



Rhythm Pharmaceuticals Reports First Quarter 2025 Financial Results and Business Update

May 7, 2025

-- First quarter 2025 net product revenue from global sales of IMCIVREE® (setmelanotide) of \$37.7 million --

-- Setmelanotide pivotal Phase 3 TRANSCEND trial met primary endpoint with -19.8% placebo-adjusted BMI reduction in patients (N=120) with acquired hypothalamic obesity --

-- U.S. and EU regulatory submissions for setmelanotide in acquired hypothalamic obesity on track to be completed in the third quarter of 2025 --

-- Topline data from Phase 2 trial of oral MC4R agonist bivamelagon on track to be announced in third quarter of 2025 --

-- Cash on-hand expected to support planned operations into 2027 --

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

BOSTON, May 07, 2025 (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, 2025.

"Following compelling topline results from our pivotal Phase 3 trial of setmelanotide in acquired hypothalamic obesity (HO), we look forward to completing U.S. and EU regulatory submissions in the third quarter of 2025 and, pending approval, bringing the first-ever known approved therapy for this serious disease to patients," said David Meeker, M.D., Chairman, Chief Executive Officer and President of Rhythm. "In addition, we are advancing our next-generation MC4R agonists as we are on track to read out topline data from the Phase 2 trial evaluating the oral-daily bivamelagon in patients with HO in the third quarter of 2025."

Dr. Meeker continued, "With BBS, we are pleased with the continued growth in the number of patients on reimbursed therapy with a steady increase in new physicians writing prescriptions in the United States along with the ongoing strong performance internationally. In addition, we believe Rhythm is well capitalized with cash runway into 2027, beyond multiple clinical and regulatory milestones expected this year and early next year."

First Quarter and Recent Business Highlights

- Revenue from global sales of IMCIVREE (setmelanotide) was \$37.7 million for the first quarter of 2025. The number of patients on reimbursed therapy increased 14% in the first quarter of 2025 compared to the fourth quarter of 2024, as patient demand for IMCIVREE remained strong. Revenue of \$24.5 million, or 65% of product revenue, was generated in the United States. The number of patients on reimbursed therapy in the United States continued to increase during the quarter. U.S. revenue was affected by an \$8.3 million decrease in inventory at the specialty pharmacy that dispenses IMCIVREE to patients and a \$1.1 million increase in product dispensed to patients, resulting in a net decrease in U.S. product revenue of \$7.2 million in the first quarter of 2025 compared to the fourth quarter of 2024. Revenue of \$13.2 million, or 35% of product revenue, was generated outside the United States, an increase of \$3.2 million quarter over quarter.
- On April 7, 2025, Rhythm announced its pivotal Phase 3 TRANSCEND trial met its primary endpoint with a -19.8% placebo-adjusted body mass index (BMI) reduction with setmelanotide in patients (N=120) with acquired HO;
 - Patients with acquired HO on setmelanotide therapy (n=81) achieved mean BMI change of -16.5% compared with +3.3% for placebo (n=39) at 52 weeks (p<0.0001);
 - -19.2% placebo-adjusted BMI reduction achieved in adult patients 18 years old and older (n=49) at 52 weeks;
 - -20.2% placebo-adjusted BMI reduction achieved in patients younger than 18 years old (n=71) at 52 weeks;
 - 80% of patients on setmelanotide achieved BMI reduction of 5% or greater at 52 weeks; and
 - No new safety signals with setmelanotide were observed, in line with setmelanotide's well-established and well-understood safety profile.
- Today, Rhythm announced new data from the Phase 3 TRANSCEND trial that demonstrated a consistent and statistically significant mean BMI reduction across three stratified age groups:
 - -19.5% placebo-adjusted BMI reduction achieved in pediatric patients ages 4 to younger than 12 years old (n=31: 20 setmelanotide, 11 placebo) at 52 weeks (p<0.0001);

- -21.0% placebo-adjusted BMI reduction achieved in adolescent patients ages 12 to younger than 18 years old (n=40: 28 setmelanotide, 12 placebo) at 52 weeks (p<0.0001); and
- -19.2% placebo-adjusted BMI reduction achieved in adult patients ages 18 and older (n=49: 33 setmelanotide, 16 placebo) at 52 weeks (p<0.0001).
- On April 7, 2025, Rhythm announced it dosed the first patients with Prader-Willi syndrome (PWS) in a 26-week, open-label Phase 2 trial of setmelanotide. The trial will assess the safety and efficacy of a daily dose of subcutaneous setmelanotide in approximately 20 patients for up to 26 weeks;
- On March 20, 2025, Rhythm announced it reacquired the rights to IMCIVREE in China, including mainland China, Hong Kong and Macau, as the Company agreed to terminate its 2021 licensing agreement with RareStone Group Ltd.
- On March 19, 2025, Rhythm announced it received orphan drug designation from Japan's Ministry of Health, Labour and Welfare (MHLW) for setmelanotide as a treatment for acquired hypothalamic obesity; and
- On March 18, 2025, Rhythm announced a new research collaboration with the Raymond A. Wood Foundation, a patient advocacy organization for survivors of craniopharyngioma and hypothalamic-pituitary brain tumors, to study the impact of fatigue on persons with craniopharyngioma.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Submit a supplemental New Drug Application to the FDA and a Type II variation request to the European Medicines Agency for setmelanotide for the treatment of patients with acquired HO in the third quarter of 2025;
- Announce topline data from the bivamelagon Phase 2 trial in acquired HO in the third quarter of 2025;
- Complete enrollment in the setmelanotide Phase 2 trial in PWS in the second half of 2025;
- Complete enrollment in the Phase 1, Part C trial evaluating the weekly, MC4R agonist RM-718 in patients with acquired HO in the second half of 2025;
- Complete enrollment in the setmelanotide Phase 3 trial substudy in congenital HO in the second half of 2025;
- Announce topline data in the 12-patient Japanese cohort of the setmelanotide Phase 3 trial in acquired HO in the first quarter of 2026; and
- Announce topline data in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in the first quarter of 2026.

First Quarter 2025 Financial Results:

Cash Position: As of March 31, 2025, cash, cash equivalents and short-term investments were approximately \$314.5 million, as compared to \$320.6 million as of December 31, 2024.

Revenue: Net product revenues relating to global sales of IMCIVREE were \$37.7 million for the first quarter of 2025, as compared to \$26.0 million for the first quarter of 2024. License revenue for the first quarter of 2025 was (\$5.0) million, a reduction entirely due to the termination of an exclusive license agreement with RareStone Group Ltd. and the repayment by Rhythm of a portion of previously-recognized license revenue.

R&D Expenses: R&D expenses were \$37.0 million in the first quarter of 2025, as compared to \$128.7 million in the first quarter of 2024. The decrease was primarily due to the R&D expenses incurred in the first quarter of 2024 related to in-process research and development costs totaling \$92.4 million associated with the acquisition of LG Chem's proprietary compound bivamelagon, which did not recur in 2025.

SG&A Expenses: SG&A expenses were \$39.1 million for the first quarter of 2025, as compared to \$34.4 million for the first quarter of 2024. 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 \$2.4 million for the first quarter of 2025, as compared to other expense, net of \$1.2 million for the first quarter of 2024.

Net Loss: Net loss attributable to common stockholders was (\$50.8) million for the first quarter of 2025, or a net loss per basic and diluted share of (\$0.81), as compared to a net loss attributable to common stockholders of (\$141.4) million for the first quarter of 2024, or a net loss per basic and diluted share of (\$2.35).

Financial Guidance: For the year ending December 31, 2025, Rhythm anticipates approximately \$285 million to \$315 million in Non-GAAP Operating Expenses. Non-GAAP Operating Expenses are derived from:

- GAAP total operating expenses, inclusive of:
 - SG&A expenses of approximately \$135 million to \$145 million;
 - R&D expenses of approximately \$150 million to \$170 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 existing cash, cash equivalents and short-term investments as of March 31, 2025, will be sufficient to fund its operating expenses and capital expenditure requirements into 2027.

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 2025 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 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. 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 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 “gaspings syndrome” can occur in neonates and low birth weight infants treated with benzyl alcohol preserved drugs.

ADVERSE REACTIONS

Most common adverse reactions (incidence $\geq 20\%$) 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, bivamelaon, and RM-718; the potential use of setmelanotide in patients with acquired hypothalamic obesity; the commercial growth of IMCIVREE; our expectations surrounding potential regulatory submissions, progress, or approvals and timing thereof for any of our product candidates, including the anticipated supplemental New Drug Application to the FDA and a Type II variation request to the European Medicines Agency; the estimated market size and addressable population for our drug products, including setmelanotide for the treatment of hypothalamic obesity; the future announcement of data from our ongoing clinical trials, including the Japanese cohort of our Phase 3 trial evaluating setmelanotide for patients with acquired hypothalamic obesity, the substudy evaluating setmelanotide for patients with congenital hypothalamic obesity, the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases, and the Phase 2 trial evaluating the oral MC4R agonist bivamelaon in acquired hypothalamic obesity; Part C of the Phase 1 trial evaluating RM-718; the open-label Phase 2 trial evaluating setmelanotide in patients with Prader-Willi syndrome; the ongoing enrollment in our clinical trials; the Company’s business strategy and plans; our anticipated financial performance and financial position for any period of time, including estimated Non-GAAP Operating Expenses for the year ending December 31, 2025; and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations; and the timing of any of the foregoing. Statements using words 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, 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 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, general economic conditions, risks related to internal control over financial reporting, and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025 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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Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 106,095	\$ 89,137
Short-term investments	208,393	231,428
Accounts receivable, net	17,825	18,512
Inventory	19,316	18,741
Prepaid expenses and other current assets	17,659	16,382
Total current assets	369,288	374,200
Property and equipment, net	462	632
Right-of-use asset	3,367	3,477
Intangible assets, net	5,960	6,174
Restricted cash	464	464
Other long-term assets	7,144	7,326
Total assets	\$ 386,685	\$ 392,273
Liabilities, Convertible Preferred Stock and Stockholders' equity		
Current liabilities:		
Accounts payable	\$ 11,912	\$ 12,328
Accrued expenses and other current liabilities	59,314	62,658
Other current liability - LG Chem	38,783	37,704
Lease liability	224	—
Deferred revenue	—	1,286
Deferred royalty obligation, current	1,590	1,541
Total current liabilities	111,823	115,517
Long-term liabilities:		
Deferred royalty obligation	107,934	108,269
Lease liability, non-current	3,839	3,938
Total liabilities	223,596	227,724
Commitments and contingencies (Note 13)		

Series A convertible preferred stock, \$0.001 par value: 150,000 shares authorized; 150,000 and 0 shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively. Liquidation preference of \$150,000 as of March 31, 2025.	144,142	142,820
Stockholders' equity:		
Preferred stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at March 31, 2025 and December 31, 2024	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 63,494,892 and 62,390,654 shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively	62	61
Additional paid-in capital	1,223,772	1,177,045
Accumulated other comprehensive (loss)	(51)	(39)
Accumulated deficit	(1,204,836)	(1,155,338)
Total stockholders' equity	18,947	21,729
Total liabilities, convertible preferred stock and stockholders' equity	\$ 386,685	\$ 392,273

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,	
	2025	2024
Revenues:		
Product revenue, net	\$ 37,718	\$ 25,967
License revenue	(5,014)	—
Total revenues	32,704	25,967
Costs and expenses:		
Cost of sales	3,648	2,807
Research and development	36,973	128,665
Selling, general, and administrative	39,087	34,382
Total costs and expenses	79,708	165,854
Loss from operations	(47,004)	(139,887)
Other income (expense):		
Other income (expense), net	(644)	524
Interest expense	(5,409)	(4,755)
Interest income	3,639	3,046
Total other (expense), net	(2,414)	(1,185)
Loss before income taxes	(49,418)	(141,072)
Provision for income taxes	80	300
Net loss	\$ (49,498)	\$ (141,372)
Accrued dividends on convertible preferred stock	(1,322)	—
Net loss attributable to common stockholders	\$ (50,820)	\$ (141,372)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.81)	\$ (2.35)
Weighted-average common shares outstanding, basic and diluted	63,059,165	60,143,558
Other comprehensive loss:		
Net loss attributable to common stockholders	\$ (50,820)	\$ (141,372)
Foreign currency translation adjustment	(2)	(71)
Unrealized (loss), net on marketable securities	(10)	(244)
Comprehensive loss	\$ (50,832)	\$ (141,687)



Source: Rhythm Pharmaceuticals, Inc.