

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 7, 2023

RHYTHM PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-38223
(Commission
File Number)

46-2159271
(IRS Employer
Identification Number)

222 Berkeley Street
12th Floor
Boston, MA 02116

(Address of principal executive offices) (Zip Code)

Registrant's telephone number, including area code: **(857) 264-4280**

N/A

(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:

- 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, \$0.001 par value per share	RYTM	The Nasdaq Stock Market LLC (Nasdaq Global Market)

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 7, 2023, Rhythm Pharmaceuticals, Inc. (the “Company”) announced its financial results for the quarter ended September 30, 2023. The full text of the press release issued by the Company in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information contained in Item 2.02 of this Current Report on Form 8-K (including Exhibit 99.1 attached hereto) 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, or the Exchange Act, except as expressly provided by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

The following Exhibit 99.1 relates to Item 2.02, and shall be deemed to be furnished, and not filed:

Exhibit No.	Description
99.1	Press release dated November 7, 2023
104	Cover Page Interactive Data File (embedded within the inline XBRL document)

SIGNATURES

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

RHYTHM PHARMACEUTICALS, INC.

Date: November 7, 2023

By: /s/ Hunter Smith
Hunter Smith
Chief Financial Officer



**Rhythm Pharmaceuticals Reports Third Quarter 2023 Financial Results
and Business Update**

-- Third quarter 2023 net revenue from global sales of IMCIVREE[®] (setmelanotide) of \$22.5 million --

-- More than 100 international patients on reimbursed IMCIVREE therapy --

-- Phase 3 hypothalamic obesity trial on track to be fully enrolled by the end of 2023 --

-- 25.5% mean BMI reduction achieved at one year of setmelanotide therapy in patients with hypothalamic obesity (n=12) who transitioned to long term extension from Ph 2 trial; three of 11 pediatric patients achieving normal body weight at one year on setmelanotide treatment --

-- Cash on-hand of \$299.3 million sufficient to fund planned operations into 2026 --

-- Company to provide update on R&D programs for investors and analysts at an event planned for Dec. 6 --

-- Management to host conference call today at 8:00 a.m. ET --

BOSTON, November 7, 2023 – Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a global commercial-stage biopharmaceutical company focused on transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases, today reported financial results and provided a business update for the third quarter ended September 30, 2023.

“We have achieved remarkable progress this year in expanding access to IMCIVREE[®] (setmelanotide) and delivering it to patients on a global level,” said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. “We remain pleased by continued strong performance more than one year into our U.S. commercial launch in Bardet-Biedl syndrome (BBS). Outside the U.S., we continue to expand access with pre-approval, reimbursed early-access for hypothalamic obesity in France and a positive recommendation for public reimbursement in Canada for patients with BBS.”

“In addition, we are excited by the strength of our 12-month LTE data in patients with hypothalamic obesity where we reported a mean body mass index (BMI) reduction of more than 25% in patients on therapy for one year, with several patients trending towards or achieving normal body weight. These data and continued enrollment progress with our ongoing Phase 3 study reinforce our confidence as we advance this high potential program. We look forward to providing an R&D update, including our RM-718 program, data from the open label part of the setmelanotide Phase 2 DAYBREAK study and data from our Phase 3 pediatrics trial, during an investor event in December.”

Third Quarter and Recent Business Highlights

Commercial Updates

- Today, Rhythm announced that more than 120 new prescriptions for IMCIVREE for BBS have been written by U.S. prescribers and that the Company has received payor approval for reimbursement for 80 prescriptions during the third quarter of 2023.
- Today, Rhythm also announced that more than 100 international patients across 11 countries cumulatively have initiated reimbursed IMCIVREE therapy, as of October 27, 2023.
- In November, Rhythm announced that the Canadian Agency for Drugs and Technologies in Health (CADTH) recommended IMCIVREE[®] (setmelanotide) be reimbursed by CADTH-participating public drug plans for weight management in adult and pediatric patients 6 years of age and older with obesity due to BBS.
- In September 2023, the Company announced that a new International Classification Diseases, Tenth Revision (ICD-10) diagnosis code for BBS was approved by the Centers for Disease Control and Prevention (CDC), and effective October 1, 2023. This BBS-specific code may improve patient identification and yield data to improve understanding of disease progression, and diagnostic and treatment journey.
- In August 2023, the Company announced that the French National Agency for Medicines and Health Products Safety (ANSM) and French National Authority for Health (HAS) granted pre-marketing early access authorization AP1 (Autorisation d'Accès Précoce), for IMCIVREE[®] (setmelanotide) for patients with lesional hypothalamic obesity. Products included in the AP1 programs are fully covered by France's National Health System and Rhythm can expect to be reimbursed for any patients receiving treatments through this program.

Clinical Development Updates

- Today, Rhythm provided an update on progress of its pivotal, Phase 3 clinical trial evaluating setmelanotide in patients with acquired hypothalamic obesity. Approximately two-thirds of planned patients have been screened with a very low screen-failure rate observed. Rhythm affirms its expectation to complete enrollment in the fourth quarter of 2023. This Phase 3 trial is designed to enroll 120 patients aged 4 years or older randomized 2:1 to setmelanotide therapy or placebo for a total of 60 weeks, including up to eight weeks for dose titration.
 - During The Obesity Society's ObesityWeek[®] October 14 – 17 in Dallas, Rhythm and its collaborators delivered a total of six presentations, including data showing that setmelanotide therapy resulted in sustained and deepened weight loss in patients with severe obesity caused by rare MC4R pathway diseases. Long-term extension study data in patients with hypothalamic obesity (n=12) demonstrate a 25.5% reduction in mean BMI from baseline on setmelanotide therapy at one year.
 - The Company also presented data that showed intervention with setmelanotide may reduce the risk of future metabolic syndrome, cardiovascular disease (CVD) and type 2 diabetes mellitus (T2DM) in patients with obesity due BBS or due to POMC or LEPR deficiency obesity; and research that showed severity of obesity was associated with increased prevalence of cardiac, endocrine/diabetes, and renal outcomes early in life based on an analysis of 318 pediatric patients with BBS enrolled in the Clinical Registry Investigating BBS (CRIBBS). Researchers concluded that timely diagnosis and early implementation of hyperphagia and weight management strategies in pediatric patients with BBS may reduce the risk and burden associated with these comorbidities. See the Company's [Oct. 17, 2023 press release](#) for more.
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- During the 61st Annual European Society for Paediatric Endocrinology (ESPE) meeting September 21-23 in The Hague, Netherlands, Rhythm delivered four oral presentations, including data presentations on genetic testing results and the effects of setmelanotide on the metabolic syndrome severity score in pediatric patients with BBS and in pediatric patients with POMC or LEPR deficiencies. See the Company's [Sept. 22, 2023 press release](#) for more.
- In September 2023, Rhythm announced that the European Medicines Agency (EMA) issued a positive opinion on the Company's orphan drug designation request for setmelanotide as a treatment for acquired hypothalamic obesity.

Corporate and Anticipated Upcoming Milestones

Rhythm also expects to achieve the following near-term milestones:

- Complete patient enrollment in the pivotal Phase 3 clinical trial in hypothalamic obesity in the fourth quarter of 2023;
- Complete submission to the U.S. Food and Drug Administration of an investigational new drug application for RM-718, a new, weekly, MC4R-specific agonist, in the fourth quarter of 2023. RM-718 is designed to be more targeted and potent than setmelanotide, and designed to be MC1R sparing, with the potential to not cause hyperpigmentation;
- Announce preliminary data from the open-label part of the Phase 2 DAYBREAK trial from approximately five genetically-defined cohorts in the fourth quarter of 2023;
- Announce topline data from the ongoing Phase 3, open-label pediatrics trial evaluating one year of setmelanotide therapy in patients with MC4R pathway deficiencies between the ages of 2 and 6 years old in the fourth quarter of 2023; and
- Provide pharmacokinetic and tolerability data from the ongoing Phase 3 switch trial evaluating a weekly formulation of setmelanotide in the fourth quarter of 2023.

Today, Rhythm announced plans to provide an update on several R&D programs during a breakfast event for investors and analysts on December 6, 2023 in Boston.

Third Quarter 2023 Financial Results:

- **Cash Position:** As of September 30, 2023, cash, cash equivalents and short-term investments were approximately \$299.3 million, as compared to \$333.3 million as of December 31, 2022.
 - **Revenue:** Net product revenues relating to global sales of IMCIVREE were \$22.5 million for the third quarter of 2023, as compared to \$4.3 million for the third quarter of 2022. For the third quarter ended September 30, 2023, 80% of the Company's product revenue was generated in the United States.
 - **R&D Expenses:** R&D expenses were \$33.6 million in the third quarter of 2023, as compared to \$21.1 million in the third quarter of 2022. The year-over-year increase was primarily due to increased costs associated with certain clinical trials and pre-clinical studies, increased headcount and increased fees for professional services and consulting.
 - **S,G&A Expenses:** S,G&A expenses were \$30.5 million for the third quarter of 2023, as compared to \$21.9 million for the third quarter of 2022. The year-over-year increase was primarily due to increased headcount in the United States and internationally, professional services and other expenses.
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- **Other income (expense), net.** Other income (expense), net was \$0.2 million for the third quarter of 2023. Total other income (expense), net for the three months ended September 30, 2023 consists of interest income of \$3.5 million earned on short-term investments, other expense of \$0.2 million from net foreign currency losses and the change in fair value of the Royalty Interest Financing Agreement (RIFA) embedded derivative and \$3.1 million of interest expense related to the Company's RIFA with HealthCare Royalty Partners.
- **Net Loss:** Net loss was (\$44.2) million for the third quarter of 2023, or a net loss per basic and diluted share of (\$0.76), as compared to a net loss of (\$40.9) million for the third quarter of 2022, or a net loss per basic and diluted share of (\$0.79).

Year to Date 2023 Financial Results:

- **Revenue:** Net product revenues relating to sales of IMCIVREE were \$53.2 million for the nine months ended September 30, 2023, as compared to \$8.1 million for the nine months ended September 30, 2022.
- **License Revenue:** The Company did not report license revenue relating to out-license arrangements in the nine months ended September 30, 2023. License revenue relating to the Company's out-license arrangement with RareStone was \$6.8 million for the nine months ended September 30, 2022.
- **R&D Expenses:** R&D expenses were \$105.1 million for the nine months ended September 30, 2023, as compared to \$85.1 million for the nine months ended September 30, 2022. This increase was primarily due to the acquisition of Xinvento B.V. and increased costs associated with headcount, certain clinical trials, pre-clinical studies and gene sequencing and was partially offset by decreased costs associated with less manufacturing of clinical materials.
- **S,G&A Expenses:** S,G&A expenses were \$85.2 million for the nine months ended September 30, 2023, as compared to \$65.7 million for the nine months ended September 30, 2022. The increase was primarily due to increased headcount to support business and commercial operations in the United States and internationally, professional services and other expenses and was partially offset by decreased marketing activities associated with the BBS U.S. launch during the prior year.
- **Other income (expense), net:** Other income (expense), net was \$0.4 million for the nine months ended September 30, 2023. Total other income (expense), net for the nine months ended September 30, 2023 consists of interest income of \$10.1 million earned on our short-term investments, other expense of \$0.4 million from net foreign currency losses and the change in fair value of the RIFA embedded derivative and \$9.3 million of interest expense related to the RIFA with HealthCare Royalty Partners.
- **Net Loss:** Net loss was (\$143.0) million for the nine months ended September 30, 2023, or a net loss per basic and diluted share of \$(2.50), as compared to a net loss of (\$138.6) million for the nine months ended September 30, 2022, or a net loss per basic and diluted share of (\$2.73).

Financial Guidance: For the year ending December 31, 2023, Rhythm anticipates approximately \$210 million to \$220 million in Non-GAAP Operating Expenses comprised of \$125 million to \$130 million from R&D expenses and \$85 million to \$90 million from S,G&A expenses. Non-GAAP operating expenses is defined as GAAP operating expenses excluding stock-based compensation (see below under "Non-GAAP Financial Measures" for more details).

Based on its current operating plans, Rhythm expects that its existing cash, cash equivalents and short-term investments as of September 30, 2023, will be sufficient to fund its operating expenses and capital expenditure requirements into 2026.

Conference Call Information

Rhythm Pharmaceuticals will host a live conference call and webcast at 8:00 a.m. ET today to review its third quarter 2023 financial results and recent business activities. Participants may register for the conference call [here](#). It is recommended that participants join the call ten minutes prior to the scheduled start.

A live webcast of the call will also be available under "Events and Presentations" in the Investor Relations section of the Rhythm Pharmaceuticals website at <https://ir.rhythmtx.com/>. The archived webcast will be available on Rhythm Pharmaceuticals' website approximately two hours after the conference call and will be available for 30 days following the call.

About Rhythm Pharmaceuticals

Rhythm is a commercial-stage biopharmaceutical company committed to transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) diseases. Rhythm's lead asset, IMCIVREE (setmelanotide), an MC4R agonist designed to treat hyperphagia and severe obesity caused by rare MC4R pathway diseases, is approved by the U.S. Food and Drug Administration (FDA) for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency confirmed by genetic testing, or patients with a clinical diagnosis of Bardet-Biedl syndrome (BBS). Both the European Commission (EC) and the UK's Medicines & Healthcare Products Regulatory Agency (MHRA) have authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare MC4R pathway diseases, as well as a preclinical suite of small molecules for the treatment of congenital hyperinsulinism. Rhythm's headquarters is in Boston, MA.

Setmelanotide Indication

In the United States, setmelanotide is indicated for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to POMC, PCSK1 or LEPR deficiency as determined by an FDA-approved test demonstrating variants in POMC, PCSK1 or LEPR genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS) or BBS.

In the European Union, setmelanotide is indicated for the treatment of obesity and the control of hunger associated with genetically confirmed Bardet-Biedl syndrome (BBS) or genetically confirmed loss-of-function biallelic proopiomelanocortin (POMC), including PCSK1, deficiency or biallelic leptin receptor (LEPR) deficiency in adults and children 6 years of age and above.

Limitations of Use

In the United States and Europe, Setmelanotide should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

Setmelanotide is not indicated for the treatment of patients with the following conditions as setmelanotide would not be expected to be effective:

- Obesity due to suspected POMC, PCSK1 or LEPR deficiency with *POMC*, *PCSK1* or *LEPR* variants classified as benign or likely benign
- Other types of obesity not related to POMC, PCSK1 or LEPR deficiency, or BBS, including obesity associated with other genetic syndromes and general (polygenic) obesity.

WARNINGS AND PRECAUTIONS

Skin Monitoring: Setmelanotide may lead to generalized increased skin pigmentation and darkening of pre-existing naevi because of its pharmacologic effect. Full body skin examinations should be conducted annually to monitor pre-existing and new skin pigmentary lesions before and during treatment with setmelanotide.

Heart rate and blood pressure monitoring: Heart rate and blood pressure should be monitored as part of standard clinical practice at each medical visit (at least every 6 months) for patients treated with setmelanotide.

Prolonged penile erection: Spontaneous penile erections have been reported in clinical trials with setmelanotide. Patients who have a penile erection lasting longer than 4 hours should be instructed to seek emergency medical attention for potential treatment of priapism.

Depression: In clinical trials, depression has been reported in patients treated with setmelanotide. Patients with depression should be monitored at each medical visit during treatment with setmelanotide. Consideration should be given to discontinuing setmelanotide if patients experience suicidal thoughts or behaviors.

Pediatric Population: The prescribing physician should periodically assess response to setmelanotide therapy. In growing children, the impact of weight loss on growth and maturation should be evaluated. The prescribing physician should monitor growth (height and weight) using age- and sex-appropriate growth curves.

Excipients: This medicinal product contains 10 mg benzyl alcohol in each ml. Benzyl alcohol may cause allergic reactions. Patients who are pregnant or breastfeeding should be advised of the potential risk from the excipient benzyl alcohol, which might accumulate over time and cause metabolic acidosis. This medicinal product should be used with caution in patients with hepatic or renal impairment, because of the potential risk from the excipient benzyl alcohol which might accumulate over time and cause metabolic acidosis.

Sodium: This medicinal product contains less than 1 mmol sodium (23 mg) per dose, that is to say essentially “sodium-free.”

ADVERSE REACTIONS

The most frequent adverse reactions are hyperpigmentation (51%), injection site reaction (39%), nausea (33%), and headache (26%).

USE IN SPECIFIC POPULATIONS

Pregnancy

There are no data from the use of setmelanotide in pregnant women. Animal studies do not indicate direct harmful effects with respect to reproductive toxicity. However, administration of setmelanotide to pregnant rabbits resulted in decreased maternal food consumption leading to embryo-fetal effects. As a precautionary measure, setmelanotide should not be started during pregnancy or while attempting to get pregnant as weight loss during pregnancy may result in fetal harm. If a patient who is taking setmelanotide has reached a stable weight and becomes pregnant, consideration should be given to maintaining setmelanotide treatment as there was no proof of teratogenicity in the nonclinical data. If a patient who is taking setmelanotide and still losing weight gets pregnant, setmelanotide should either be discontinued, or the dose reduced while monitoring for the recommended weight gain during pregnancy. The treating physician should carefully monitor weight during pregnancy in a patient taking setmelanotide.

Breast-feeding

It is unknown whether setmelanotide is excreted in human milk. A nonclinical study showed that setmelanotide is excreted in the milk of nursing rats. No quantifiable setmelanotide concentrations were detected in plasma from nursing pups. A risk to the newborn/infant cannot be excluded. A decision must be made whether to discontinue breastfeeding or to discontinue/abstain from setmelanotide therapy taking into account the benefit of breastfeeding for the child and the benefit of therapy for the mother.

Fertility

No human data on the effect of setmelanotide on fertility are available. Animal studies did not indicate harmful effects with respect to fertility.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337. [See Summary of Product Characteristics' APPENDIX V](#) for a list of European national reporting systems to communicate adverse reactions.

Please see the full Prescribing Information for additional Important Safety Information.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding the potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, including the anticipated timing for initiation of clinical trials and release of clinical trial data, our expectations surrounding potential regulatory submissions, approvals and timing thereof, including the anticipated IND application for RM-718, our business strategy and plans, including regarding commercialization of setmelanotide in certain international regions, expectations surrounding sales and reimbursement of IMCIVREE, our anticipated financial performance and financial position, including estimated Non-GAAP Operating Expenses for the year ending December 31, 2023, the sufficiency of our cash, cash equivalents and short-term investments to fund our operations, and our participation in upcoming events and presentations. Statements using word such as “expect”, “anticipate”, “believe”, “may”, “will”, “aim” and similar terms are also forward-looking statements. Such statements are subject to numerous risks and uncertainties, including, but not limited to, risks relating to our liquidity and expenses, our ability to enroll patients in clinical trials, the design and outcome of clinical trials, the ability to achieve necessary regulatory approvals, risks associated with data analysis and reporting, failure to identify and develop additional product candidates, unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, risks associated with the laws and regulations governing our international operations and the costs of any related compliance programs, the impact of competition, risks relating to product liability lawsuits, inability to maintain our collaborations, or the failure of these collaborations, our reliance on third parties, risks relating to intellectual property, our ability to hire and retain necessary personnel, the impact of the COVID-19 pandemic and general economic conditions on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, failure to realize the anticipated benefits of our acquisition of Xinvento B.V. or significant integration difficulties related to the acquisition, and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023 and our other filings with the Securities and Exchange Commission. Except as required by law, we undertake no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

Non-GAAP Financial Measures

This press release includes Non-GAAP Operating Expenses, a supplemental measure of our performance that is not required by, or presented in accordance with, U.S. GAAP and should not be considered as an alternative to operating expenses or any other performance measure derived in accordance with GAAP.

We define Non-GAAP Operating Expenses as GAAP operating expenses excluding stock-based compensation.

We caution investors that amounts presented in accordance with our definition of Non-GAAP Operating Expenses may not be comparable to similar measures disclosed by our competitors because not all companies and analysts calculate this non-GAAP financial measure in the same manner. We present this non-GAAP financial measure because we consider it to be an important supplemental measure of our performance and believe it is frequently used by securities analysts, investors, and other interested parties in the evaluation of companies in our industry. Management believes that investors' understanding of our performance is enhanced by including this non-GAAP financial measure as a reasonable basis for comparing our ongoing results of operations.

Management uses this non-GAAP financial measure for planning purposes, including the preparation of our internal annual operating budget and financial projections; to evaluate the performance and effectiveness of our operational strategies; and to evaluate our capacity to expand our business. This non-GAAP financial measure has limitations as an analytical tool, and should not be considered in isolation, or as an alternative to, or a substitute for operating expenses or other financial statement data presented in accordance with GAAP in our consolidated financial statements.

Rhythm has not provided a quantitative reconciliation of forecasted Non-GAAP Operating Expenses to forecasted GAAP operating expenses because the Company is unable, without making unreasonable efforts, to calculate the reconciling item, stock-based compensation expenses, with confidence. This item, which could materially affect the computation of forward-looking GAAP operating expenses, is inherently uncertain and depends on various factors, some of which are outside of Rhythm's control.

Corporate Contact:

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Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)
(Unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2023	2022	2023	2022
Revenues:				
Product revenue, net	\$ 22,504	\$ 4,284	\$ 53,194	\$ 8,094
License revenue	—	—	—	6,754
Total revenues	<u>22,504</u>	<u>4,284</u>	<u>53,194</u>	<u>14,848</u>
Costs and expenses:				
Cost of sales	2,412	497	6,069	1,105
Research and development	33,570	21,116	105,059	85,082
Selling, general, and administrative	30,475	21,938	85,158	65,715
Total costs and expenses	<u>66,457</u>	<u>43,551</u>	<u>196,286</u>	<u>151,902</u>
Loss from operations	(43,953)	(39,267)	(143,092)	(137,054)
Other income (expense):				
Other income (expense), net	(159)	(370)	(369)	(370)
Interest expense	(3,149)	(2,144)	(9,342)	(2,190)
Interest income	3,466	920	10,126	988
Total other income, net	<u>158</u>	<u>(1,594)</u>	<u>415</u>	<u>(1,572)</u>
(Loss) income before taxes	(43,795)	(40,861)	(142,677)	(138,626)
Provision for income taxes	368	—	368	—
Net loss	<u>\$ (44,163)</u>	<u>\$ (40,861)</u>	<u>\$ (143,045)</u>	<u>\$ (138,626)</u>
Net loss per share, basic and diluted	<u>\$ (0.76)</u>	<u>\$ (0.79)</u>	<u>\$ (2.50)</u>	<u>\$ (2.73)</u>
Weighted-average common shares outstanding, basic and diluted	<u>57,874,960</u>	<u>51,400,922</u>	<u>57,154,803</u>	<u>50,712,452</u>
Other comprehensive loss:				
Net loss	\$ (44,163)	\$ (40,861)	\$ (143,045)	\$ (138,626)
Reclassification of losses on RareStone equity into net loss		300		
Foreign currency translation adjustment	76	—	49	—
Unrealized gain (loss), net on marketable securities	(175)	267	(70)	(338)
Comprehensive loss	<u>\$ (44,262)</u>	<u>\$ (40,294)</u>	<u>\$ (143,066)</u>	<u>\$ (138,964)</u>

Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	\$ 64,593	\$ 127,677
Short-term investments	234,667	205,611
Accounts receivable, net	14,541	6,224
Inventory	7,762	2,917
Prepaid expenses and other current assets	7,638	11,807
Total current assets	329,201	354,236
Property and equipment, net	1,545	2,197
Right-of-use asset	888	1,182
Intangible assets, net	7,242	7,883
Restricted cash	328	328
Other long-term assets	14,995	16,655
Total assets	<u>\$ 354,199</u>	<u>\$ 382,481</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 3,839	\$ 4,797
Accrued expenses and other current liabilities	44,271	32,894
Deferred revenue	1,286	1,434
Lease liability	748	684
Total current liabilities	50,144	39,809
Long-term liabilities:		
Deferred royalty obligation	104,699	75,810
Lease liability	692	1,260
Derivative liability	1,190	1,340
Total liabilities	156,725	118,219
Stockholders' equity:		
Preferred stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2023 and December 31, 2022	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 59,089,352 and 56,612,429 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	59	56
Additional paid-in capital	1,050,631	974,356
Accumulated other comprehensive loss	(113)	(92)
Accumulated deficit	(853,103)	(710,058)
Total stockholders' equity	197,474	264,262
Total liabilities and stockholders' equity	<u>\$ 354,199</u>	<u>\$ 382,481</u>