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): May 2, 2023

RHYTHM PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-38223**	**46-2159271**
(State or other jurisdiction of incorporation)	(Commission File Number)	(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 May 2, 2023, Rhythm Pharmaceuticals, Inc. (the "Company") announced its financial results for the quarter ended March 31, 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 May 2, 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: May 2, 2023

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

Exhibit 99.1

Rhythm Pharmaceuticals Reports First Quarter 2023 Financial Results and Business Update

-- Strong U.S. commercial progress continues for IMCIVREE® (setmelanotide) with more than 300 new prescriptions for Bardet-Biedl syndrome (BBS) received since FDA approval --

-- Launched IMCIVREE for BBS in Germany with federal reimbursement --

-- First patients dosed in pivotal Phase 3 trial evaluating setmelanotide in acquired hypothalamic obesity; completion of enrollment expected 1Q 2024 --

-- Executing on six clinical trials with three data readouts expected in 2H 2023 --

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

BOSTON, May 2, 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 first quarter ended March 31, 2023.

"Rhythm is off to a strong start in 2023 with IMCIVREE® (setmelanotide) commercial progress with more than 300 new U.S. prescriptions for Bardet-Biedl syndrome (BBS) since launch. Internationally, we are excited about our first European launch for BBS in Germany with federal reimbursement and looking forward to further expansion into additional global markets this year," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm.

Dr. Meeker added, "We also are pleased to announce that we dosed the first patients in our pivotal Phase 3 trial in hypothalamic obesity, and we anticipate completing enrollment in the first quarter of 2024. With an identified and engaged patient population and encouraging data from our Phase 2 trial, hypothalamic obesity represents a meaningful opportunity for Rhythm. In addition, we are advancing our newly acquired program from Xinvento in congenital hyperinsulinism (CHI), a natural strategic fit for our portfolio."

First Quarter and Recent Business Highlights

Commercial Update

- Today, Rhythm announced that more than 300 new prescriptions for IMCIVREE for BBS have been written from more than 175 physicians in the United States since U.S. Food and Drug Administration (FDA) approval on June 16, 2022 through March 31, 2023. The Company also announced that it had received payor approval for reimbursement for more than 160 of those prescriptions, as of March 31, 2023. More than 100 new prescriptions were written in the first quarter of 2023.
- On April 24, 2023, the Company announced the commercial launch of IMCIVREE in Germany for the treatment of obesity and control of hunger associated with BBS with federal reimbursement. The German Federal Joint Committee (G-BA) ruled that IMCIVREE is eligible for reimbursement by Statutory Health Insurances for BBS based on its unanimous vote to exclude IMCIVREE from its lifestyle exemption list for patients with BBS.

Clinical Development Updates

Hypothalamic Obesity

- Today, Rhythm announced that the first patients have been dosed in its pivotal Phase 3 trial evaluating setmelanotide in patients with acquired hypothalamic obesity. The Phase 3 clinical trial incorporates FDA feedback and 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. The primary endpoint is the percent change in Body Mass Index (BMI) after approximately 52 weeks on a therapeutic regimen of setmelanotide versus placebo.

Additional Updates

- In March 2023, Rhythm published research in the peer-reviewed journal *Advances in Therapy* that demonstrated setmelanotide improved hyperphagia and reduced obsessive focus on food and reduced body weight in patients with BBS.
- Also in March, Rhythm announced the publication of an assessment of the substantial impact of severe hyperphagia on patients' quality of life in the open-access journal *Obesity Science and Practice*. This study was the first to estimate the impact of hyperphagia on health state utilities independently of any specific underlying indication.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Present data analysis from the Phase 2 and long-term extension trials in hypothalamic obesity at a medical conference in the fall of 2023;
- Complete regulatory review by Health Canada and, pending approval, make IMCIVREE commercially available in Canada for the treatment of BBS, or POMC, PCSK1 or LEPR deficiencies in the second half of 2023;
- Initiate a Phase 3, randomized, double-blind trial in patients naïve to setmelanotide therapy ("de novo study") to evaluate the weekly formulation of setmelanotide in patients with BBS in the second half of 2023;
- Announce preliminary data from the open-label part of the Phase 2 DAYBREAK trial from one or more genetically-defined cohorts in the second half 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 second half of 2023;
- Announce topline data from the ongoing Phase 3 switch trial evaluating a weekly formulation of setmelanotide in the second half of 2023;
- Unveil lead development candidate for the treatment of CHI and provide updates on early-stage R&D efforts in the fourth quarter of 2023; and
- Complete patient enrollment in the pivotal Phase 3 clinical trial in hypothalamic obesity in the first quarter of 2024.

First Quarter 2023 Financial Results:

- **Cash Position:** As of March 31, 2023, cash, cash equivalents and short-term investments were approximately $294.6 million, as compared to $333.3 million as of December 31, 2022.
- **Revenue:** Net product revenues relating to global sales of IMCIVREE were $11.5 million for the first quarter of 2023, as compared to $1.5 million for the first quarter of 2022. For the first quarter ended March 31, 2023, 83% of the Company's product revenue was generated in the United States.
- **R&D Expenses:** R&D expenses were $37.9 million in the first quarter of 2023, as compared to $32.5 million in the first quarter of 2022. The year-over-year increase of approximately $5.4 million was due to the $5.4M acquisition of Xinvento B.V.'s in-process research & development assets. In addition, there were decreased clinical trial costs, clinical trial manufacturing costs and milestone payments, which were partially offset by increased costs due to additional headcount and gene sequencing.
- **S,G&A Expenses:** S,G&A expenses were $24.6 million for the first quarter of 2023, as compared to $21.4 million for the first quarter of 2022. The year-over-year increase was primarily due to investments in North America and International commercial activities and headcount.
- **Net Loss:** Net loss was $52.2 million for the first quarter of 2023, or a net loss per basic and diluted share of $0.92, as compared to a net loss of $52.8 million for the first quarter of 2022, or a net loss per basic and diluted share of $1.05.

Financial Guidance: For the year ending December 31, 2023, Rhythm continues to anticipate approximately $200 million to $220 million in Non-GAAP Operating Expenses (see below under "Non-GAAP Financial Measures" for more details), comprised of $120 million to $130 million from R&D expenses and $80 million to $90 million from S,G&A expenses. Based on its current operating plans, Rhythm expects that its existing cash, cash equivalents and short-term investments as of March 31, 2023 will be sufficient to fund its operating expenses and capital expenditure requirements into 2025.

Conference Call Information

Rhythm Pharmaceuticals will host a live conference call and webcast at 8:00 a.m. ET today to review its first quarter 2023 financial results and recent business activities. Participants may register for the conference call here. While not required, 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, our business strategy and plans, including regarding commercialization of setmelanotide in certain international regions, expectations surrounding sales and reimbursement of IMCIVREE, the potential financial impact, growth prospects and benefits of our acquisition of Xinvento B.V., expectations surrounding lead development candidate selection for the treatment of CHI and related timing, our anticipated financial performance and financial position, including estimated Non-GAAP Operating Expenses for the year ending December 31, 2023, and 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 Annual Report on Form 10-K for the year ended December 31, 2022 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.

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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 March 31,	
	2023	**2022**
Revenues:		
Product revenue, net	$ 11,469	$ 1,498
Costs and expenses:		
Cost of sales	1,421	230
Research and development	37,945	32,510
Selling, general, and administrative	24,634	21,449
Total costs and expenses	64,000	54,189
Loss from operations	(52,531)	(52,691)
Other income:		
Other expense, net	(27)	(233)
Interest expense	(3,061)	—
Interest income	3,440	160
Total other income (expense), net	352	(73)
Net loss	$ (52,179)	$ (52,764)
Net loss per share, basic and diluted	$ (0.92)	$ (1.05)
Weighted-average common shares outstanding, basic and diluted	56,708,975	50,326,627
Other comprehensive loss:		
Net loss	$ (52,179)	$ (52,764)
Foreign currency translation adjustment	21	—
Unrealized gain (loss), net on marketable securities	65	(628)
Comprehensive loss	$ (52,093)	$ (53,392)

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

	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 109,661	$ 127,677
Short-term investments	184,921	205,611
Accounts receivable, net	8,117	6,224
Inventory	5,487	2,917
Prepaid expenses and other current assets	9,652	11,807
Total current assets	317,838	354,236
Property and equipment, net	2,001	2,197
Right-of-use asset	1,088	1,182
Intangible assets, net	7,669	7,883
Restricted cash	328	328
Other long-term assets	16,754	16,655
Total assets	$ 345,678	$ 382,481
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 13,347	$ 4,797
Accrued expenses and other current liabilities	30,549	32,894
Deferred revenue	1,428	1,434
Lease liability	705	684
Total current liabilities	46,029	39,809
Long-term liabilities:		
Deferred royalty obligation	77,520	75,810
Lease liability	1,076	1,260
Derivative liability	1,290	1,340
Total liabilities	125,915	118,219
Stockholders' equity:		
Preferred Stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at March 31, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 56,852,404 and 56,612,429 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively	56	56
Additional paid-in capital	981,950	974,356
Accumulated other comprehensive loss	(6)	(92)
Accumulated deficit	(762,237)	(710,058)
Total stockholders' equity	219,763	264,262
Total liabilities and stockholders' equity	$ 345,678	$ 382,481