Rhythm Pharmaceuticals Reports First Quarter 2026 Financial Results and Business Update

May 5, 2026

-- IMCIVREE® (setmelanotide) launched in the U.S. for acquired hypothalamic obesity; more than 150 patient start forms received in the first six weeks following FDA approval on March 19, 2026 --

-- First quarter 2026 net product revenue from global sales of IMCIVREE of $60.1 million --

-- IMCIVREE granted Marketing Authorization by European Commission for the treatment of obesity and control of hunger in patients with acquired hypothalamic obesity --

-- Japanese regulatory review of New Drug Application for setmelanotide to treat acquired hypothalamic obesity underway --

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

BOSTON, May 05, 2026 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a global commercial-stage biopharmaceutical company focused on transforming the lives of patients living with rare neuroendocrine diseases, today reported financial results and provided a business update for the first quarter ended March 31, 2026.

"The U.S and European approvals of IMCIVREE mark a transformational milestone for patients with acquired hypothalamic obesity (HO) who, until now, had no approved therapies for this devastating disease," said David Meeker, M.D., Chairman, Chief Executive Officer and President of Rhythm Pharmaceuticals. "The U.S. launch is off to a strong start, as patient demand and physician engagement in this early stage of the launch reinforce the significant unmet need and long-term opportunity in acquired HO. With European Commission authorization for acquired HO earlier than expected, we look forward to engaging with country-level officials with launches anticipated in 2027. We are progressing rapidly in Japan for acquired HO, as well; regulatory review is underway, and if approved, we anticipate launching there in the second half of 2026."

Dr. Meeker added, "In addition, we continue to advance our MC4R agonism pipeline with upcoming data readouts from ongoing trials of setmelanotide in Prader-Willi syndrome and our weekly injectable, RM-718, in acquired HO. Our ongoing global commercial efforts and clinical development pipeline position Rhythm to achieve long-term, sustained growth."

Recent Business and Development Highlights

- Today, the Company announced that more than 150 patient start forms had been received for IMCIVREE in acquired HO, within the first six weeks following approval by the U.S. Food and Drug Administration (FDA) on March 19, 2026;
- Revenue from global sales of IMCIVREE was $60.1 million for the first quarter of 2026, an increase of 5% on a sequential basis from the fourth quarter of 2025, primarily driven by sales of IMCIVREE for the treatment of patients with Bardet-Biedl syndrome (BBS) and an increase in the number of patients on reimbursed therapy globally. In the first quarter of 2026, revenue of $36.9 million, or 61% of product revenue, was generated in the United States, a decrease of 5% on a sequential basis primarily driven by specialty pharmacy inventory and some patients receiving drug under the Company's bridging program as they transitioned insurance plans during the quarter. Revenue of $23.2 million, or 39% of product revenue, was generated outside the United States, a sequential increase of $4.9 million or 27%;
- Today, the Company announced positive data from the Japanese cohort of its Phase 3 TRANSCEND study and that its Japanese New Drug Application (JNDA) for setmelanotide to treat acquired HO has been accepted, validated and is now under review by Japan's Pharmaceuticals and Medical Devices Agency (PMDA). The Company anticipates PMDA's decision on the application in the second half of 2026 and, if positive, expects commercial launch by the end of 2026;
- On May 4, 2026, the Company announced new data presented at the Pediatric Endocrine Society Annual Meeting demonstrating sustained reductions in BMI and BMI Z-score through 2.5 years of setmelanotide treatment and observed weight category improvements in pediatric patients (n=10) with acquired HO and a second presentation demonstrating weight category improvement in the pediatric subpopulation of participants after 1 year of setmelanotide treatment;
- On May 1, 2026, the Company announced the European Commission granted Marketing Authorization to IMCIVREE for the treatment of obesity and control of hunger in patients with acquired HO, following the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) adoption of a positive opinion recommending expansion of the current marketing authorization to include the treatment of acquired HO on March 26, 2026;
- On April 3, 2026, the Company announced the appointment of Kim Popovits, the former Chairman of the Board, Chief

Executive Officer and President of Genomic Health, to Rhythm's Board of Directors and the resignation of Ed Mathers;

- On March 19, 2026, the Company announced IMCIVREE was approved by the FDA for the treatment of acquired HO;
- On March 16, 2026, Rhythm announced topline results from the Phase 3 EMANATE trial. The trial did not achieve the primary endpoint in each of its four independent substudies evaluating setmelanotide in genetically caused MC4R pathway diseases. The Company reported positive signals from post-hoc analyses in two genetic indications, SRC1 (NCOA1) deficiency and POMC insufficiency, and continues to explore a path forward with next-generation MC4R agonists bivamelagon and RM-718;
- On March 1, 2026, the Company announced additional positive data from its global Phase 3 TRANSCEND trial of setmelanotide in patients with acquired HO. This new data set included 12 patients from a Japanese cohort and 10 supplemental patients who were enrolled in addition to the primary 120-patient pivotal cohort; and
- On February 26, 2026, the Company announced it completed an End-of-Phase-2 meeting with FDA regarding bivamelagon in acquired HO and disclosed encouraging open-label extension data from its Phase 2 trial that showed bivamelagon achieved persistent BMI reductions at six and nine months of therapy.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Announce six-month results from the ongoing exploratory Phase 2 trial of setmelanotide in Prader-Willi Syndrome (PWS) in the second quarter of 2026;
- Announce results from the Phase 1/2, Part C trial evaluating the weekly, MC4R agonist RM-718 in patients with acquired HO mid-year 2026;
- Decision by Japan's PMDA for IMCIVREE in acquired HO anticipated in the second half of 2026;
- Complete enrollment in the setmelanotide substudy in congenital HO in the second half of 2026;
- Complete enrollment in the Phase 1/2, Part D trial evaluating RM-718 in PWS in the second half of 2026;
- Initiate a pivotal Phase 3 trial evaluating bivamelagon in acquired HO by year-end 2026.

First Quarter 2026 Financial Results

Cash Position: As of March 31, 2026, cash, cash equivalents and short-term investments were approximately $340.6 million, as compared to $388.9 million as of December 31, 2025.

Revenue: Net product revenues from global sales of IMCIVREE were $60.1 million for the first quarter of 2026, as compared to $37.7 million for the first quarter of 2025.

R&D Expenses: R&D expenses were $41.7 million in the first quarter of 2026, as compared to $37.0 million in the first quarter of 2025. The increase was primarily due to increased headcount which was partially offset by a net decrease in clinical trial expenses and a decrease in chemistry, manufacturing, and controls costs.

SG&A Expenses: SG&A expenses were $63.6 million for the first quarter of 2026, as compared to $39.1 million for the first quarter of 2025. The year-over-year increase was primarily due to increased headcount and an increase in marketing to support continued revenue growth.

Other expense, net: Other expense, net was $(2.7) million for the first quarter of 2026, as compared to other expense, net of $(2.4) million for the first quarter of 2025.

Net Loss: Net loss attributable to common stockholders was $(56.7) million for the first quarter of 2026, or a net loss per basic and diluted share of $(0.83), as compared to a net loss attributable to common stockholders of $(50.8) million for the first quarter of 2025, or a net loss per basic and diluted share of $(0.81).

Financial Guidance: For the year ending December 31, 2026, Rhythm anticipates approximately $385 million to $415 million in Non-GAAP Operating Expenses. Non-GAAP Operating Expenses are derived from:

- GAAP total operating expenses, inclusive of:
  - R&D expenses of approximately $197 million to $213 million;
  - SG&A expenses of approximately $188 million to $202 million; and
  - Excluding stock-based compensation.

Non-GAAP Operating Expenses is defined as GAAP operating expenses excluding stock-based compensation and fixed consideration related to in-licensing (see below under "Non-GAAP Financial Measures" for more details).

Based on its current operating plans, Rhythm expects that its cash, cash equivalents and short-term investments as of March 31,

2026 will be sufficient to fund the Company's planned operations for at least 24 months.

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 2026 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 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 rare neuroendocrine diseases. Rhythm's lead asset, IMCIVREE ® (setmelanotide), an MC4R agonist designed to treat hyperphagia and severe obesity, is approved by the U.S. Food and Drug Administration (FDA) to reduce excess body weight and maintain weight reduction long term in adult and pediatric patients aged 4 years and older with acquired hypothalamic obesity, adult and pediatric patients 2 years of age and older with syndromic or monogenic obesity due to Bardet-Biedl syndrome (BBS) or genetically confirmed pro-opiomelanocortin (POMC), including proprotein convertase subtilisin/kexin type 1 (PCSK1), deficiency or leptin receptor (LEPR) deficiency. 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 2 years of age and above. The European Commission (EC) has authorized setmelanotide for the treatment of obesity and control of hunger in patients 4 years of age and above with acquired hypothalamic obesity. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare diseases, as well as investigational MC4R agonists bivamelagon and RM-718, and 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 to reduce excess body weight and maintain weight reduction long term in adult and pediatric patients aged 4 years and older with acquired HO, and in adult and pediatric patients aged 2 years and older with syndromic or monogenic obesity due to Bardet-Biedl syndrome (BBS) or Pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (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).

In the European Union and the United Kingdom, setmelanotide is indicated for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 2 years of age and above. In the European Union and the United Kingdom, setmelanotide should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

Limitations of Use

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 BBS or POMC, PCSK1, or LEPR deficiency, including obesity associated with other genetic syndromes and general (polygenic) obesity

Contraindication

Prior serious hypersensitivity to setmelanotide or any of the excipients in IMCIVREE. Serious hypersensitivity reactions (e.g., anaphylaxis) have been reported.

WARNINGS AND PRECAUTIONS

Disturbance in Sexual Arousal: Spontaneous penile erections in males and sexual adverse reactions in females have occurred. Inform patients that these events may occur and instruct patients who have an erection lasting longer than 4 hours to seek emergency medical attention.

Depression and Suicidal Ideation: Depression, suicidal ideation and depressed mood have occurred. Monitor patients for new onset or worsening depression or suicidal thoughts or behaviors. Consider discontinuing IMCIVREE if patients experience suicidal thoughts or behaviors, or clinically significant or persistent depression symptoms occur.

Hypersensitivity Reactions: Serious hypersensitivity reactions (e.g., anaphylaxis) have been reported. If suspected, advise patients to promptly seek medical attention and discontinue IMCIVREE.

Skin Hyperpigmentation, Darkening of Pre-existing Nevi, and Development of New Melanocytic Nevi: Generalized or focal increases in skin pigmentation, darkening of pre-existing nevi, development of new melanocytic nevi and increase in size of existing melanocytic nevi have occurred. Perform a full body skin examination prior to initiation and periodically during treatment to monitor pre-existing and new pigmented 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. Serious and fatal adverse reactions including "gasping syndrome" can occur in neonates and low birth weight infants treated with benzyl alcohol preserved drugs.

ADVERSE REACTIONS

Most common adverse reactions (incidence ≥20%) included skin hyperpigmentation, injection site reactions, nausea, headache, diarrhea, abdominal pain, vomiting, depression, and spontaneous penile erection.

USE IN SPECIFIC POPULATIONS

Treatment with IMCIVREE is not recommended when breastfeeding. Discontinue IMCIVREE when pregnancy is recognized unless the benefits of therapy outweigh the potential risks to the fetus.

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 section 4.8 of the Summary of Product Characteristics for information on reporting suspected adverse reactions in Europe.

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 safety, efficacy, potential benefits of, and clinical design or progress of any of our products or product candidates at any dosage or in any indication, including, setmelanotide, bivamelagon, and RM-718; the use of setmelanotide in patients with acquired HO and the success of our commercial launch; our expectations surrounding potential regulatory submissions, progress, or approvals and timing thereof for any of our product candidates, including the review and anticipated decision by Japan's PMDA and potential marketing approval in Japan and launches in the European Union and the timing thereof; the commercial growth of IMCIVREE; the estimated market size and addressable population for our drug products, including setmelanotide for the treatment of acquired HO; the future announcement of data from our ongoing clinical trials, including the substudy evaluating setmelanotide for patients with congenital hypothalamic obesity, Part C of the Phase 1 trial evaluating RM-718, and the open-label Phase 2 trial evaluating setmelanotide, in patients with PWS and the ongoing enrollment in our clinical trials; existing or future collaboration agreements; the Company's business strategy and plans; our anticipated financial performance and financial position for any period of time, including our estimated Non-GAAP Operating Expenses for the year ending December 31, 2026; and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations for at least 24 months; and the timing of any of the foregoing. Statements using words 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, 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, 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, our ability to successfully commercialize setmelanotide, our liquidity and expenses, our ability to retain our key employees and consultants, and to attract, retain and motivate qualified personnel, and general economic conditions, and the other important factors, including those discussed under the caption "Risk Factors" in Rhythm's Quarterly Report on Form 10-Q for the quarter ended March 31, 2026 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 press release or to update them to reflect events or circumstances occurring after the date of this press 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 and fixed consideration related to in-licensing.

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,	
	2026	**2025**
Revenues:		
Product revenue, net	$ 60,112	$ 37,718
License revenue	—	(5,014)
Total revenues	60,112	32,704
Costs and expenses:		
Cost of sales	7,157	3,648
Research and development	41,725	36,973
Selling, general, and administrative	63,591	39,087
Total costs and expenses	112,473	79,708
Loss from operations	(52,361)	(47,004)
Other income (expense):		
Other income (expense), net	(1,704)	(644)
Interest expense	(4,583)	(5,409)
Interest income	3,554	3,639
Total other income (expense), net	(2,733)	(2,414)
Loss before income taxes	(55,094)	(49,418)
Provision (benefit) for income taxes	545	80
Net loss	$ (55,639)	$ (49,498)
Accrued dividends on convertible preferred stock	(1,104)	(1,322)
Net loss attributable to common stockholders	$ (56,743)	$ (50,820)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.83)	$ (0.81)
Weighted-average common shares outstanding, basic and diluted	67,974,193	63,059,165
Net loss	$ (55,639)	$ (50,820)
Other comprehensive income (loss):		
Foreign currency translation adjustment	1,784	(2)
Unrealized gain (loss), net on marketable securities	$ (544)	(10)
Comprehensive loss	$ (54,399)	$ (50,832)

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

	March 31, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 62,137	$ 54,301
Short-term investments	278,488	334,648
Accounts receivable, net	34,192	26,081
Inventory	28,651	25,753
Prepaid expenses and other current assets	26,622	26,133
Total current assets	430,090	466,916
Property and equipment, net	1,065	1,104
Right-of-use asset	2,936	3,049
Intangible assets, net	5,106	5,319
Restricted cash	601	522
Other long-term assets	2,518	3,286
Total assets	$ 442,316	$ 480,196
Liabilities, Convertible Preferred Stock and Stockholders' equity		
Current liabilities:		
Accounts payable	$ 12,299	$ 13,947
Accrued expenses and other current liabilities	80,045	83,855
Lease liability	676	650
Deferred revenue	48	194
Deferred royalty obligation, current	10,130	7,296
Total current liabilities	103,198	105,942
Long-term liabilities:		
Deferred royalty obligation	98,339	100,886
Lease liability, non-current	3,163	3,342
Total liabilities	204,700	210,170
Commitments and contingencies (Note 14)		
Series A convertible preferred stock, $0.001 par value: 150,000 shares authorized; 115,000 and 132,500 shares issued and outstanding at March 31, 2026 and December 31, 2025, respectively. Liquidation preference of $115,000 and $132,500 as of March 31, 2026, and December 31, 2025, respectively.	114,710	130,957
Stockholders' equity:		
Preferred stock, $0.001 par value: 9,850,000 shares authorized; no shares issued and outstanding at March 31, 2026 and December 31, 2025	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 68,445,084 and 67,205,321 shares issued and outstanding at March 31, 2026 and December 31, 2025, respectively	69	67
Additional paid-in capital	1,529,909	1,491,675
Accumulated other comprehensive income (loss)	444	(796)
Accumulated deficit	(1,407,516)	(1,351,877)
Total stockholders' equity	122,906	139,069
Total liabilities, convertible preferred stock and stockholders' equity	$ 442,316	$ 480,196

Source: Rhythm Pharmaceuticals, Inc.