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): March 1, 2022

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 March 1, 2022, Rhythm Pharmaceuticals, Inc. (the "Company") announced its financial results for the quarter and year ended December 31, 2021. 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 March 1, 2022
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: March 1, 2022

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

Exhibit 99.1

Rhythm Pharmaceuticals Reports Fourth Quarter and Full Year 2021 Financial Results

-- Preparations continue for U.S. commercial launch in June 2022 in BBS and Alström syndrome, pending FDA approval --

-- First commercial sales of IMCIVREE in Germany and France expected in 2Q2022 –

-- Enrollment completed in Phase 2 trial evaluating setmelanotide in patients with hypothalamic obesity --

-- IMCIVREE fourth quarter sales of $1.8M and 2021 sales of $3.2M –

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

BOSTON, March 1, 2022 – Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a commercial-stage biopharmaceutical company committed to transforming the care of people living with rare genetic diseases of obesity, today reported financial results and provided a business update for the fourth quarter and full year ended December 31, 2021.

“2021 was a pivotal year for Rhythm, with tremendous progress across clinical development and regulatory efforts globally, as we deliver on our promise to transform the treatment of rare genetic diseases of obesity,” said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. “We first made IMCIVREE® (setmelanotide) commercially available last year to people living with obesity due to POMC, PCSK1 or LEPR deficiency, and now we look forward to our first commercial sales in Europe in the second quarter of this year. With a potential U.S. approval for IMCIVREE in BBS and Alström syndrome now set for June, our commercial team continues to drive patient identification and pre-launch activities as we prepare to deliver IMCIVREE to the waiting BBS and Alström syndrome communities at approval.”

Dr. Meeker continued, “We also significantly expanded our clinical development efforts with the Phase 3 EMANATE and Phase 2 DAYBREAK trials, the pediatrics trial for patients between the ages of 2 and 6, and the switch study evaluating a once-weekly formulation of setmelanotide. We are excited to have completed enrollment in our Phase 2 hypothalamic obesity trial with plans to read out data in the first half of this year. These studies have the potential to meaningfully expand the reach of setmelanotide and address the severe obesity and hyperphagia associated with rare genetic diseases of obesity, and, in the case of hypothalamic obesity, an acquired deficiency in the hypothalamic melanocortin pathway.”

Fourth Quarter and Recent Business Highlights:

Commercial Readiness for Bardet Biedl

- In February 2022, Rhythm provided an update on ongoing preparations in support of a potential U.S. commercial launch of setmelanotide for the treatment of obesity and hyperphagia in patients with BBS. Mary Morris, caregiver for her two adult children living with BBS, and key opinion leaders Dr. Robert Haws of the Marshfield Clinic in Wisconsin and Dr. Rushika Conroy of Baystate Children's Hospital in Springfield, Mass., discussed the significant need for an effective therapy for hyperphagia and obesity associated with BBS and discussed setmelanotide’s potential efficacy. In addition, Rhythm management shared details on the Company’s commercial organization, patient identification results to date and launch strategies.

International Updates

- Today, Rhythm announced that the German Federal Joint Committee (G-BA) unanimously voted to exclude IMCIVREE from its lifestyle exemption list for POMC, PCSK1 or LEPR deficiency obesity. In Germany, drugs classified as lifestyle drugs, which include those designed to effect weight loss, smoking cessation or hair loss, are not eligible for reimbursement. This first-ever exclusion marks an important recognition that IMCIVREE is designed to treat rare genetic diseases that manifest as obesity and that this group of diseases is distinct from general obesity. With this exemption status, IMCIVREE would be eligible for national coverage and reimbursement. The Company expects its first commercial sales in Germany in the second quarter of 2022.

- Today, Rhythm announced that the French Haute Autorité de Santé (HAS) granted paid early access for IMCIVREE for patients with POMC, PCSK1 or LEPR deficiency obesity, which the company expects to commence in the second quarter of 2022.

- In addition, Rhythm announced that it has submitted reimbursement dossiers for POMC, PCSK1 or LEPR deficiency obesities in seven countries, including France, Germany, Italy, Spain, the Netherlands, Israel, and the United Kingdom, with positive feedback from EU payers and favorable HTA ratings from authorities in several countries.

- In December 2021, Rhythm announced an exclusive licensing agreement with RareStone LTD, formerly Citrine Medicine, for the development and commercialization of IMCIVREE in China, including mainland China, Hong Kong and Macau.

Clinical Development Updates:

- Today, Rhythm announced it has completed enrollment in its Phase 2 open-label, proof-of-concept study designed to evaluate setmelanotide in patients with hypothalamic obesity.

- Today, Rhythm announced the first patient has been dosed in the Phase 3 trial evaluating setmelanotide in pediatric patients aged 2 to younger than 6 years old.

- In February 2022, Rhythm announced positive data from its long-term extension study evaluating setmelanotide in patients with BBS.

- In January 2022, Rhythm announced the dosing of the first patients in the Phase 2 DAYBREAK clinical trial.

- Also in January 2022, Rhythm announced the dosing of the first patients in the Phase 3 switch trial evaluating a one-weekly formulation of setmelanotide in patients 6 years of age and older.

Regulatory Updates:

Bardet-Biedl Syndrome and Alström Syndrome:

- The Company's supplemental New Drug Application (sNDA) for IMCIVREE® (setmelanotide) for the treatment of obesity and control of hunger in adult and pediatric patients 6 years of age and older with Bardet-Biedl syndrome (BBS) or Alström syndrome has been accepted and is under review by the U.S. Food and Drug Administration (FDA). The FDA updated the Prescription Drug User Fee Act (PDUFA) goal date to June 16, 2022.

- Rhythm's Type II variation application to the European Medicines Agency (EMA) for setmelanotide for the treatment of obesity and control of hunger in adult and pediatric patients 6 years of age and older with BBS also is under review. The Company recently decided to withdraw the proposed Alström syndrome indication from this submission.

Key Upcoming Milestones:

- Today, Rhythm announced its Phase 3 EMANATE clinical trial will be initiated in the first half of 2022.

- In the first half of 2022, Rhythm expects to:
    - Announce preliminary data from the ongoing Phase 2 study in patients with hypothalamic obesity;
    - Announce new data from the ongoing exploratory Phase 2 Basket Study evaluating setmelanotide in patients with obesity due to a variant in the MC4 receptor; and
    - Announce long term-extension trial data in the in the first half of 2022, including: 12-month data for SRC1 and SH2B1 deficiency obesities and 24-month data for BBS and biallelic POMC, PCSK1 or LEPR deficiency obesities.
- Rhythm anticipates that the EMA's Committee for Medicinal Products for Human Use (CHMP) will make it recommendation on the Company's Type II variation application to the for setmelanotide for the treatment of obesity and control of hunger in adult and pediatric patients 6 years of age and older with BBS in the second half of 2022.
- The Company expects to 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 2022.

Fourth Quarter and Full Year 2021 Financial Results:

- **Cash Position:** As of December 31, 2021, cash, cash equivalents and short-term investments were approximately $294.9 million, as compared to $172.8 million as of December 31, 2020.
- **Revenue**: Product net revenues relating to sales of IMCIVREE in the United States were $1.8 million for the fourth quarter of 2021 and $3.2 million for the year ended December 31, 2021. Rhythm did not generate any product revenues in the fourth quarter or year ended 2020, as IMCIVREE became commercially available in the first quarter of 2021.
- **R&D Expenses:** R&D expenses were $31.6 million in the fourth quarter of 2021 and $104.1 million for the year ended December 31, 2021, as compared to $22.0 million in the fourth quarter of 2020 and $90.5 million for the year ended December 31, 2020. The year-over-year increase was due to an increase of $11.2 million in clinical trial costs associated with the Phase 2 DAYBREAK and Phase 3 EMANATE trials, Phase 3 pediatrics trial, QTc study, Phase 2 hypothalamic obesity study, and increased enrollment in the long-term extension study; an increase of $7.7 million in salaries, benefits and stock-based compensation related to the hiring of additional full-time employees to support the growth in research and development programs; and an increase of $1.3 million of pre-clinical expenses associated with second generation drug development. These increases were partially offset by the conclusion of prior studies; a decrease of $3.0 million due to the milestone expenses associated with the license agreement with Ipsen on filing the new drug application (NDA) and marketing authorisation application (MAA) for setmelanotide, which occurred in the fourth quarter of 2020; a decrease of $2.0 million primarily related to purchases of setmelanotide active pharmaceutical ingredient and drug product for clinical trials and commercialization; and a decrease of $1.4 million in costs associated with accessing sequencing data from third-party biobanks for genetic research efforts.
- **S,G&A Expenses:** S,G&A expenses were $21.0 million in the fourth quarter of 2021 and $68.5 million for the year ended December 31, 2021, as compared to $13.1 million for the fourth quarter of 2020 and $46.1 million for the year ended December 31, 2020. The year-over-year increase was due to an increase of $9.9 million due to increased compensation and benefits related costs associated with additions to Rhythm's executive leadership team, increased headcount to support expanding business operations as well as to establish commercial operations in the United States and internationally; an increase of $5.9 million in commercial expenses to support the build out of commercial operations in the United States and internationally as well as corporate legal and consulting support for the formation of European affiliates; an increase of $3.7 million related to efforts to drive patient engagement and disease awareness about rare genetic causes of obesity and to prepare for commercialization of setmelanotide in the U.S.; an increase of $1.6 million due to fees associated with the sale of the Company's priority review voucher to Alexion; and increase of $1.3 million for employee recruitment and other related expenses to support expanding business operations in the United States and internationally.

- **Net Loss:** Net loss was $42.9 million for the fourth quarter of 2021 and $69.6 million for the year ended December 31, 2021, or a net loss per basic and diluted share of $0.85 and $1.40, respectively, as compared to a net loss of $34.9 million for the fourth quarter of 2020 and $134.0 million for the year ended December 31, 2020, or a net loss per basic and diluted share of $0.79 and $3.04, respectively.

Financial Guidance: Based on its current operating plans, Rhythm expects that its existing cash, cash equivalents and short-term investments as of December 31, 2021 will be sufficient to fund its operating expenses and capital expenditure requirements into at least the second half of 2023.

Conference Call Information

Rhythm Pharmaceuticals will host a live conference call and webcast at 8:00 a.m. ET today to discuss this update, as well as review its fourth quarter and full year 2021 financial results and recent business activities. The conference call may be accessed by dialing (844) 498-0570 (domestic) or (409) 983-9726 (international) and referring to conference ID 9840866. A webcast of the call will be available under "Events and Presentations" in the Investor Relations section of the Rhythm Pharmaceuticals website at http://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 treatment paradigm for people living with rare genetic diseases of obesity. Rhythm's precision medicine, IMCIVREE (setmelanotide), was approved in November 2020 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 obesity due to POMC, PCSK1 or LEPR deficiency confirmed by genetic testing and in July and September 2021, respectively, by the European Commission (EC) and Great Britain's Medicines & Healthcare Products Regulatory Agency (MHRA) for the treatment of obesity and the control of hunger associated with genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above. IMCIVREE is the first-ever FDA-approved and EC- and MHRA-authorized therapy for patients with these rare genetic diseases of obesity. The Company submitted a supplemental New Drug Application (sNDA) to the FDA, which was accepted for filing in November 2021 and is currently assigned a Prescription Drug User Fee Act (PDUFA) goal date of June 16, 2022, for the treatment of obesity and control of hunger in adult and pediatric patients six years of age and older with Bardet-Biedl Syndrome (BBS) or Alström syndrome. A Type II variation application to the European Medicines Agency seeking regulatory approval and authorization for setmelanotide to treat obesity and control of hunger in adult and pediatric patients 6 years of age and older with BBS also is under review. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare genetic diseases of obesity and is leveraging the Rhythm Engine and the largest known obesity DNA database -- now with approximately 45,000 sequencing samples -- to improve the understanding, diagnosis and care of people living with severe obesity due to certain genetic deficiencies. Rhythm's headquarters is in Boston, MA.

IMCIVREE® (setmelanotide) Indication

In the United States, IMCIVREE is indicated for chronic weight management in adult and pediatric patients 6 years of age and older with obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency, confirmed by an FDA-approved genetic test demonstrating variants in *POMC*, *PCSK1*, or *LEPR* genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS).

In the EU and Great Britain, IMCIVREE is indicated for the treatment of obesity and the control of hunger associated with genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above. IMCIVREE should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

Limitations of Use

IMCIVREE is not indicated for the treatment of patients with the following conditions as IMCIVREE 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, including obesity associated with other genetic syndromes and general (polygenic) obesity.

Important Safety Information

WARNINGS AND PRECAUTIONS

Disturbance in Sexual Arousal: Sexual adverse reactions may occur in patients treated with IMCIVREE. Spontaneous penile erections in males and sexual adverse reactions in females occurred in clinical studies with IMCIVREE. Instruct patients who have an erection lasting longer than 4 hours to seek emergency medical attention.

Depression and Suicidal Ideation: Some drugs that target the central nervous system, such as IMCIVREE, may cause depression or suicidal ideation. Monitor patients for new onset or worsening of depression. Consider discontinuing IMCIVREE if patients experience suicidal thoughts or behaviors.

Skin Pigmentation and Darkening of Pre-Existing Nevi: IMCIVREE may cause generalized increased skin pigmentation and darkening of pre-existing nevi due to its pharmacologic effect. This effect is reversible upon discontinuation of the drug. Perform a full body skin examination prior to initiation and periodically during treatment with IMCIVREE to monitor pre-existing and new skin pigmentary lesions.

Risk of Serious Adverse Reactions Due to Benzyl Alcohol Preservative in Neonates and Low Birth Weight Infants: IMCIVREE is not approved for use in neonates or infants.

ADVERSE REACTIONS

- The most common adverse reactions (incidence ≥23%) were injection site reactions, skin hyperpigmentation, nausea, headache, diarrhea, abdominal pain, back pain, fatigue, vomiting, depression, upper respiratory tract infection, and spontaneous penile erection.

USE IN SPECIFIC POPULATIONS

Discontinue IMCIVREE when pregnancy is recognized unless the benefits of therapy outweigh the potential risks to the fetus.

Treatment with IMCIVREE is not recommended for use while breastfeeding.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

See Full Prescribing Information, EU SmPC and MHRA SmPC for IMCIVREE.

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 and our expectations surrounding potential regulatory submissions, approvals and timing thereof, our business strategy and plans, including regarding commercialization of setmelanotide, sales of our lead product candidate IMCIVREE, management changes, our participation in upcoming events and presentations, and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations. Statements using word such as "expect", "anticipate", "believe", "may", "will" and similar terms are also forward-looking statements. Such statements are subject to numerous risks and uncertainties, including, but not limited to, the impact of our management transition, our ability to enroll patients in clinical trials, the design and outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, risks associated with data analysis and reporting, our liquidity and expenses, the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and general economic conditions, 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, 2021 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.

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

	Three months ended December 31,		Year ended December 31,	
	2021	2020	2021	2020
Product revenue, net	$ 1,817	$ —	$ 3,154	$ —
Costs and expenses:				
Cost of sales	236	—	599	—
Research and development	31,574	21,954	104,128	90,450
Selling, general, and administrative	20,997	13,119	68,486	46,125
Total costs and expenses	52,807	35,073	173,213	136,575
Loss from operations	(50,990)	(35,073)	(170,059)	(136,575)
Other income:				
Other income	—	—	100,000	—
Interest income, net	134	176	447	2,579
Total other income, net	134	176	100,447	2,579
Loss before taxes	(50,856)	(34,897)	(69,612)	(133,996)
Benefit from income taxes	(7,989)	—	—	—
Net loss	$ (42,867)	$ (34,897)	$ (69,612)	$ (133,996)
Net loss per share, basic and diluted	$ (0.85)	$ (0.79)	$ (1.40)	$ (3.04)
Weighted-average common shares outstanding, basic and diluted	50,270,801	44,216,694	49,600,294	44,127,220

Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 59,248	$ 100,854
Short-term investments	235,607	71,938
Accounts receivable	1,025	—
Prepaid expenses and other current assets	12,507	8,876
Total current assets	308,387	181,668
Property and equipment, net	2,813	3,195
Right-of-use asset	1,522	1,807
Intangible assets, net	4,658	—
Restricted cash	328	403
Other long-term assets	11,815	—
Total assets	$ 329,523	$ 187,073
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,737	$ 4,900
Accrued expenses and other current liabilities	30,084	12,559
Contract liability	7,000	—
Lease liability	606	535
Total current liabilities	43,427	17,994
Long-term liabilities:		
Lease liability	1,945	2,551
Total liabilities	45,372	20,545
Stockholders' equity:		
Preferred Stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at December 31, 2021 and December 31, 2020	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 50,283,574 and 44,235,903 shares issued and outstanding December 31, 2021 and December 31, 2020, respectively	50	44
Additional paid-in capital	813,041	625,762
Accumulated other comprehensive (loss) income	(1)	49
Accumulated deficit	(528,939)	(459,327)
Total stockholders' equity	284,151	166,528
Total liabilities and stockholders' equity	$ 329,523	$ 187,073