



Rhythm Pharmaceuticals Reports Second Quarter 2024 Financial Results and Business Update

August 6, 2024

-- Second quarter 2024 net revenue from global sales of IMCIVREE® (setmelanotide) of \$29.1 million --

-- Dosed first patients in supplemental, 12-patient Japanese cohort in global Phase 3 trial evaluating setmelanotide in hypothalamic obesity; On track for topline data from 120-patient, pivotal cohort in 1H 2025 --

-- Completed sNDA submission seeking to expand U.S. label of IMCIVREE to treat pediatric patients as young as 2 years old in approved indications --

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

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

BOSTON, Aug. 06, 2024 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a global commercial-stage biopharmaceutical company focused on transforming the lives of patients and their families living with rare neuroendocrine diseases, today reported financial results and provided a business update for the second quarter ended June 30, 2024.

"We delivered a strong quarter executing on our global strategy to bring IMCIVREE® (setmelanotide) to patients with rare melanocortin-4 receptor (MC4R) diseases to treat their hyperphagia and severe obesity," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. "This included steady growth in global sales of IMCIVREE, and the achievement of regulatory milestones in Europe and the United States that brings us closer to expanding its availability to patients as young as 2 years old. We believe this pediatric expansion will improve long-term clinical outcomes by treating these genetically-caused diseases when hyperphagia and obesity begin."

Dr. Meeker continued, "We are making rapid progress in developing our MC4R portfolio assets to bring much needed therapeutic options to patients with rare MC4R diseases, starting with acquired hypothalamic obesity. We remain on track to report topline data from the pivotal cohort of our ongoing, global Phase 3 trial of setmelanotide for this indication in the first half of 2025, and we recently began dosing patients in a supplemental cohort in Japan, where the prevalence rate is higher than in the United States and Europe. In addition, we dosed the first patients with hypothalamic obesity in the Phase 2 trial evaluating an oral MC4R agonist, LB54640, and we are advancing our dose-finding, Phase 1 trial of the weekly RM-718."

Second Quarter and Recent Business Highlights

- Today, Rhythm announced that approximately 100 new prescriptions for IMCIVREE for Bardet-Biedl syndrome (BBS) were written by U.S. prescribers and the Company has received payor approval for reimbursement for approximately 70 prescriptions during the second quarter of 2024;
- Today, Rhythm announced it dosed the first patients in the supplemental, 12-patient Japanese cohort of its global Phase 3 trial evaluating setmelanotide in hypothalamic obesity;
- Today, Rhythm announced it has completed submission of its supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration to expand the label of IMCIVREE to treat pediatric patients between the ages of 2 and younger than 6 years old in approved indications;
- On July 31, 2024, Rhythm announced that the European Commission (EC) expanded the marketing authorization for IMCIVREE to include children as young as 2 years old with obesity due to BBS or pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency;
- On July 23, 2024, Rhythm announced that it dosed the first patients in the Company's Phase 2 clinical trial evaluating LB54640, an investigational oral MC4R agonist, in hypothalamic obesity;
- Effective July 1, 2024, Alastair "Al" Garfield, Ph.D. was appointed Chief Scientific Officer;
- On June 3, 2024, Rhythm announced that it presented the first patient and caregiver reported experiences from qualitative interviews following the completion of a Phase 2 trial that evaluated treatment with setmelanotide in hypothalamic obesity during the Endocrine Society Annual Meeting & Expo (ENDO 2024);
- On May 22, 2024, Rhythm announced that the National Institute for Health and Care Excellence (NICE) in Great Britain issued guidance that recommends IMCIVREE as an option for treating obesity and the control of hunger (hyperphagia) in patients between 6 years old and younger than 18 with BBS; and
- On April 1, 2024, Rhythm announced that the Company entered into an investment agreement with current shareholders,

led by Perceptive Advisors LLC and its Discovery Fund and a life-sciences focused institutional investor for the sale of its series A convertible preferred stock for gross proceeds of \$150 million to the Company. This transaction closed on April 15, 2024.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Announce Stage 2 data from the Phase 2 DAYBREAK clinical trial during a medical meeting in the second half of 2024;
- Complete enrollment in two or more substudies in the Phase 3 EMANATE trial evaluating setmelanotide in genetically caused MC4R pathway diseases in the second half of 2024;
- Announce top-line data in the Phase 3 trial evaluating setmelanotide in hypothalamic obesity in the first half of 2025;

Second Quarter 2024 Financial Results:

- **Cash Position:** As of June 30, 2024, cash, cash equivalents and short-term investments were approximately \$319.1 million, as compared to \$275.8 million as of December 31, 2023.
- **Revenue:** Net product revenues relating to global sales of IMCIVREE were \$29.1 million for the second quarter of 2024, as compared to \$19.2 million for the second quarter of 2023. For the second quarter ended June 30, 2024, 74% of the Company's product revenue was generated in the United States.
- **R&D Expenses:** R&D expenses were \$30.2 million in the second quarter of 2024, as compared to \$33.5 million in the second quarter of 2023. The year-over-year decrease was primarily due to a decrease in clinical trial costs. This decrease was partially offset by increased headcount.
- **S,G&A Expenses:** S,G&A expenses were \$36.4 million for the second quarter of 2024, as compared to \$30.0 million for the second quarter of 2023. The year-over-year increase was primarily due to increased headcount and expenses for professional services.
- **Other income (expense), net.** Other income (expense), net increased by \$8.8 million from the second quarter of 2023 to the second quarter of 2024, primarily due to a gain of \$8.9 million on settlement of the forward contract associated with the issuance of our convertible preferred stock.
- **Net Loss:** Net loss attributable to common stockholders was (\$33.6) million for the second quarter of 2024, or a net loss per basic and diluted share of (\$0.55), as compared to a net loss attributable to common stockholders of (\$46.7) million for the second quarter of 2023, or a net loss per basic and diluted share of (\$0.82).

Year to Date 2024 Financial Results:

- **Revenue:** Net product revenues relating to sales of IMCIVREE were \$55.0 million for the six months ended June 30, 2024, as compared to \$30.7 million for the six months ended June 30, 2023.
- **R&D Expenses:** R&D expenses were \$158.9 million for the six months ended June 30, 2024, as compared to \$71.5 million for the six months ended June 30, 2023. This increase was primarily due to the acquisition of LG Chem's proprietary compound LB54640 for \$92.4 million in the six months ended June 30, 2024, and increased costs associated with headcount and certain clinical trials.
- **S,G&A Expenses:** S,G&A expenses were \$70.8 million for the six months ended June 30, 2024, as compared to \$54.7 million for the six months ended June 30, 2023. The increase was primarily due to increased headcount to support business and commercial operations in the United States and internationally, expenses for professional services and other expenses.
- **Other income (expense), net:** Other (income)/ expense, net was \$7.5 million for the six months ended June 30, 2024, as compared to \$0.2 million for the six months ended June 30, 2023. This increase was primarily due to a gain of \$8.9 million on settlement of the forward contract associated with the issuance of our convertible preferred stock. This was partially offset by recognition of \$1.9 million of non-cash interest expense in the six months ended June 30, 2024 associated with accretion of the non-current liability payable to LG Chem in July, 2025 and an increase in non-cash interest expense of \$1.5 million related to amortization of debt discount and deferred financing fees associated with a higher deferred royalty obligation balance, based on the receipt of our final \$25.0 million sales milestone in the three months ended September 30, 2023.
- **Net Loss:** Net loss attributable to common stockholders was (\$174.9) million for the six months ended June 30, 2024, or a net loss attributable to common stockholders per basic and diluted share of \$(2.89), as compared to a net loss attributable to common stockholders of (\$98.9) million for the six months ended June 30, 2023, or a net loss per basic and diluted share of (\$1.74).

Financial Guidance: For the year ending December 31, 2024, Rhythm anticipates approximately \$250 million to \$270 million in Non-GAAP Operating Expenses. Non-GAAP Operating Expenses are derived from:

- GAAP total operating expenses, inclusive of:

- S,G&A expenses of \$105 million to \$110 million; and
- R&D expenses of \$145 million to \$160 million,
 - inclusive of \$10 million to \$15 million of LB54640 development costs;
- but which excludes:
 - Stock-based compensation, and
 - \$92.4 million in fixed consideration related to in-licensing of global rights to 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 June 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 8:00 a.m. ET today to review its second 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 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) 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. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare diseases, as well as 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 Bardet-Biedl syndrome (BBS) or genetically confirmed loss-of-function biallelic proopiomelanocortin (POMC), including PCSK1, deficiency or biallelic leptin receptor (LEPR) deficiency in adults and children 6 years of age and above.

Limitations of Use

In the United States and Europe, Setmelanotide should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

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.

WARNINGS AND PRECAUTIONS

Skin Monitoring: Setmelanotide may lead to generalized increased skin pigmentation and darkening of pre-existing naevi because of its pharmacologic effect. Full body skin examinations should be conducted annually to monitor pre-existing and new skin pigmentary lesions before and during treatment with setmelanotide.

Heart rate and blood pressure monitoring: 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.

Prolonged penile erection: Spontaneous penile erections have been reported in clinical trials with setmelanotide. Patients who

have a penile erection lasting longer than 4 hours should be instructed to seek emergency medical attention for potential treatment of priapism.

Depression: In clinical trials, depression has been reported in patients treated with setmelanotide. Patients with depression should be monitored at each medical visit during treatment with setmelanotide. Consideration should be given to discontinuing setmelanotide if patients experience suicidal thoughts or behaviors.

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. The prescribing physician should monitor growth (height and weight) using age- and sex-appropriate growth curves.

Excipients: This medicinal product contains 10 mg benzyl alcohol in each ml. Benzyl alcohol may cause allergic reactions. Patients who are pregnant or breastfeeding should be advised of the potential risk from the excipient benzyl alcohol, which might accumulate over time and cause metabolic acidosis. This medicinal product should be used with caution in patients with hepatic or renal impairment, because of the potential risk from the excipient benzyl alcohol which might accumulate over time and cause metabolic acidosis.

Sodium: This medicinal product contains less than 1 mmol sodium (23 mg) per dose, that is to say essentially “sodium-free.”

ADVERSE REACTIONS

The most frequent adverse reactions are hyperpigmentation (51%), injection site reaction (39%), nausea (33%), and headache (26%).

USE IN SPECIFIC POPULATIONS

Pregnancy

There are no data from the use of setmelanotide in pregnant women. Animal studies do not indicate direct harmful effects with respect to reproductive toxicity. However, administration of setmelanotide to pregnant rabbits resulted in decreased maternal food consumption leading to embryo-fetal effects. As a precautionary measure, setmelanotide should not be started during pregnancy or while attempting to get pregnant as weight loss during pregnancy may result in fetal harm. If a patient who is taking setmelanotide has reached a stable weight and becomes pregnant, consideration should be given to maintaining setmelanotide treatment as there was no proof of teratogenicity in the nonclinical data. If a patient who is taking setmelanotide and still losing weight gets pregnant, setmelanotide should either be discontinued, or the dose reduced while monitoring for the recommended weight gain during pregnancy. The treating physician should carefully monitor weight during pregnancy in a patient taking setmelanotide.

Breast-feeding

It is unknown whether setmelanotide is excreted in human milk. A nonclinical study showed that setmelanotide is excreted in the milk of nursing rats. No quantifiable setmelanotide concentrations were detected in plasma from nursing pups. A risk to the newborn/infant cannot be excluded. A decision must be made whether to discontinue breastfeeding or to discontinue/abstain from setmelanotide therapy taking into account the benefit of breastfeeding for the child and the benefit of therapy for the mother.

Fertility

No human data on the effect of setmelanotide on fertility are available. Animal studies did not indicate harmful effects with respect to fertility.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337. See [Summary of Product Characteristics' APPENDIX V](#) for a list of European national reporting systems to communicate adverse reactions.

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, LB54640 and RM-718 the potential expansion of IMCIVREE for use by patients as young as 2 years old; 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 2 DAYBREAK trial, Phase 3 trial evaluating setmelanotide for patients with hypothalamic obesity, Phase 1 clinical trial of RM-718, and Phase 2 trial evaluating LB54640; the enrollment of patients in the Phase 3 EMANATE trial; 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 June 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.

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Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)
(Unaudited)

	<u>Three months ended June 30,</u>		<u>Six months ended June 30,</u>	
	<u>2024</u>	<u>2023</u>	<u>2024</u>	<u>2023</u>
Revenues:				
Product revenue, net	\$ 29,078	\$ 19,221	\$ 55,045	\$ 30,691
Total revenues	<u>29,078</u>	<u>19,221</u>	<u>55,045</u>	<u>30,691</u>
Costs and expenses:				
Cost of sales	2,947	2,236	5,753	3,657
Research and development	30,194	33,543	158,858	71,487

Selling, general, and administrative	36,415	30,046	70,797	54,674
Total costs and expenses	69,556	65,825	235,408	129,818
Loss from operations	(40,478)	(46,604)	(180,363)	(99,127)
Other (expense) income:				
Other income (expense), net	300	(17)	824	34
Gain on forward contract	8,900	—	8,900	—
Interest expense	(4,603)	(3,303)	(9,358)	(6,449)
Interest income	4,097	3,221	7,143	6,660
Total other income (expense), net	8,694	(99)	7,509	245
Loss before income taxes	(31,784)	(46,703)	(172,854)	(98,882)
Provision for income taxes	479	—	779	—
Net loss	\$ (32,263)	\$ (46,703)	\$ (173,633)	\$ (98,882)
Accrued dividends on convertible preferred stock	(1,302)	—	(1,302)	—
Net loss attributable to common stockholders	\$ (33,565)	\$ (46,703)	\$ (174,935)	\$ (98,882)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.55)	\$ (0.82)	\$ (2.89)	\$ (1.74)
Weighted-average common shares outstanding, basic and diluted	61,011,824	56,867,662	60,577,691	56,788,757
Other comprehensive loss:				
Net loss attributable to common stockholders	\$ (33,565)	\$ (46,703)	\$ (174,935)	\$ (98,882)
Foreign currency translation adjustment	(302)	(48)	(373)	(27)
Unrealized gain (loss), net on marketable securities	(134)	40	(378)	105
Comprehensive loss	\$ (34,001)	\$ (46,711)	\$ (175,686)	\$ (98,804)

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	\$ 161,669	\$ 60,081
Short-term investments	157,461	215,765
Accounts receivable, net	17,598	14,867
Inventory	11,994	8,624
Prepaid expenses and other current assets	8,644	8,931
Total current assets	357,366	308,268
Property and equipment, net	973	1,341
Right-of-use asset	3,696	781
Intangible assets, net	6,601	7,028
Restricted cash	460	328
Other long-term assets	12,750	14,999
Total assets	\$ 381,846	\$ 332,745
Liabilities, convertible preferred stock and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 4,543	\$ 4,885
Accrued expenses and other current liabilities	48,077	48,262
Deferred revenue	1,286	1,286
Lease liability	816	770
Total current liabilities	54,722	55,203
Long-term liabilities:		
Deferred royalty obligation	108,372	106,143
Lease liability, non-current	3,301	490

Derivative liability	660	1,150
Other long-term liabilities	35,596	—
Total liabilities	<u>202,351</u>	<u>162,986</u>
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 June 30, 2024 and December 31, 2023, respectively	140,152	—
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at June 30, 2024 and December 31, 2023	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 61,095,949 and 59,426,559 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively	60	59
Additional paid-in capital	1,108,269	1,064,302
Accumulated other comprehensive income (loss)	(617)	134
Accumulated deficit	<u>(1,068,369)</u>	<u>(894,736)</u>
Total stockholders' equity	<u>39,343</u>	<u>169,759</u>
Total liabilities, convertible preferred stock and stockholders' equity	\$ 381,846	\$ 332,745



Source: Rhythm Pharmaceuticals, Inc.