Rhythm Pharmaceuticals Reports Fourth Quarter and Full Year 2024 Financial Results and Business Update

February 26, 2025

-- Fourth quarter 2024 net revenue from global sales of IMCIVREE® (setmelanotide) of $41.8 million --

-- On track to report topline data from global Phase 3 trial evaluating setmelanotide in acquired hypothalamic obesity in the second quarter of 2025 --

-- Completed enrollment in the Phase 2 trial of oral MC4R agonist bivamelagon in acquired hypothalamic obesity --

-- FDA approved expanded label for IMCIVREE to include children as young as 2 years old --

-- Raised approximately $75 million in gross proceeds under ATM equity offering program; Cash runway extended into 2027 --

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

BOSTON, Feb. 26, 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 fourth quarter and full year ended December 31, 2024.

“Rhythm delivered solid IMCIVREE global sales growth in the fourth quarter and is poised to drive continued growth in 2025,” said David Meeker, M.D., Chairman, Chief Executive Officer and President of Rhythm. “Our Phase 3 trial in acquired hypothalamic obesity (HO) remains on track as we expect to disclose topline data in the second quarter of 2025. We are well capitalized as we strengthened our balance sheet with approximately $75 million in gross proceeds raised under our ATM program, which extends our cash runway into 2027, enabling us to execute on multiple clinical milestones expected in the year ahead.”

Fourth Quarter and Recent Business Highlights

- Revenue from global sales of IMCIVREE was $41.8 million for the fourth quarter of 2024, an increase of 26% percent on a sequential basis from the third quarter of 2024, primarily driven by sales of IMCIVREE for the treatment of Bardet-Biedl syndrome (BBS). In the fourth quarter of 2024, revenue of $31.7 million, or 76% of product revenue, was generated in the United States, an increase of 36.2% on a sequential basis; revenue of $10.1 million, or 24% of product revenue, was generated outside the United States;
- Today, the Company announced that it raised approximately $75 million in gross proceeds from the sale of approximately 1.3 million shares of common stock at a weighted average price of $56.30 under its “at the market” equity offering program (the “ATM Program”) during the fourth quarter of 2024 and in January 2025;
- Today, Rhythm announced that it completed enrollment in the Phase 2 trial evaluating oral MC4R agonist bivamelagon (LB54640) in acquired hypothalamic obesity;
- Today, Rhythm announced a strategic partnership agreement with Trispera Pharma Solutions in Turkey;
- On January 10, 2025, Rhythm announced that it:
    - Completed enrollment in its supplemental, 12-patient Japanese cohort of the global Phase 3 trial evaluating setmelanotide in acquired HO. Data from this supplemental cohort will serve as the basis for a regulatory submission in Japan;
    - Completed enrollment in the Phase 3 EMANATE trial of setmelanotide, which is comprised of four substudies: SH2B1 (n=121); POMC and/or PCSK1 (n=79); SRC1 (n=73); and LEPR (n=23). The four-substudy design of this trial allows for independent data readouts and potential registration for each genetic cohort;
- On December 20, 2024, the Company announced that the U.S. Food and Drug Administration (FDA) approved an expanded indication for IMCIVREE to include children as young as 2 years old with syndromic or monogenic obesity due to BBS or genetically confirmed pro-opiomelanocortin (POMC), including proprotein convertase subtilisin/kexin type 1 (PCSK1), deficiency or leptin receptor (LEPR) deficiency;
- On December 3, 2024, Rhythm announced that the United Kingdom’s Medicines & Healthcare products Regulatory Agency (MHRA) has expanded the marketing authorization for IMCIVREE to include the treatment of obesity and control of hunger associated with genetically confirmed BBS or genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adult and pediatric patients as young as 2 years old and older; and
- During the annual meeting of the European Society for Paediatric Endocrinology in November 2024, Rhythm presented new, real-world data that showed four pediatric patients, two with acquired HO and two with congenital HO, achieved >5%

weight reduction at three months on setmelanotide. The Company announced plans for a new, 34-week substudy in the ongoing setmelanotide Phase 3 trial in 39 patients with congenital HO aged 4 years and older.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Begin enrolling the first patients with congenital HO in a 34-week substudy of setmelanotide being conducted under the protocol for its global Phase 3 trial in the first quarter of 2025;
- Begin enrolling the first patients with Prader-Willi syndrome (PWS) in a 26-week, open-label Phase 2 trial evaluating setmelanotide in the first quarter of 2025;
- Begin enrolling patients with acquired HO in Part C of the Phase 1 trial evaluating the weekly, MC4R agonist RM-718 in the first quarter of 2025;
- Announce top-line data in the Phase 3 trial evaluating setmelanotide in acquired HO in the second quarter of 2025;
- Announce topline data from the Phase 2 trial of bivamelagon in acquired HO in the second half of 2025; and
- Announce top-line data in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in the first half of 2026.

Fourth Quarter and Full Year 2024 Financial Results:

Cash Position: As of December 31, 2024, cash, cash equivalents and short-term investments were approximately $320.6 million, as compared to $275.8 million as of December 31, 2023. The December 31, 2024 cash balance does not include gross proceeds of approximately $34.7 million from the sale of stock under the Company's ATM program executed during January 2025.

Revenue: Net product revenues relating to global sales of IMCIVREE were $41.8 million for the fourth quarter of 2024 and $130.1 million for the full year of 2024, as compared to $24.2 million for the fourth quarter of 2023 and $77.4 million for the full year of 2023.

R&D Expenses: R&D expenses were $41.2 million in the fourth quarter of 2024 and $238.0 million for the full year of 2024, as compared to $29.9 million in the fourth quarter of 2023 and $135.0 million for the full year of 2023. The year-over-year increase was primarily due to increased costs associated with the acquisition of bivamelagon from LG Chem and its development with certain additional product manufacturing activity, ongoing clinical trials and increased headcount. This increase was partially offset by decreased costs in other trials.

SG&A Expenses: SG&A expenses were $38.1 million for the fourth quarter of 2024 and $144.3 million for the full year of 2024, as compared to $32.4 million for the fourth quarter of 2023 and $117.5 million for the full year of 2023. The year-over-year increase was primarily due to increased headcount, marketing and promotions costs and expenses for professional services and partially offset by inventory-related costs that were capitalized as labor and overhead.

Other income (expense), net: Other income (expense), net was ($2.1) million for the fourth quarter of 2024 and $5.2 million for the full year of 2024.

Net Loss: Net loss attributable to common stockholders was ($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, as compared to a net loss attributable to common stockholders of ($41.6) million for the fourth quarter of 2023 and ($184.7) million for the full year of 2023, or a net loss per basic and diluted share of ($0.70) and ($3.20), respectively.

Financial Guidance: For the year ended December 31, 2024, the Company had GAAP total operating expenses of $382.3 million, inclusive of $92.4 million associated with the acquisition of the rights to bivamelagon from LG Chem. The Company today reported non-GAAP Operating Expenses for the year ended December 31, 2024 of $250.2 million, which is derived from GAAP total operating expenses of $382.3 million less $39.7 million in stock-based compensation and $92.4 million associated with the acquisition of the rights to bivamelagon from LG Chem.

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 December 31, 2024, and including proceeds from the sale of stock through its ATM program during January 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 fourth quarter and full year 2024 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 LB54640 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; the estimated market size and addressable population for our drug products; the announcement of data from our clinical trials, including 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 bivamelagon 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; 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 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 Annual Report on Form 10-K for the year ended December 31, 2024 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.

Corporate Contact:
David Connolly
Head of Investor Relations and Corporate Communications
Rhythm Pharmaceuticals, Inc.
857-264-4280
dconnolly@rhythmtx.com

Media Contact:
Sheryl Seapy
Real Chemistry
(949) 903-4750
sseapy@realchemistry.com

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,	
	2024	**2023**	**2024**	**2023**
evenues:				
Product revenue, net	$ 41,830	$ 24,234	$ 130,126	$ 77,428
Total revenues	41,830	24,234	130,126	77,428
Costs and expenses:				
Cost of sales	3,787	3,233	13,368	9,302
Research and development	41,168	29,892	237,957	134,951
Selling, general, and administrative	38,130	32,374	144,304	117,532
Total costs and expenses	83,085	65,499	395,629	261,785
Loss from operations	(41,255)	(41,265)	(265,503)	(184,357)
Other income (expense):				
Other income (expense), net	(195)	39	2,239	190
Gain on settlement of forward contract	—	—	8,900	—
Interest expense	(5,447)	(4,018)	(20,603)	(13,892)
Interest income	3,514	3,819	14,711	13,945
Total other income (expense), net	(2,128)	(160)	5,247	243
Loss before income taxes	(43,383)	(41,425)	(260,256)	(184,114)
Provision for income taxes	(89)	196	346	564
Net loss	$ (43,294)	$ (41,621)	$ (260,602)	$ (184,678)
Accrued dividends on convertible preferred stock	(1,340)	—	(3,970)	—
Net loss attributable to common stockholders	$ (44,634)	$ (41,621)	$ (264,572)	$ (184,678)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.72)	$ (0.70)	$ (4.34)	$ (3.20)
Weighted-average common shares outstanding, basic and diluted	61,596,442	59,211,199	60,995,204	57,673,128
Other comprehensive loss:				
Net loss attributable to common stockholders	$ (44,634)	$ (41,621)	$ (264,572)	$ (184,678)
Foreign currency translation adjustment	977	76	2	(140)
Unrealized (loss) gain, net on marketable securities, net of tax	(412)	(175)	(175)	366
Comprehensive loss	$ (44,069)	$ (41,720)	$ (264,745)	$ (184,452)

Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 89,137	$ 60,081
Short-term investments	231,428	215,765
Accounts receivable, net	18,512	14,867
Inventory	18,741	8,624
Prepaid expenses and other current assets	16,382	8,931
Total current assets	374,200	308,268
Property and equipment, net	632	1,341
Right-of-use asset	3,477	781
Intangible assets, net	6,174	7,028
Restricted cash	464	328
Other long-term assets	7,326	14,999
Total assets	$ 392,273	$ 332,745
Liabilities, Convertible Preferred Stock and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 12,328	$ 4,885
Accrued expenses and other current liabilities	62,658	48,262
Other current liability - LG Chem	37,704	—
Deferred revenue	1,286	1,286
Deferred royalty obligation, current	1,541	—
Lease liability	—	770
Total current liabilities	115,517	55,203
Long-term liabilities:		
Deferred royalty obligation	108,269	106,143
Lease liability, non-current	3,938	490
Derivative liability	—	1,150
Total liabilities	227,724	162,986
Commitments and contingencies		
Series A convertible preferred stock, $0.001 par value: 150,000 shares authorized; 150,000 and 0 shares issued and outstanding at December 31, 2024 and December 31, 2023, respectively. Liquidation preference of $150,000 as of December 31, 2024.	142,820	—
Stockholders' equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized; 62,390,654 and 59,426,559 shares issued and outstanding at December 31, 2024 and December 31, 2023, respectively	61	59
Additional paid-in capital	1,177,045	1,064,302
Accumulated other comprehensive (loss) income	(39)	134
Accumulated deficit	(1,155,338)	(894,736)
Total stockholders' equity	21,729	169,759
Total liabilities, convertible preferred stock and stockholders' equity	$ 392,273	$ 332,745

Source: Rhythm Pharmaceuticals, Inc.