Rhythm Pharmaceuticals Reports Third Quarter 2024 Financial Results and Business Update

November 5, 2024

-- Third quarter 2024 net revenue from global sales of IMCIVREE® (setmelanotide) of $33.3 million --

-- Adult patients with acquired hypothalamic obesity (N=8) achieved mean BMI reduction of 12.8% on setmelanotide therapy at three months in French early-access program --

-- Top-line data readout for Phase 3 trial evaluating setmelanotide in 120 patients with acquired hypothalamic obesity on track for 1H 2025 --

-- sNDA to expand IMCIVREE label to include patients as young as 2 years old in approved indications accepted by FDA for Priority Review with PDUFA goal date of Dec. 26, 2024 --

-- Cash on-hand expected to support planned operations into 2026 --

-- Management to host conference call today at 5:00 p.m. ET --

BOSTON, Nov. 05, 2024 (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 third quarter ended September 30, 2024.

“Rhythm continued our strong clinical and commercial execution aimed at bringing treatments to patients with rare melanocortin-4 receptor (MC4R) pathway diseases to address hyperphagia and severe obesity,” said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. “Growth in global sales of IMCIVREE ® (setmelanotide) – primarily driven by prescriptions for patients with Bardet-Biedl syndrome (BBS) – remains steady, and we look forward to a potential expansion of its availability to patients as young as 2 years old, pending U.S. Food and Drug Administration (FDA) approval. We believe being able to treat children at a younger age can improve long-term clinical outcomes by addressing the underlying cause of hyperphagia and early-onset, severe obesity.”

Dr. Meeker continued, “We are particularly excited about new data from the early-access program in France for adult patients with hypothalamic obesity presented today at ObesityWeek®. Importantly, these real-world data support the potential for success of our Phase 3 trial and highlight the opportunity for adult patients who have lived with acquired hypothalamic obesity for a decade or more.”

Third Quarter and Recent Business Highlights

- Revenue from global sales of IMCIVREE was $33.3 million for the third quarter of 2024, an increase of 14% percent on a sequential basis from the second quarter of 2024, primarily driven by sales for BBS. In the third quarter of 2024, revenue of $23.3 million, or 70% of product revenue, was generated in the United States, an increase of 8% on a sequential basis; revenue of $10.0 million, or 30% of product revenue, was generated outside the United States, an increase of 35% on a sequential basis.
- During The Obesity Society’s ObesityWeek ® conference in San Antonio in November 2024, Rhythm and its collaborators delivered several presentations, including:
    - Real-world data from patients participating in an early-access program in France that showed eight adult patients with acquired hypothalamic obesity achieved a mean BMI reduction of 12.8% at three months on setmelanotide therapy; five of these patients achieved a mean BMI reduction of 21.3% after six months on therapy. These results demonstrate the clinical benefit of setmelanotide in adult patients who had lived with hypothalamic obesity for an average of 12.1 years;
    - Data from the extension phase of the Company’s Phase 2 trial of setmelanotide showed that patients with acquired hypothalamic obesity who were on therapy for more than one year (n=11) achieved a mean overall percent reduction in fat mass of 29.6%, versus a reduction in lean muscle mass of 7.7%; and four male patients between 11 and 14 years old at baseline exhibited an increase in muscle mass; and
    - Encouraging data from the Company’s exploratory Phase 2 DAYBREAK study showed that 27 of 32 patients on setmelanotide, or 84% of patients, achieved or maintained 5% BMI reduction from baseline through stage 2, compared to 5 of 17 patients, or 29.4% of patients, who transitioned to placebo for stage 2; also in DAYBREAK, a

12.4% mean percent BMI reduction was observed for all patients on continuous setmelanotide therapy over 40 weeks (n=32).

- On October, 24, 2024, the Company announced a joint research collaboration with Axovia Pharmaceuticals, led by its CEO and Co-founder Professor Philip Beales, in order to better understand the global epidemiology of BBS, how various symptoms impact patients and their families' lives, the underlying genetics of this disease, and the need for improved diagnosis and additional treatment options.
- On August 26, 2024, the Company announced that the FDA accepted for Priority Review its supplemental New Drug Application (sNDA) for IMCIVREE to treat children as young as 2 years old in its approved indications. The FDA assigned a Prescription Drug User Fee Act (PDUFA) goal date of December 26, 2024.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Complete enrollment in the supplemental, 12-patient Japanese cohort of its global Phase 3 trial evaluating setmelanotide in acquired hypothalamic obesity by the end of 2024;
- Complete enrollment in two substudies in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases by the end of 2024;
- Complete enrollment in the Phase 2 trial evaluating oral MC4R agonist bivamelagon (LB54640) in acquired hypothalamic obesity in the first quarter of 2025;
- Following submission and approval of a protocol amendment, begin dosing patients with acquired hypothalamic obesity in Part C of the Phase 1 trial evaluating the weekly, MC4R agonist RM-718 in the first quarter of 2025; and
- Announce top-line data in the Phase 3 trial evaluating setmelanotide in acquired hypothalamic obesity in the first half of 2025.

Third Quarter 2024 Financial Results:

Cash Position: As of September 30, 2024, cash, cash equivalents and short-term investments were approximately $298.4 million, as compared to $275.8 million as of December 31, 2023.

Revenue: Net product revenues relating to global sales of IMCIVREE were $33.3 million for the third quarter of 2024, as compared to $22.5 million for the third quarter of 2023.

R&D Expenses: R&D expenses were $37.9 million in the third quarter of 2024, as compared to $33.6 million in the third quarter of 2023. The year-over-year increase was primarily due to increased costs associated with certain clinical 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 $35.4 million for the third quarter of 2024, as compared to $30.5 million for the third quarter of 2023. The year-over-year increase was primarily due to increased headcount, marketing and promotions costs and expenses for professional services.

Other income (expense), net: Other income (expense), net was ($0.1) million for the third quarter of 2024.

Net Loss: Net loss attributable to common stockholders was ($45.0) million for the third quarter of 2024, or a net loss per basic and diluted share of ($0.73), as compared to a net loss attributable to common stockholders of ($44.2) million for the third quarter of 2023, or a net loss per basic and diluted share of ($0.76).

Year to Date 2024 Financial Results:

Revenue: Net product revenues relating to sales of IMCIVREE were $88.3 million for the nine months ended September 30, 2024, as compared to $53.2 million for the nine months ended September 30, 2023.

R&D Expenses: R&D expenses were $196.8 million for the nine months ended September 30, 2024, as compared to $105.1 million for the nine months ended September 30, 2023. This increase was primarily due to costs associated with the acquisition and development of bivamelagon (LB54640), increased headcount and certain clinical trial costs. This increase was partially offset by one-time, non-recurring costs associated with the acquisition of Xinvento B.V. in 2023.

SG&A Expenses: SG&A expenses were $106.2 million for the nine months ended September 30, 2024, as compared to $85.2 million for the nine months ended September 30, 2023. The increase was primarily due to increased headcount, professional services fees, marketing and data analytics, and general office expenses to accommodate a growing workforce.

Other income (expense), net: Other income (expense), net was $7.4 million for the nine months ended September 30, 2024, as compared to $0.4 million for the nine months ended September 30, 2023. The increase was primarily due to a gain on settlement of the forward contract associated with the issuance of convertible preferred stock during the second quarter of 2024, the change in fair value of the embedded derivative of the debt royalty obligation to HealthCare Royalty Partners (HCR) and increased interest income based on higher investment balances. This increase was partially offset by accretion of the non-current liability payable to LG Chem due in July 2025 and non-cash interest expense related to amortization of debt discount from the HCR milestone payment received in September 2023.

Net Loss: Net loss attributable to common stockholders was ($219.9) million for the nine months ended September 30, 2024, or a net loss attributable to common stockholders per basic and diluted share of $(3.62), as compared to a net loss attributable to common stockholders of ($143.0) million for the nine months ended September 30, 2023, or a net loss per basic and diluted share of ($2.50).

Financial Guidance: For the year ending December 31, 2024, Rhythm today reduced its anticipated Non-GAAP Operating Expenses to approximately $245 million to $255 million from $250 million to $270 million. Non-GAAP Operating Expenses are derived from:

- GAAP total operating expenses, inclusive of:
  - S,G&A expenses of approximately $113 million; and
  - R&D expenses of approximately $137 million;
- and excluding:
  - Stock-based compensation, and
  - $92.4 million in fixed consideration related to in-licensing of global rights to bivamelagon (LB54640), which was recognized in the first quarter of 2024.

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 September 30, 2024, will be sufficient to fund its operating expenses and capital expenditure requirements into 2026.

Conference Call Information
Rhythm Pharmaceuticals will host a live conference call and webcast at 5:00 p.m. ET today to review its third quarter 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 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) for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency confirmed by genetic testing, or patients with a clinical diagnosis of Bardet-Biedl syndrome (BBS). 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 6 years of age and above. The EC has also authorized setmelanotide for control of hunger and treatment of obesity in children as young as 2 years old, living with BBS or POMC, PCSK1, or LEPR deficiency. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare diseases, as well as investigational MC4R agonists bivamelagon (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 for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to POMC, PCSK1 or 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) or BBS.

In the European Union, 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 Europe, 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 POMC, PCSK1 or LEPR deficiency, or BBS, 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

Skin Pigmentation and Darkening of Pre-Existing Nevi: Generalized increased skin pigmentation and darkening of pre-existing nevi have occurred because of its pharmacologic effect. Full body skin examinations prior to initiation and periodically during treatment should be conducted to monitor pre-existing and new pigmentary lesions.

Heart rate and blood pressure monitoring: In Europe, heart rate and blood pressure should be monitored as part of standard clinical practice at each medical visit (at least every 6 months) for patients treated with setmelanotide.

Disturbance in Sexual Arousal: Spontaneous penile erections in males and sexual adverse reactions in females have occurred. Patients who have an erection lasting longer than 4 hours should seek emergency medical attention.

Depression and Suicidal Ideation: Depression and suicidal ideation have occurred. Patients should be monitored for new onset or worsening depression or suicidal thoughts or behaviors. Consideration should be given to discontinuing setmelanotide 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 setmelanotide.

Pediatric Population: The prescribing physician should periodically assess response to setmelanotide therapy. In growing children, the impact of weight loss on growth and maturation should be evaluated. In Europe, the prescribing physician should monitor growth (height and weight) using age- and sex-appropriate growth curves.

Risk of Serious Adverse Reactions Due to Benzyl Alcohol Preservative in Neonates and Low Birth Weight Infants: Setmelanotide 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

Lactation: Not recommended when breastfeeding.

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 U.S. Prescribing Information and EU Summary of Product Characteristics 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, bivamelagon (LB54640), RM-718, the potential expansion of IMCIVREE for use by patients as young as 2 years old and the anticipated PDUFA date, and 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 announcement of data from our clinical trials, including our Phase 3 trial evaluating setmelanotide for patients with acquired hypothalamic obesity, the enrollment of patients in the Japanese cohort of our global Phase 3 trial evaluating setmelanotide in acquired hypothalamic obesity, the Phase 3 EMANATE trial and the Phase 2 trial evaluating the oral MC4R agonist bivamelagon (LB54640) in acquired hypothalamic obesity; dosing of patients in Part C of the Phase 1 trial evaluating RM-718; the Company’s business strategy and plans, including regarding commercialization of setmelanotide; our anticipated financial performance and financial position for any period of time, including estimated Non-GAAP Operating Expenses for the year ending December 31, 2024; 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 September 30, 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 stock-based compensation expenses. These items, which could materially affect the computation of forward-looking GAAP operating expenses, are inherently uncertain and depend 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 September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Revenues:				
Product revenue, net	$ 33,251	$ 22,504	$ 88,296	$ 53,194
Total revenues	33,251	22,504	88,296	53,194
Costs and expenses:				
Cost of sales	3,828	2,412	9,581	6,069
Research and development	37,931	33,570	196,789	105,059
Selling, general, and administrative	35,377	30,475	106,174	85,158
Total costs and expenses	77,136	66,457	312,544	196,286
Loss from operations	(43,885)	(43,953)	(224,248)	(143,092)
Other income (expense):				

Other income (expense), net	1,088	(159)	2,434	(369)
Gain on settlement of forward contract	—	—	8,900	—
Interest expense	(5,242)	(3,149)	(15,156)	(9,342)
Interest income	4,054	3,466	11,196	10,126
Total other income (expense), net	(100)	158	7,374	415
Loss before income taxes	(43,985)	(43,795)	(216,874)	(142,677)
Provision for income taxes	(344)	368	436	368
Net loss	$ (43,641)	$ (44,163)	$ (217,310)	$ (143,045)
Accrued dividends on convertible preferred stock	(1,329)	—	(2,631)	—
Net loss attributable to common stockholders	$ (44,970)	$ (44,163)	$ (219,941)	$ (143,045)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.73)	$ (0.76)	$ (3.62)	$ (2.50)
Weighted-average common shares outstanding, basic and diluted	61,219,918	57,874,960	60,793,329	57,154,803
Other comprehensive loss:				
Net loss attributable to common stockholders	$ (44,970)	$ (44,163)	$ (219,941)	$ (143,045)
Foreign currency translation adjustment	(602)	76	(975)	49
Unrealized (loss) gain, net on marketable securities, net of tax	615	(175)	237	(70)
Comprehensive loss	$ (44,957)	$ (44,262)	$ (220,679)	$ (143,066)

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

	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 47,521	$ 60,081
Short-term investments	250,869	215,765
Accounts receivable, net	19,306	14,867
Inventory	13,895	8,624
Prepaid expenses and other current assets	8,750	8,931
Total current assets	340,341	308,268
Property and equipment, net	801	1,341
Right-of-use asset	3,588	781
Intangible assets, net	6,388	7,028
Restricted cash	462	328
Other long-term assets	11,992	14,999
Total assets	$ 363,572	$ 332,745
Liabilities, Convertible Preferred Stock and Stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,887	$ 4,885
Accrued expenses and other current liabilities	54,797	48,262
Other current liability	36,635	--
Deferred revenue	1,286	1,286
Lease liability	--	770
Total current liabilities	97,605	55,203
Long-term liabilities:		
Deferred royalty obligation	109,241	106,143
Lease liability, non-current	4,030	490

Derivative liability	--	1,150
Total liabilities	210,876	162,986
Commitments and contingencies (Note 15)		
Series A convertible preferred stock, $0.001 par value: 150,000 shares authorized; 150,000 and 0 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively. Liquidation preference of $150,000 as of September 30, 2024.	141,481	—
Stockholders' equity:		
Preferred stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 61,436,251 and 59,426,559 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	61	59
Additional paid-in capital	1,123,768	1,064,302
Accumulated other comprehensive (loss) income	(604)	134
Accumulated deficit	(1,112,010)	(894,736)
Total stockholders' equity	11,215	169,759
Total liabilities, convertible preferred stock and stockholders' equity	$ 363,572	$ 332,745

Source: Rhythm Pharmaceuticals, Inc.