Rhythm Pharmaceuticals Reports Fourth Quarter and Full Year 2025 Financial Results and Business Update

February 26, 2026

-- Fourth quarter 2025 net product revenue from global sales of IMCIVREE® (setmelanotide) of $57.3 million --

-- March 20, 2026 PDUFA goal date for sNDA for setmelanotide in acquired hypothalamic obesity (HO) --

-- Phase 2 open-label extension data showed bivamelagon achieved persistent BMI reductions at six and nine months; Completed positive end-of-Phase-2 meeting with FDA --

-- On track to report topline data from 12-patient Japanese cohort of setmelanotide Phase 3 trial in acquired HO in March 2026 --

-- On track to report topline data from Phase 3 EMANATE trial evaluating setmelanotide in rare genetically caused melanocortin-4 receptor (MC4R) pathway diseases in March 2026 --

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

BOSTON, Feb. 26, 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 fourth quarter and full year ended December 31, 2025.

“Rhythm delivered solid IMCIVREE global sales growth and made continued progress developing therapies to address hyperphagia and severe obesity for people with rare MC4R pathway diseases in 2025,” said David Meeker, M.D., Chairman, Chief Executive Officer and President of Rhythm. “We enter 2026 focused on long-term growth and well-capitalized to achieve important commercial and clinical milestones. We are prepared to bring IMCIVREE to patients with acquired HO in the United States, pending FDA approval."

He added, “We continue to advance clinical development of our next-generation of MC4R pathway agonists to improve patients’ lives. Following a highly constructive end-of-phase-2 meeting with the FDA where we shared encouraging open-label extension data focused on our oral MC4R agonist bivamelagon, we remain on track to initiate a Phase 3 trial evaluating bivamelagon in acquired HO by year-end 2026.”

Recent Business Highlights

- Revenue from global sales of IMCIVREE was $57.3 million for the fourth quarter of 2025, an increase of 12% on a sequential basis from the third 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 fourth quarter of 2025, revenue of $39.0 million, or 68% of product revenue, was generated in the United States, an increase of 2% on a sequential basis. Revenue of $18.3 million, or 32% of product revenue, was generated outside the United States, a sequential increase of $5.2 million or 40%. The sequential increase in ex-US revenue was mostly due to a one-time, $3.2 million charge, recorded during the third quarter of 2025 following an agreement between Rhythm and the French Economic Committee for Health Products (CEPS) on the final reimbursed price for IMCIVREE for BBS and pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiencies, to account for the difference between what had been accrued to date and what was then owed as a result of the final agreement with CEPS, as previously announced on November 4, 2025.
- On November 5, 2025, Rhythm announced that it entered into Product Listing Agreements in Canada in the provinces of Ontario, Alberta, British Columbia, Saskatchewan, Nova Scotia and with the Federal Non-Insured Health Benefits (NIHB) Program for the public reimbursement of IMCIVREE for weight management in eligible adult and pediatric patients aged 6 years and older with clinically or genetically confirmed BBS and obesity.

Recent Clinical Development Highlights

- On February 26, the Company announced it completed a positive 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;
- On December 11, 2025, Rhythm announced positive preliminary results from its exploratory Phase 2 trial of setmelanotide

in patients with PWS. Setmelanotide demonstrated BMI and hyperphagia reductions at month 3 and month 6, as well as safety and tolerability consistent with setmelanotide's well-established clinical profile;
- On December 11, 2025, the Company announced that it has initiated a Part D arm in the Phase 1/2 trial of MC4R agonist RM-718 that will enroll up to 20 patients with PWS; and
- During ObesityWeek® 2025 in November 2025, Rhythm and its partners delivered three presentations including data from the Company's pivotal Phase 3 TRANSCEND trial evaluating setmelanotide in acquired HO, the largest randomized, placebo-controlled trial in acquired HO to date. Highlights of the presentations included:
    - Setmelanotide achieved significant BMI reductions in patients with acquired HO on concomitant treatment with GLP-1 therapy;
    - The results from interviews with 14 trial participants and 16 caregivers of trial participants younger than 12 showed that patients consistently reported meaningful and beneficial changes in hunger, weight, energy levels, and physical activity with setmelanotide therapy; and
    - Setmelanotide treatment was associated with significant improvement across most cardiometabolic parameters and proteomic biomarkers. Results showed improvements across nonambulatory blood pressure, lipid levels, and hematologic and chemistry parameters.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Launch IMCIVREE in the United States for the treatment of acquired HO pending FDA approval; the FDA's assigned PDUFA goal date is March 20, 2026;
- Complete enrollment in the Phase 1/2, Part C trial evaluating the weekly, MC4R agonist RM-718 in patients with acquired HO in the first quarter of 2026;
- Announce topline data in the 12-patient Japanese cohort of the setmelanotide Phase 3 trial in acquired HO in March 2026;
- Announce topline data in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in March 2026;
- Announce six-month results from the ongoing exploratory Phase 2 trial of setmelanotide in PWS in the first half of 2026;
- Complete enrollment in the setmelanotide Phase 3 trial substudy in congenital HO in the second half of 2026;
- Initiate a pivotal Phase 3 trial evaluating bivamelagon in acquired HO by year-end 2026;
- Complete enrollment in the Phase 1/2, Part D trial evaluating RM-718 in PWS in the second half of 2026.

Fourth Quarter and Full Year 2025 Financial Results:

Cash Position: As of December 31, 2025, cash, cash equivalents and short-term investments were approximately $388.9 million, as compared to $320.6 million as of December 31, 2024.

Revenue: Net product revenues relating to global sales of IMCIVREE were $57.3 million for the fourth quarter of 2025 and $194.8 million for the full year of 2025, as compared to $41.8 million for the fourth quarter of 2024 and $130.1 million for the full year of 2024.

R&D Expenses: R&D expenses were $42.0 million in the fourth quarter of 2025 and $167.3 million for the full year of 2025, as compared to $41.2 million in the fourth quarter of 2024 and $238.0 million for the full year of 2024. The year-over-year decrease was primarily due to a decrease in acquired In-Process Research and Development ("IPR&D") costs associated with the acquisition of LG Chem, Ltd.'s proprietary compound bivamelagon in the year 2024, which did not recur in 2025 and a decrease in clinical trial costs.

SG&A Expenses: SG&A expenses were $57.5 million for the fourth quarter of 2025 and $194.9 million for the full year of 2025, as compared to $38.1 million for the fourth quarter of 2024 and $144.3 million for the full year of 2024. The year-over-year increase was primarily due to higher costs associated with additional headcount to support expanding business operations and to establish commercial operations in international regions, increased marketing and promotion costs and increased professional services costs.

Other income (expense), net: Other income (expense), net was $(0.5) million for the fourth quarter of 2025 and $(4.0) million for the full year of 2025.

Net Loss: Net loss attributable to common stockholders was $48.8 million for the fourth quarter of 2025 and $201.9 million for the full year of 2025, or a net loss per basic and diluted share of $(0.73) and $(3.11), respectively, as compared to a net loss attributable to common stockholders of $44.6 million for the fourth quarter of 2024 and $264.6 million for the full year of 2024, or a net loss per basic and diluted share of $(0.72) and $(4.34), respectively.

Financial Guidance: For the year ended December 31, 2025, the Company had GAAP total operating expenses of $362.3 million. The Company today reported non-GAAP Operating Expenses for the year ended December 31, 2025 of $295.5 million, which is derived from GAAP total operating expenses less $66.8 million in stock-based compensation.

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:
  - SG&A expenses of approximately $197 million to $213 million;
  - R&D 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 December 31, 2025 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 fourth quarter and full year 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 "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 potential use of setmelanotide in patients with acquired hypothalamic obesity; our expectations surrounding potential regulatory submissions, progress, or approvals and timing thereof for any of our product candidates, including the March 20, 2026 PDUFA goal date for our sNDA for setmelanotide in acquired hypothalamic obesity; the commercial growth of IMCIVREE; 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; Part C of the Phase 1 trial evaluating RM-718, and the open-label Phase 2 trial evaluating setmelanotide in patients with PWS; 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 Annual Report on Form 10-K for the year ended December 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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Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Revenues:				
Product revenue, net	$ 57,253	$ 41,830	$ 194,771	$ 130,126
License revenue	—	—	(5,014)	—
Total revenues	57,253	41,830	189,757	130,126
Costs and expenses:				
Cost of sales	4,802	3,787	19,492	13,368
Research and development	42,033	41,168	167,340	237,957
Selling, general, and administrative	57,481	38,130	194,941	144,304
Total costs and expenses	104,316	83,085	381,773	395,629
Loss from operations	(47,063)	(41,255)	(192,016)	(265,503)
Other income (expense):				
Other income (expense), net	57	(195)	1,264	2,239
Gain on settlement of forward contract	—	—	—	8,900
Interest expense	(4,615)	(5,447)	(20,583)	(20,603)
Interest income	4,085	3,514	15,293	14,711
Total other income (expense), net	(473)	(2,128)	(4,026)	5,247
Loss before income taxes	(47,536)	(43,383)	(196,042)	(260,256)
Provision for income taxes	(31)	(89)	497	346
Net loss	$ (47,505)	$ (43,294)	$ (196,539)	$ (260,602)
Accrued dividends on convertible preferred stock	(1,332)	(1,340)	(5,378)	(3,970)
Net loss attributable to common stockholders	$ (48,837)	$ (44,634)	$ (201,917)	$ (264,572)

Net loss per share attributable to common stockholders, basic and diluted	$ (0.73)	$ (0.72)	$ (3.11)	$ (4.34)
Weighted-average common shares outstanding, basic and diluted	66,876,883	61,596,442	64,984,361	60,995,204
Other comprehensive loss:				
Net loss attributable to common stockholders	$ (48,837)	$ (44,634)	$ (201,917)	(264,572)
Foreign currency translation adjustment	11	977	(1,261)	2
Unrealized (loss) gain, net on marketable securities, net of tax	119	(412)	504	(175)
Comprehensive loss	$ (48,707)	$ (44,069)	$ (202,674)	$ (264,745)

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

	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 54,301	$ 89,137
Short-term investments	334,648	231,428
Accounts receivable, net	26,081	18,512
Inventory	25,753	18,741
Prepaid expenses and other current assets	26,133	16,382
Total current assets	466,916	374,200
Property and equipment, net	1,104	632
Right-of-use asset	3,049	3,477
Intangible assets, net	5,319	6,174
Restricted cash	522	464
Other long-term assets	3,286	7,326
Total assets	$ 480,196	$ 392,273
Liabilities, Convertible Preferred Stock and Stockholders' equity		
Current liabilities:		
Accounts payable	$ 13,947	$ 12,328
Accrued expenses and other current liabilities	83,855	62,658
Other current liability - LG Chem	—	37,704
Deferred revenue	194	1,286
Deferred royalty obligation, current	7,296	1,541
Lease liability	650	—
Total current liabilities	105,942	115,517
Long-term liabilities:		
Deferred royalty obligation	100,886	108,269
Lease liability, non-current	3,342	3,938
Derivative liability	—	—
Total liabilities	210,170	227,724
Commitments and contingencies (Note 12)		
Series A convertible preferred stock, $0.001 par value: 150,000 shares authorized; 132,500 and 150,000 shares issued and outstanding at December 31, 2025 and December 31, 2024, respectively. Liquidation preference of $132,500 and $150,000 as of December 31, 2025, and December 31, 2024, respectively.	130,957	142,820
Stockholders' equity:		

Common stock, $0.001 par value: 120,000,000 shares authorized; 67,205,321 and 62,390,654 shares issued and outstanding at December 31, 2025 and December 31, 2024, respectively	67	61
Additional paid-in capital	1,491,675	1,177,045
Accumulated other comprehensive (loss) income	(796)	(39)
Accumulated deficit	(1,351,877)	(1,155,338)
Total stockholders' equity	139,069	21,729
Total liabilities, convertible preferred stock and stockholders' equity	$ 480,196	$ 392,273

Source: Rhythm Pharmaceuticals, Inc.