Rhythm Pharmaceuticals Announces Preliminary, Unaudited Fourth Quarter and Full Year 2025 Net Product Revenues and Upcoming Milestones

January 9, 2026

-- Q4 2025 preliminary net product revenues from global sales of IMCIVREE® (setmelanotide) of approximately $57 million for the fourth quarter of 2025, an 11% increase over Q3 2025 --

-- FY 2025 preliminary net product revenue of approximately $194 million, approximately 50% increase from FY2024 --

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

-- On track to report topline data from 12-patient Japanese cohort of setmelanotide Phase 3 trial in acquired hypothalamic obesity in first quarter of 2026 --

-- On track to report topline data from Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in first quarter of 2026 --

BOSTON, Jan. 09, 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 announced preliminary unaudited net product revenues from global sales of IMCIVREE® (setmelanotide) for the fourth quarter and full year of 2025 and upcoming milestones.

“2025 was a year of strong execution and reflects significant progress toward our mission of transforming the lives of patients with rare neuroendocrine diseases,” said David Meeker, M.D., Chairman, Chief Executive Officer and President of Rhythm. “Our preliminary fourth quarter and full-year 2025 net product revenues reflect consistent growth in both the United States and international markets, driven by a steady increase in patients on reimbursed therapy and continued progress in securing access to IMCIVREE.”

Dr. Meeker continued, “Looking ahead, we are focused on delivering sustainable, long-term growth as we prepare to launch IMCIVREE for patients with acquired hypothalamic obesity (HO) in the United States, pending FDA approval. Additionally in 2026, we are excited about top-line data readouts from the Japanese cohort of our Phase 3 trial in acquired HO and the Phase 3 EMANATE trial, initiating a Phase 3 trial to evaluate our oral MC4R agonist, bivamelagon, in acquired HO and advancing setmelanotide and RM-718 for patients with Prader-Willi syndrome.”

Preliminary Unaudited Fourth Quarter and Full Year 2025 Net Product Revenues
Based on preliminary unaudited financial information, Rhythm expects net product revenues from global sales of IMCIVREE to be approximately $57 million for the fourth quarter of 2025, an increase of 11% percent on a sequential basis from the third quarter of 2025. Net product revenues for the full year of 2025 are expected to be approximately $194 million, compared to $130 million for the full year of 2024, an increase of approximately 50% year over year. U.S. sales of IMCIVREE contributed approximately 68% of fourth quarter preliminary unaudited net product revenues and approximately 69% of full-year 2025 preliminary unaudited net product revenues. The Company plans to report its fourth quarter and full year 2025 financial results in late February 2026.

Anticipated Upcoming Milestones

Setmelanotide

Acquired Hypothalamic Obesity (HO)

- Launch IMCIVREE in the United States for the treatment of acquired hypothalamic obesity pending FDA approval; the FDA’s assigned PDUFA goal date is March 20, 2026;
- Announce topline data in the 12-patient Japanese cohort of the setmelanotide Phase 3 trial in acquired HO in the first quarter of 2026.

Congenital HO

- Complete enrollment in the setmelanotide Phase 3 trial substudy in congenital HO in the first half of 2026.

Genetically Caused MC4R Pathway Diseases

- Announce topline data in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in the first quarter of 2026.

Prader-Willi Syndrome (PWS)

- In December 2025, Rhythm announced positive preliminary data for the exploratory phase 2 trial of setmelanotide in patients with PWS that showed BMI and hyperphagia reductions at month 3 and month 6, as well as safety and tolerability consistent with setmelanotide's well-established clinical profile. Rhythm anticipates announcing six-month results from 18 patients from the ongoing Phase 2 trial in the first half of 2026.

Bivamelagon

- Pending further feedback from U.S. and European regulatory agencies, initiate a pivotal Phase 3 trial evaluating bivamelagon in acquired HO in 2026.

RM-718

- Complete enrollment in the Phase 1, Part C trial evaluating the weekly, MC4R agonist RM-718 in patients with acquired HO in the first quarter of 2026.

Financial Disclosure Advisory
This release contains certain estimated preliminary financial results for the fourth quarter and fiscal year ended December 31, 2025. These estimates are based on the information available to the Company at this time. The Company's financial close process for the fourth quarter and full year 2025 is not yet complete and, as a result, actual results may vary from the estimated preliminary results presented here. The estimated preliminary financial results have not been audited or reviewed by the Company's independent registered public accounting firm. These estimates should not be viewed as a substitute for the Company's full interim or annual financial statements. Accordingly, you should not place undue reliance on this preliminary data.

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 http://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 our preliminary, unaudited revenues for the fourth quarter and full year 2025; 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; the commercial growth of IMCIVREE; our expectations surrounding potential regulatory submissions, progress, or approvals and timing thereof for any of our product candidates; 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 Prader-Willi syndrome; the ongoing enrollment in our clinical trials; 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 three months ended September 30, 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.

Corporate Contact:

David Connolly
Head of Investor Relations and Corporate Communications
Rhythm Pharmaceuticals, Inc.
dconnolly@rhythmtx.com

Media Contact:
Layne Litsinger
Real Chemistry
llitsinger@realchemistry.com

Source: Rhythm Pharmaceuticals, Inc.