Alnylam Pharmaceuticals Reports Third Quarter 2023 Financial Results and Highlights Recent Period Activity

Nov 02, 2023

– Achieved Third Quarter 2023 Global Net Product Revenues of $313 Million, Representing 35% Year-Over-Year Growth Compared to Q3 2022 –

– Received Complete Response Letter from U.S. FDA for Supplemental New Drug Application for Patisiran for the Treatment of the Cardiomyopathy of ATTR Amyloidosis –

– Company Remains On Track to Report Topline Results from HELIOS-B Phase 3 Study in Early 2024 –

– Reported Positive Topline Results from KARDIA-1 Phase 2 Study of Zilebesiran –

– Updated 2023 Guidance for Revenues from Collaborations and Royalties to $575 Million to $625 Million; Reiterated 2023 Guidance for Combined Net Product Revenues of $1,200 Million to $1,285 Million –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 2, 2023--

Alnylam Pharmaceuticals, Inc. (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Alnylam+Pharmaceuticals%2C+Inc.&index=1&md5=b5da0ed07b8e2f1c7e4d1b594ac10131) (Nasdaq: ALNY), the leading RNAi therapeutics company, today reported its consolidated financial results for the third quarter ended September 30, 2023 and reviewed recent business highlights.

“We continued to deliver strong commercial results in the third quarter, with the successful launch of AMVUTTRA contributing to 35% year-over-year growth in net product revenues,” said Yvonne Greenstreet, MBChB, Chief Executive Officer of Alnylam. “While we were disappointed by the recent Complete Response Letter for ONPATTRO for the treatment of the cardiomyopathy of ATTR amyloidosis, we remain confident in the HELIOS-B Phase 3 study of vutrisiran, which is on track to deliver topline results in early 2024. We’re also excited about advancements across our broad pipeline of RNAi therapeutics, including recent positive topline results from the KARDIA-1 Phase 2 study of zilebesiran, as well as upcoming Phase 1 results for ALN-TTRsc04 and ALN-KHK by year-end. With this continued execution we remain on track to meet our *Alnylam P^{5}x25* goals of becoming a top-tier biotech company delivering sustained innovation and exceptional financial results.”

Third Quarter 2023 and Recent Significant Corporate Highlights

Commercial Performance

Total TTR: ONPATTRO® (patisiran) & AMVUTTRA® (vutrisiran)

- Achieved global net product revenues for ONPATTRO and AMVUTTRA for the third quarter of $82 million and $149 million, respectively, representing 35% total TTR reported year-over-year growth compared to Q3 2022.
- Attained over 3,790 hATTR amyloidosis patients with polyneuropathy worldwide on commercial treatment with ONPATTRO or AMVUTTRA as of September 30, 2023.

Total Ultra-Rare: GIVLAARI® (givosiran) & OXLUMO® (lumasiran)

- Achieved global net product revenues for GIVLAARI and OXLUMO for the third quarter of $54 million and $29 million, respectively, representing 33% total Ultra-Rare reported year-over-year growth compared to Q3 2022.

- Attained over 625 patients worldwide on commercial GIVLAARI treatment as of September 30, 2023.
- Attained over 375 patients worldwide on commercial OXLUMO treatment as of September 30, 2023.

Leqvio® (inclisiran)

- Leqvio launch in the U.S. and other markets is ongoing, with focus on patient on-boarding, removing access hurdles and enhancing medical education. In the U.S., Leqvio is covered at or near label for 76% of patients. More than 55% of Leqvio source of business in the U.S. is now through "Buy and Bill" acquisition mode. FDA expanded the label to include primary hyperlipidemia (patients at increased risk of ASCVD) and the removal of four adverse reactions from the safety section as well as Limitations of Use. In Q3 2023, Leqvio was approved in China and in Japan and is now approved in 93 countries.

R&D Highlights

Reported positive topline results from the KARDIA-1 Phase 2 dose-ranging study of **zilebesiran**, an investigational RNAi therapeutic in development to treat hypertension in patients at high cardiovascular risk.

Published results from Phase 1 study of zilebesiran in the *New England Journal of Medicine*.

Reported updated positive interim results for the ongoing single ascending dose portion of the Phase 1 study of **ALN-APP** in patients with early-onset Alzheimer's disease at the 2023 Alzheimer's Association International Conference and at the 16th Clinical Trials on Alzheimer's Disease conference.

- Based on the achievement of specified clinical proof-of-principle criteria for the Phase 1 ALN-APP program, Alnylam received a $100 million milestone payment from Regeneron.

Presented new 24-month results from an interim analysis of the open-label extension period of the APOLLO-B Phase 3 study of **patisiran** in patients with the cardiomyopathy of transthyretin-mediated amyloidosis at the Heart Failure Society of America Annual Scientific Meeting 2023.

Published results from Phase 3 APOLLO-B study of patisiran in the *New England Journal of Medicine*.

Sanofi reported positive data for **fitusiran**, in development for the treatment of hemophilia A or B, with or without inhibitors, from the Phase 3 open-label extension study (ATLAS-OLE).

- Sanofi is currently in discussions with the FDA regarding filing in 2024.

Additional Business Updates

- Entered into a global strategic collaboration with Roche for the co-development and co-commercialization of **zilebesiran**.
- Recognized by *Science* magazine as a Top Employer for the fifth consecutive year.

Upcoming Events

Alnylam announces today that it will present a review of its RNAi platform and pipeline progress at a virtual R&D Day on December 13, 2023.

Alnylam announces today that results from the KARDIA-1 Phase 2 dose-ranging study of **zilebesiran** will be presented as a Late-Breaker at the American Heart Association Scientific Sessions 2023 on November 11, 2023, in Philadelphia, Pennsylvania.

In addition, in late 2023:

- Alnylam intends to report topline results from the Phase 1 study of **ALN-TTRsc04**.
- Alnylam intends to report topline results from the Phase 1 study of **ALN-KHK**.
- Vir is conducting multiple trials evaluating the potential for **ALN-HBV02** (VIR-2218) and VIR-3434 to achieve a functional cure for chronic hepatitis B. Phase 2 data readouts are on track for Q4 2023.
    - Vir also announced that initial Phase 2 data readouts for the SOLSTICE trial evaluating **ALN-HBV02** (VIR-2218) and VIR-3434 as monotherapy and in combination for the treatment of people living with chronic hepatitis delta, the most aggressive form of viral hepatitis, are expected in Q4 2023.

Financial Results for the Quarter Ended September 30, 2023

	Three Months Ended September 30,	
(in thousands, except per share amounts)	**2023**	**2022**
Net product revenues	$ 313,153	$ 232,267
Net revenue from collaborations	$ 427,472	$ 29,297
Royalty revenue	$ 9,905	$ 2,742
GAAP Operating income (loss)	$ 213,867	$ (258,040)
Non-GAAP Operating income (loss)	$ 277,804	$ (129,922)
GAAP Net income (loss)	$ 147,753	$ (405,920)
Non-GAAP Net income (loss)	$ 228,534	$ (193,366)
GAAP net income (loss) per common share - basic	$ 1.18	$ (3.32)
Non-GAAP net income (loss) per common share - basic	$ 1.83	$ (1.58)

GAAP net income (loss) per common share - diluted	$ 1.15	$ (3.32)
Non-GAAP net income (loss) per common share - diluted	$ 1.74	$ (1.58)

* For an explanation of our use of non-GAAP financial measures refer to the "Use of Non-GAAP Financial Measures" section later in this press release and for a reconciliation of each non-GAAP financial measure to the most comparable GAAP measures, see the table at the end of this press release.

Net Product Revenues

	Three Months Ended September 30,		Year over Year % Growth	
(in thousands, except percentages)	2023	2022	As Reported	At CER*
ONPATTRO net product revenues	$ 81,589	$ 144,950	(44)%	(46)%
AMVUTTRA net product revenues	148,680	25,229	489 %	488 %
Total TTR net product revenues	$ 230,269	$ 170,179	35 %	34 %
GIVLAARI net product revenues	54,148	45,659	19 %	17 %
OXLUMO net product revenues	28,736	16,429	75 %	69 %
Total net product revenues	$ 313,153	$ 232,267	35 %	33 %

* CER = Constant Exchange Rate, representing growth calculated as if the exchange rates had remained unchanged from those used in the third quarter 2022. CER is a Non-GAAP measure. For an explanation of our use of non-GAAP financial measures refer to the "Use of Non-GAAP Financial Measures" section later in this press release and for a reconciliation of each non-GAAP financial measure to the most comparable GAAP measures, see the table at the end of this press release.

- Net product revenues increased 35% during the third quarter 2023, as compared to the prior year, and 33% at CER. The increase is primarily related to growth in our TTR product revenues driven by the launch of AMVUTTRA in the third quarter of 2022 as well as increased patients on GIVLAARI and OXLUMO therapies. The increase was offset by a decrease of demand for ONPATTRO due to patient switched to AMVUTTRA.

Net Revenues from Collaborations

- The increase in net revenues from collaborations, as compared to the prior year, is due to revenue related to our collaborations with Roche and Regeneron. During the quarter, we recognized $310 million of revenue from the upfront payment received from Roche and $65 million in connection with our Regeneron Collaboration. The $65 million of revenue is a cumulative adjustment from the $100 million milestone received for achieving certain criteria during early clinical development for our CNS program, ALN-APP.

Operating Expenses

	Three Months Ended September 30,		**2023 vs 2022**
(in thousands, except percentages)	**2023**	**2022**	**% Change***
Cost of goods sold	$ 79,473	$ 36,507	118 %
Cost of goods sold as a percentage of net product revenues	*25.4 %*	*15.7 %*	*9.7 %*
Cost of collaborations	$ 4,836	$ 4,609	5 %
GAAP research and development expenses	$ 253,179	$ 245,371	3 %
Non-GAAP research and development expenses	$ 224,024	$ 192,409	16 %
GAAP selling, general and administrative expenses	$ 199,175	$ 235,859	(16)%
Non-GAAP selling, general and administrative expenses	$ 164,393	$ 160,703	2 %

*For dollar values, we calculate the percentage of change during Q3 2023 compared to Q3 2022. For cost of goods sold as a percentage of net product revenues, we calculate the basis point change during Q3 2023 compared to Q3 2022.

Cost of Goods Sold

- Cost of goods sold as a percent of net product revenues increased during the third quarter 2023, as compared to the prior year, primarily due to recording an impairment of ONPATTRO inventory that had been manufactured for future demand associated with the ATTR cardiomyopathy indication for patisiran for which we did not receive regulatory approval.

Research & Development (R&D) Expenses

- GAAP and non-GAAP R&D expenses increased during the third quarter 2023, as compared to the prior year, primarily due to increased costs related to clinical activities and increased

headcount to support our R&D pipeline and an expense for the achievement of a certain clinical milestone payable to a partner. Increased GAAP R&D expenses were offset by decreased stock-based compensation expense related to the accounting for certain performance-based awards that vested in 2022.

Selling, General & Administrative (SG&A) Expenses

- GAAP SG&A expenses decreased during the third quarter 2023, as compared to the prior year, primarily due to decreased stock-based compensation expense related to the accounting for certain performance-based awards that vested in 2022. Non-GAAP SG&A expenses increased in the third quarter of 2023, compared to 2022, primarily due to increased headcount and other investments supporting our strategic growth including the global launch of AMVUTTRA.

Other Financial Highlights

- Cash, cash equivalents and marketable securities were $2.41 billion as of September 30, 2023 compared to $2.19 billion as of December 31, 2022 with the increase primarily related to the $310 million upfront payment received from Roche in connection with our partnership to co-develop and co-commercialize zilebesiran, offset by our operating loss year-to-date.

The adjustments to the non-GAAP measures provided in the financial results above and in the financial guidance below are described under “Use of Non-GAAP Financial Measures” later in this press release. A reconciliation of our GAAP to non-GAAP results presented in this release is included in the tables at the end of this press release.

2023 Financial Guidance

Full year 2023 financial guidance has been updated as follows:

	Provided 2/23/2023[1]	**Updated 11/2/2023**
Combined net product revenues for ONPATTRO, AMVUTTRA, GIVLAARI and OXLUMO[1]	$1,200 million - $1,285 million	Unchanged
Net Product Revenue Growth vs. 2022 at reported FX rates[1]	34% to 44%	Unchanged
*Net Product Revenue Growth vs. 2022 at CER**	34% to 44%	Unchanged
Net revenues from collaborations and royalties	$100 million - $175 million	$575 million - $625 million
GAAP R&D and SG&A expenses	$1,790 million - $1,885 million	Unchanged
Non-GAAP R&D and SG&A expenses[2]	$1,575 million - $1,650 million	Unchanged

[1] Uses December 31, 2022 FX rates including: 1 EUR = 1.07 USD and 1 USD = 131 JPY

[2] Excludes $215 - $235 million of stock-based compensation expense from estimated GAAP R&D and SG&A expenses

*CER = Constant Exchange Rate, representing growth calculated as if the exchange rates had remained unchanged from those used in the twelve months ended December 31, 2022. CER is a Non-GAAP measure.

Use of Non-GAAP Financial Measures

This press release contains non-GAAP financial measures, including expenses adjusted to exclude certain non-cash expenses and certain losses 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 non-GAAP financial measures we present include Non-GAAP Operating income (loss), Non-GAAP Net income (loss), Non-GAAP Net income (loss) per common share - basic, Non-GAAP Net income (loss) per common share - diluted and Non-GAAP R&D and SG&A expenses. 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 and realized and unrealized losses on marketable equity securities. 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 realized and unrealized 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.

Percentage changes in revenue growth at CER, also a non-GAAP financial measure, are presented excluding the impact of changes in foreign currency exchange rates for investors to understand the underlying business performance. The current period's foreign currency revenue values are converted into U.S. dollars using the average exchange rates from the prior period.

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 historical GAAP and non-GAAP measures presented in this release is provided later in this press release.

Conference Call Information

Management will provide an update on the Company and discuss third quarter 2023 results as well as expectations for the future via conference call on Thursday, November 2, 2023 at 8:30 am ET. To access the call, please register online at https://register.vevent.com/register/BI29582eb5fd274b758554baecef8f03fa (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fregister.vevent.com%2Fregister%2FBI29582eb5fd274b758554baecef8f03fa&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=https%3A%2F%2Fregister.vevent.com%2Fregister%2FBI29582eb5fd274b758554baecef8f03fa&index=2&md5=adc0d9ca6271197d11b2feac41da8efe). Participants are requested to register at a minimum 15 minutes before the start of the call. A replay of the call will be available two hours after the call and archived on the same web page for six months.

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=53737944&newsitemid=20231102700234&lan=en-US&anchor=www.alnylam.com%2Fevents&index=3&md5=aef72691c3d944c51e1c7f22e6a1ab43). 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 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, 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 reducing the production of TTR protein before it is made. Reducing the pathogenic protein leads to a reduction in amyloid deposits in tissues. For more information about ONPATTRO, including full Prescribing Information (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2Fsites%2Fdefault%2Ffiles%2Fpdfs%2FONPATTRO-Prescribing-Information.pdf&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Prescribing+Information&index=4&md5=36bdc65718f4eb99e934033205834d9a), visit ONPATTRO.com (https://cts.businesswire.com/ct/CT?

id=smartlink&url=https%3A%2F%2Fwww.onpattro.com%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=ONPATTRO.com&index=5&md5=9b5db099af3afa89e9f493cc90938f37).

About AMVUTTRA® (vutrisiran)

AMVUTTRA® (vutrisiran) is an RNAi therapeutic approved in the United States for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. It is a double-stranded small interfering RNA (siRNA) that targets mutant and wild-type transthyretin (TTR) messenger RNA (mRNA). Using Alnylam's Enhanced Stabilization Chemistry (ESC)-GalNAc-conjugate delivery platform, AMVUTTRA is designed for increased potency and high metabolic stability to allow for subcutaneous injection once every three months (quarterly). Results from the pivotal HELIOS-A Phase 3 study demonstrate AMVUTTRA rapidly reduces serum TTR levels, has the potential to reverse neuropathy impairment relative to baseline and improves other key measures of disease burden relative to external placebo in patients with the polyneuropathy of hATTR amyloidosis. For more information about AMVUTTRA, including the full U.S. Prescribing Information (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.alnylam.com%2Fsites%2Fdefault%2Ffiles%2Fpdfs%2Famvuttra-us-prescribing-information.pdf&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Prescribing+Information&index=6&md5=51689a87d18e059e7020dfc0d96984b6), visit AMVUTTRA.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.amvuttra.com&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=AMVUTTRA.com&index=7&md5=7b6128811c732c2cfa47fa1b683da3d4).

About GIVLAARI® (givosiran)

GIVLAARI (givosiran) 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 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=53737944&newsitemid=20231102700234&lan=en-

US&anchor=Prescribing+Information&index=8&md5=d2ff79403625eb3dc3e9d2ebaaf580cf), visit GIVLAARI.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=http%3A%2F%2Fwww.givlaari.com%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=GIVLAARI.com&index=9&md5=68f69664657291b7b6a7c20d478e0379).

About OXLUMO® (lumasiran)

OXLUMO (lumasiran) is an RNAi therapeutic targeting hydroxyacid oxidase 1 (HAO1). HAO1 encodes glycolate oxidase (GO). Thus, by silencing HAO1 and depleting the GO enzyme, OXLUMO inhibits production of oxalate – the metabolite that directly contributes to the pathophysiology of PH1. OXLUMO utilizes Alnylam's Enhanced Stabilization Chemistry (ESC)-GalNAc-conjugate technology, which enables subcutaneous dosing with increased potency and durability and a wide therapeutic index. OXLUMO has received regulatory approvals from the U.S. Food and Drug Administration (FDA) for the treatment of primary hyperoxaluria type 1 (PH1) to lower urinary and plasma oxalate levels in pediatric and adult patients and from the European Medicines Agency (EMA) for the treatment of PH1 in all age groups. 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. In the ILLUMINATE-B pediatric Phase 3 study, OXLUMO demonstrated an efficacy and safety profile consistent to that observed in ILLUMINATE-A. In the ILLUMINATE-C study, OXLUMO resulted in substantial reductions in plasma oxalate in patients with advanced PH1. Across all three studies, injection site reactions (ISRs) were the most common drug-related adverse reaction. OXLUMO is administered via subcutaneous injection once monthly for three months, then once quarterly beginning one month after the last loading dose 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=https%3A%2F%2Fwww.alnylam.com%2Fsites%2Fdefault%2Ffiles%2Fpdfs%2FOXLUMO-Prescribing-Information.pdf&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Prescribing+Information&index=10&md5=dd880beed5d4340f88943f98dbe21e09), visit OXLUMO.com (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.oxlumo.com%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=OXLUMO.com&index=11&md5=460033b8c3b3d4129348eba98d6220bf).

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 Pharmaceuticals (Nasdaq: ALNY) has led 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 and prevalent diseases with unmet need. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach yielding transformative medicines. Since its founding in 2002, Alnylam has led the RNAi Revolution and continues to deliver on a bold vision to turn scientific possibility into reality. Alnylam’s commercial RNAi therapeutic products are ONPATTRO® (patisiran), AMVUTTRA® (vutrisiran), GIVLAARI® (givosiran), OXLUMO® (lumasiran) and Leqvio® (inclisiran), which is being developed and commercialized by Alnylam’s partner, Novartis. Alnylam has a deep pipeline of investigational medicines, including multiple product candidates that are in late-stage development. Alnylam is executing on its “*Alnylam P^{5}x25*” 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=53737944&newsitemid=20231102700234&lan=en-US&anchor=www.alnylam.com&index=12&md5=925eeb8ac14b30cb92ef7601109c7278) and engage with us on X (formerly Twitter) at @Alnylam (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Ftwitter.com%2FAlnylam&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=%40Alnylam&index=13&md5=8c479af8b8c2d8d002eaf856ce143b97), or on LinkedIn (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.linkedin.com%2Fcompany%2Falnylam-pharmaceuticals%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=LinkedIn&index=14&md5=fb81f9eade737a52e9295273f430c24b), Facebook (https://cts.businesswire.com/ct/CT?

id=smartlink&url=https%3A%2F%2Fwww.facebook.com%2FAlnylamPharma%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Facebook&index=15&md5=e33b928a0863d33426dd204170473700) on Instagram (https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.instagram.com%2Falnylampharma%2F&esheet=53737944&newsitemid=20231102700234&lan=en-US&anchor=Instagram&index=16&md5=85c6d3ff0f509804a7de9a430bb489c5).

Alnylam Forward Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. All statements other than historical statements of fact regarding Alnylam's expectations, beliefs, goals, plans or prospects including, without limitation, expectations regarding Alnylam's aspiration to become a leading biotech company and the planned achievement of its "*Alnylam P^{5}x25*" strategy, the potential for Alnylam to identify new potential drug development candidates and advance its research and development programs, Alnylam's ability to obtain approval for new commercial products or additional indications for its existing products, and Alnylam's projected commercial and financial performance, should be considered forward-looking statements. 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; Alnylam's ability to successfully execute on its "*Alnylam P^{5}x25*" 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 Alnylam's product candidates, including vutrisiran; actions or advice of regulatory agencies and Alnylam's ability to obtain and maintain regulatory approval for its product candidates, including vutrisiran, as well as favorable pricing and reimbursement; successfully launching, marketing and selling Alnylam's approved products globally; delays, interruptions or failures in the manufacture and supply of Alnylam's product candidates or its marketed products; delays or interruptions in the supply of resources needed to advance Alnylam's research and development programs, including as may arise from recent disruptions in the supply of non-human primates; obtaining, maintaining and protecting intellectual property; Alnylam's ability to successfully expand the indication AMVUTTRA 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 Roche, Novartis, Sanofi, Regeneron and Vir; the outcome of litigation; the risks of future government investigations; and unexpected expenditures; as well as those risks more fully discussed in the "Risk Factors" filed with

Alnylam's 2022 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as may be updated from time to time in Alnylam's subsequent Quarterly Reports on Form 10-Q 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. Patisiran has not been approved by any regulatory agency for the treatment of ATTR amyloidosis with cardiomyopathy. No conclusions can or should be drawn regarding its safety or effectiveness in treating cardiomyopathy in this population. 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, 2023	September 30, 2022	September 30, 2023	September 30, 2022
Statements of Operations				
Revenues:				
Net product revenues	$ 313,153	$ 232,267	$ 895,186	$ 632,654
Net revenues from collaborations	427,472	29,297	469,778	64,267
Royalty revenue	9,905	2,742	23,610	5,462
Total revenues	750,530	264,306	1,388,574	702,383
Operating costs and expenses:				
Cost of goods sold	79,473	36,507	196,241	94,002

Cost of collaborations and royalties	4,836	4,609	28,307	23,549
Research and development	253,179	245,371	732,274	620,976
Selling, general and administrative	199,175	235,859	597,523	560,314
Total operating costs and expenses	536,663	522,346	1,554,345	1,298,841
Income (loss) from operations	213,867	(258,040)	(165,771)	(596,458)
Other (expense) income:				
Interest expense	(30,893)	(41,084)	(89,883)	(126,055)
Interest income	25,425	7,820	65,155	10,731
Other expense, net	(57,658)	(38,053)	(105,331)	(131,604)
Loss on the extinguishment of debt	—	(76,586)	—	(76,586)
Total other expense, net	(63,126)	(147,903)	(130,059)	(323,514)
Income (loss) before income taxes	150,741	(405,943)	(295,830)	(919,972)
(Provision for) benefit from income taxes	(2,988)	23	(6,542)	(3,691)
Net income (loss)	$ 147,753	$ (405,920)	$ (302,372)	$ (923,663)
Net income (loss) per common share - basic	$ 1.18	$ (3.32)	$ (2.43)	$ (7.62)
Net income (loss) per common share - diluted	$ 1.15	$ (3.32)	$ (2.43)	$ (7.62)
Weighted-average common shares-basic	125,220	122,166	124,667	121,158
Weighted-average common shares-diluted	131,337	122,166	124,667	121,158

ALNYLAM PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands, except per share amounts)

(Unaudited)

	September 30, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 1,033,024	$ 866,394
Marketable debt securities	1,362,843	1,297,890
Marketable equity securities	10,411	28,122
Accounts receivable, net	325,445	237,963
Inventory	95,771	128,962
Prepaid expenses and other current assets	157,958	132,916
Total current assets	2,985,452	2,692,247
Property, plant and equipment, net	525,591	523,494
Operating lease right-of-use assets	203,485	215,136
Restricted investments	49,390	49,390
Other assets	75,155	66,092
Total assets	$ 3,839,073	$ 3,546,359
LIABILITIES AND STOCKHOLDERS' DEFICIT		
Current liabilities:		
Accounts payable	$ 73,840	$ 98,094
Accrued expenses	713,094	545,460
Operating lease liability	41,516	41,967
Deferred revenue	77,140	42,105
Liability related to the sale of future royalties	44,195	40,289
Total current liabilities	949,785	767,915
Operating lease liability, net of current portion	247,711	261,339
Deferred revenue, net of current portion	196,086	193,791
Convertible debt	1,019,809	1,016,942
Liability related to the sale of future royalties, net of current portion	1,310,814	1,252,015
Other liabilities	280,734	212,580
Total liabilities	4,004,939	3,704,582

Commitments and contingencies (Note 13)		
Stockholders' deficit:		
Preferred stock, $0.01 par value per share, 5,000 shares authorized and no shares issued and outstanding as of September 30, 2023 and December 31, 2022	—	—
Common stock, $0.01 par value per share, 250,000 shares authorized; 125,454 shares issued and outstanding as of September 30, 2023; 123,925 shares issued and outstanding as of December 31, 2022	1,255	1,240
Additional paid-in capital	6,736,939	6,454,540
Accumulated other comprehensive loss	(32,339)	(44,654)
Accumulated deficit	(6,871,721)	(6,569,349)
Total stockholders' deficit	(165,866)	(158,223)
Total liabilities and stockholders' deficit	$ 3,839,073	$ 3,546,359

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, 2022.

ALNYLAM PHARMACEUTICALS, INC.

RECONCILIATION OF SELECTED GAAP MEASURES TO NON-GAAP MEASURES

(In thousands, except per share amounts)

(Unaudited)

	Three Months Ended September 30, 2023	Three Months Ended September 30, 2022	Nine Months Ended September 30, 2023	Nine Months Ended September 30, 2022
Reconciliation of GAAP to Non-GAAP research and development:				
GAAP research and development	$ 253,179	$ 245,371	$ 732,274	$ 620,976
Less: Stock-based compensation expenses	(29,155)	(52,962)	(78,188)	(75,217)

Non-GAAP research and development	$ 224,024	$ 192,409	$ 654,086	$ 545,759

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

GAAP selling, general and administrative	$ 199,175	$ 235,859	$ 597,523	$ 560,314
Less: Stock-based compensation expenses	(34,782)	(75,156)	(101,498)	(112,665)
Non-GAAP selling, general and administrative	$ 164,393	$ 160,703	$ 496,025	$ 447,649

Reconciliation of GAAP to Non-GAAP operating income (loss):

GAAP operating income (loss)	$ 213,867	$ (258,040)	$ (165,771)	$ (596,458)
Add: Stock-based compensation expenses	63,937	128,118	179,686	187,882
Non-GAAP operating income (loss)	$ 277,804	$ (129,922)	$ 13,915	$ (408,576)

Reconciliation of GAAP to Non-GAAP net income (loss):

GAAP net income (loss)	$ 147,753	$ (405,920)	$ (302,372)	$ (923,663)
Add: Stock-based compensation expenses	63,937	128,118	179,686	187,882
Add: Realized and unrealized loss on marketable equity securities	16,844	7,850	17,711	40,108
Add: Loss on the extinguishment of debt	—	76,586	—	76,586

Non-GAAP net income (loss)	$ 228,534	$ (193,366)	$ (104,975)	$ (619,087)

Reconciliation of GAAP to Non-GAAP net income (loss) per common share- basic:

GAAP net income (loss) per common share - basic	$ 1.18	$ (3.32)	$ (2.43)	$ (7.62)
Add: Stock-based compensation expenses	0.51	1.05	1.44	1.55
Add: Realized and unrealized loss on marketable equity securities	0.13	0.06	0.14	0.33
Add: Loss on the extinguishment of debt	—	0.63	—	0.63
Non-GAAP net income (loss) per common share - basic	$ 1.83	$ (1.58)	$ (0.84)	$ (5.11)

Reconciliation of GAAP to Non-GAAP net income (loss) per common share- diluted:

GAAP net income (loss) per common share - diluted	$ 1.15	$ (3.32)	$ (2.43)	$ (7.62)
Add: Stock-based compensation expenses	0.49	1.05	1.44	1.55
Add: Realized and unrealized loss on marketable equity securities	0.13	0.06	0.14	0.33
Add: Loss on the extinguishment of debt	—	0.63	—	0.63
Non-GAAP net income (loss) per common share - diluted	$ 1.74	$ (1.58)	$ (0.84)	$ (5.11)

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

ALNYLAM PHARMACEUTICALS, INC.

RECONCILIATION OF GAAP TO NON-GAAP

PRODUCT REVENUE GROWTH AT CONSTANT CURRENCY

(Unaudited)

	September 30, 2023	
	Three Months Ended	Nine Months Ended
ONPATTRO net product revenue growth, as reported	(44)%	(37)%
Add: Impact of foreign currency translation	(2)	—
ONPATTRO net product revenue growth at constant currency	(46)%	(37)%
AMVUTTRA net product revenue growth, as reported	489%	1416%
Add: Impact of foreign currency translation	(1)	18
AMVUTTRA net product revenue growth at constant currency	488%	1434%
Total TTR net product revenue growth, as reported	35%	43%
Add: Impact of foreign currency translation	(1)	1
Total TTR net product revenue growth at constant currency	34%	44%
GIVLAARI net product revenue growth, as reported	19%	27%
Add: Impact of foreign currency translation	(2)	—
GIVLAARI net product revenue growth at constant currency	17%	27%
OXLUMO net product revenue growth, as reported	75%	68%
Add: Impact of foreign currency translation	(6)	(1)
OXLUMO net product revenue growth at constant currency	69%	67%

Total net product revenue growth, as reported	35	%	41	%
Add: Impact of foreign currency translation	(2	)	1	
Total net product revenue growth at constant currency	33	%	42	%

View source version on businesswire.com (http://businesswire.com): https://www.businesswire.com/news/home/20231102700234/en/ (https://www.businesswire.com/news/home/20231102700234/en/)

Alnylam Pharmaceuticals, Inc.
Christine Regan Lindenboom
(Investors and Media)
617-682-4340

Josh Brodsky
(Investors)
617-551-8276

Source: Alnylam Pharmaceuticals, Inc.

VIEW ALL PRESS RELEASES

(/press-releases)