined investment in operations and its ability to achieve a self-sustainable financial profile in the future without the need for future equity financing; Alnylam's ability to maintain strategic business collaborations; Alnylam's dependence on third parties for the development and commercialization of certain products, including Novartis, Regeneron and Vir; the outcome of litigation; the potential impact of a current government investigation and the risk of future 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 its other SEC filings. 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, 2021	September 30, 2020	September 30, 2021	September 30, 2020
Statements of Operations				
Revenues:				
Net product revenues	$ 167,044	$ 99,206	$ 463,624	$ 248,677
Net revenues from collaborations	20,136	26,647	121,328	80,614
Royalty revenue	453	—	800	—
Total revenues	187,633	125,853	585,752	329,291
Operating costs and expenses:				
Cost of goods sold	28,091	20,826	81,370	52,393
Cost of collaborations and royalties	4,572	971	21,110	2,635
Research and development	194,572	161,783	563,106	486,350
Selling, general and administrative	142,075	167,472	434,257	422,129
Total operating costs and expenses	369,310	351,052	1,099,843	963,507
Loss from operations	(181,677)	(225,199)	(514,091)	(634,216)
Other (expense) income:				

Interest expense	(40,274)	(28,731)	(106,205)	(55,979)
Interest income	225	2,072	1,084	10,717
Other (expense) income, net	17,490	(594)	27,370	67,477
Total other (expense) income, net	(22,559)	(27,253)	(77,751)	22,215
Loss before income taxes	(204,236)	(252,452)	(591,842)	(612,001)
Provision for income taxes	(278)	(839)	(2,522)	(2,740)
Net loss	$ (204,514)	$ (253,291)	$ (594,364)	$ (614,741)
Net loss per common share - basic and diluted	$ (1.72)	$ (2.18)	$ (5.04)	$ (5.37)
Weighted-average common shares used to compute basic and diluted	119,141	115,986	118,005	114,554
net loss per common share				

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

	Three Months Ended September 30, 2021	Three Months Ended September 30, 2020	Nine Months Ended September 30, 2021	Nine Months Ended September 30, 2020
Reconciliation of GAAP to Non-GAAP research and development:				
GAAP Research and development	$ 194,572	$ 161,783	$ 563,106	$ 486,350

Less: Stock-based compensation expenses	(12,417)	(13,703)	(49,878)	(45,542)
Less: Upfront payment on license and collaboration agreement	(10,000)	—	(10,000)	—
Non-GAAP Research and development	$ 172,155	$ 148,080	$ 503,228	$ 440,808

Reconciliation of GAAP to Non-GAAP selling, general and administrative:

GAAP Selling, general and administrative	$ 142,075	$ 167,472	$ 434,257	$ 422,129
Less: Stock-based compensation expenses	(20,950)	(23,561)	(71,257)	(60,055)
Less: Costs associated with the strategic financing collaboration	—	(763)	—	(1,083)
Less: Loss on contractual settlement	—	(650)	—	(650)
Less: Change in estimate of contingent liabilities	—	(28,000)	—	(28,000)
Non-GAAP Selling, general and administrative	$ 121,125	$ 114,498	$ 363,000	$ 332,341

Reconciliation of GAAP to Non-GAAP operating loss:

GAAP operating loss	$ (181,677)	$ (225,199)	$ (514,091)	$ (634,216)
Add: Stock-based compensation expenses	33,367	37,264	121,135	105,597
Add: Costs associated with the strategic financing collaboration	—	763	—	1,083

Add: Upfront payment on license and collaboration agreement	10,000	—	10,000	—
Add: Loss on contractual settlement	—	650	—	650
Add: Change in estimate of contingent liabilities	—	28,000	—	28,000
Non-GAAP operating loss	$ (138,310)	$ (158,522)	$ (382,956)	$ (498,886)

Reconciliation of GAAP to Non-GAAP net loss:

GAAP net loss	$ (204,514)	$ (253,291)	$ (594,364)	$ (614,741)
Add: Stock-based compensation expenses	33,367	37,264	121,135	105,597
Add: Costs associated with the strategic financing collaboration	—	763	—	1,083
Add: Upfront payment on license and collaboration agreement	10,000	—	10,000	—
Add: Loss on contractual settlement	—	650	—	8
Add: Change in estimate of contingent liabilities	—	28,000	—	28,000
Less: Unrealized (gain) loss on marketable equity securities	(18,691)	3,017	(61,273)	(66,626)
Non-GAAP net loss	$ (179,838)	$ (183,597)	$ (524,502)	$ (546,679)

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

GAAP net loss per common share - basic and diluted	$ (1.72)	$ (2.18)	$ (5.04)	$ (5.37)

Add: Stock-based compensation expenses	0.28	0.32	1.03	0.91
Add: Costs associated with the strategic financing collaboration	—	0.01	—	0.01
Add: Upfront payment on license and collaboration agreement	0.08	—	0.08	—
Add: Loss on contractual settlement	—	0.01	—	0.01
Add: Change in estimate of contingent liabilities	—	0.24	—	0.24
Less: Unrealized (gain) loss on marketable equity securities	(0.16)	0.02	(0.52)	(0.57)
Non-GAAP net loss per common share - basic and diluted	$ (1.51)	$ (1.58)	$ (4.44)	(4.77)

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, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 2,327,865	$ 1,874,395
Restricted investments	40,890	40,725
Accounts receivable, net	141,064	102,413
Inventory	110,949	92,302
Prepaid expenses and other assets	130,751	90,712
Property, plant and equipment, net	484,941	465,029
Operating lease right-of-use lease assets	235,853	241,485
Receivable related to the sale of future royalties	—	500,000
Total assets	$ 3,472,313	$ 3,407,061
Accounts payable, accrued expenses and other liabilities	$ 505,074	$ 445,783
Total deferred revenue	292,167	352,301

Operating lease liability	326,642	329,911
Liability related to the sale of future royalties	1,158,738	1,071,541
Long-term debt	433,799	191,278
Total stockholders' equity (119.5 million shares issued and outstanding at September 30, 2021; 116.4 million shares issued and outstanding at December 31, 2020)	755,893	1,016,247
Total liabilities and stockholders' equity	$ 3,472,313	$ 3,407,061

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, 2020.

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