



Rhythm Pharmaceuticals Reports First Quarter 2024 Financial Results and Business Update

May 7, 2024

-- First quarter 2024 net revenue from global sales of IMCIVREE[®] (setmelanotide) of \$26.0 million --

-- On track to submit sNDA to the FDA to treat pediatric patients between 2 and younger than 6 years old in approved indications in second quarter of 2024 --

-- Phase 3 trial evaluating setmelanotide in hypothalamic obesity remains on track for topline data readout in 1H 2025; dosing of first patients in Japan anticipated in 2Q 2024 --

-- Secured \$150 million in convertible preferred stock financing, funding planned operations into 2026 --

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

BOSTON, May 07, 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 first quarter ended March 31, 2024.

"We continue to execute our global strategy of delivering IMCIVREE[®] (setmelanotide) to patients with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. "We had another solid quarter marked by steady growth in global sales of IMCIVREE, which is now available in 14 markets."

Dr. Meeker continued, "We remain focused on expanding the global opportunity for setmelanotide in additional indications, most notably in hypothalamic obesity with our ongoing, global Phase 3 trial. This pivotal trial is progressing as planned, and we anticipate dosing the first patients with hypothalamic obesity in Japan in the second quarter of 2024. In addition, we expect to submit a supplementary New Drug Application (sNDA) to the FDA seeking a label expansion for IMCIVREE to treat pediatric patients between 2 and younger than 6 years old in approved indications in the second quarter of 2024. While we work to maximize the setmelanotide opportunity, we are also advancing our broader MC4R portfolio with continued progress with our RM-718 and LB54640 programs."

First 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 that the Company has received payor approval for reimbursement for approximately 70 prescriptions during the first quarter of 2024.
- On May 2, Rhythm and its collaborators delivered one oral presentation and two posters at The Pediatric Endocrine Society's (PES) Annual Meeting May 2-5, 2024 in Chicago, IL, which highlighted previously disclosed data that showed setmelanotide achieved clinically meaningful weight reduction in pediatric patients with hypothalamic obesity, BBS or POMC and LEPR deficiency obesities.
- On April 29, the Company announced the publication of results from its Phase 2 study of setmelanotide for the treatment of hypothalamic obesity in the peer-reviewed journal *The Lancet Diabetes & Endocrinology*. The publication highlights that setmelanotide achieved a mean percent reduction in BMI of 15% from baseline at 16 weeks of therapy (N=18), and preliminary data from Rhythm's long-term extension study showing setmelanotide achieved mean BMI reduction of approximately 26% at one year (n=12).
- On April 1, 2024, Rhythm announced 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 ("Preferred Stock") for gross proceeds of \$150 million to the Company. This transaction closed on April 15, 2024.
- On March 25, Rhythm announced that the first patients had been dosed in the Company's Phase 1 clinical trial of RM-718, an investigational, weekly MC4R agonist designed to be MC1R-sparing and to potentially avoid hyperpigmentation.

Anticipated Upcoming Milestones

Rhythm expects to achieve the following near-term milestones:

- Complete submission of a supplementary New Drug Application (sNDA) to the FDA seeking a label expansion to treat

pediatric patients between 2 and younger than 6 years old in approved indications in the second quarter of 2024, with EMA approval for this pediatric expansion potentially in the fourth quarter of 2024;

- Begin dosing patients in the Japanese, 12-patient supplemental cohort of the Phase 3 trial evaluating setmelanotide in hypothalamic obesity in the second quarter of 2024;
- Begin dosing the first patients in the Phase 2 SIGNAL trial evaluating LB54640, an investigational oral small molecule MC4R agonist, in patients with hypothalamic obesity, in the third quarter of 2024. The 28-patient SIGNAL trial is a randomized, placebo-controlled, double-blind study designed to evaluate three dose levels of LB54640. The primary endpoint of the study is the change from baseline in body mass index after 14 weeks of treatment, and patients may continue on therapy for up to 52 weeks;
- Announce data from stage 2 of the exploratory Phase 2 DAYBREAK study evaluating setmelanotide in certain genetically-caused MC4R pathway diseases in the third quarter of 2024;
- Complete enrollment in two or more substudies in the Phase 3 EMANATE trial evaluating setmelanotide in a total of four genetically caused MC4R pathway diseases in the second half of 2024;
- Complete the Company's Phase 1 clinical trial of RM-718, an investigational, weekly MC4R-specific agonist, and announce data from this trial – including data from a planned cohort of patients with hypothalamic obesity - in the first half of 2025; and
- Announce top-line data in the Phase 3 trial evaluating setmelanotide in hypothalamic obesity in the first half of 2025.

First Quarter and Full Year 2023 Financial Results:

- **Cash Position:** As of March 31, 2024, cash, cash equivalents and short-term investments were approximately \$201 million, as compared to \$275.8 million as of December 31, 2023. This decrease in cash during the quarter included \$40 million for an up-front payment to LG Chem for global rights to LB-54640 paid in January 2024. This cash on-hand amount does not include gross proceeds of \$150 million from the sale of preferred stock during the second quarter.
- **Revenue:** Net product revenues relating to sales of IMCIVREE were \$26.0 million for the three months ended March 31, 2024, as compared to \$11.5 million for the three months ended March 31, 2023. For the three months ended March 31, 2024 and 2023, a substantial amount of our product revenue, or 74% and 83%, respectively, was generated from sales of our product in the United States.
- **R&D Expenses:** R&D expenses were \$128.7 million for the three months ended March 31, 2024, as compared to \$37.9 million for the three months ended March 31, 2023. The year-over-year increase was primarily due to in-process research and development costs totaling \$92.4 million associated with the acquisition of LG Chem's proprietary compound LB54640. Additional increased costs were associated with salaries, benefits and stock-based compensation related to the hiring of additional full-time employees, and clinical trial activities. These increases were partially offset by the one-time costs recorded in the three months ended March 31, 2023, for the purchase of research and development assets from Xinvento, BV.
- **S,G&A Expenses:** S,G&A expenses were \$34.4 million for the three months ended March 31, 2024, as compared to \$24.6 million for the three months ended March 31, 2023. The year-over-year increase was primarily due to increased compensation and benefits related costs associated with additional headcount to support our expanding business operations and commercial operations, increases in professional services and consulting costs and increases in costs associated with ongoing sales and marketing activities.
- **Other income (expense), net:** Other income (expense), net decreased by \$1.6 million to (\$1.2) million for the three months ended March 31, 2024, from \$0.4 million for the three months ended March 31, 2023, primarily due to an increase in non-cash interest expense from amortization of debt discount and deferred financing fees associated with a higher deferred royalty obligation balance, and recognition of non-cash interest expense associated with accretion of the non-current liability payable to LG Chem in July 2025; this decrease was partially offset by an increase in other income from the change in fair value of the embedded derivative of \$0.5 million associated with the Revenue Interest Financing Agreement, or RIFA, entered into with HealthCare Royalty Partners in June 2022.
- **Net Loss:** Net loss was \$(141.4) million for the three months ended March 31, 2024, or a net loss per basic and diluted share of (\$2.35), as compared to a net loss of (\$52.2) million for the three months ended March 31, 2023, or a net loss per basic and diluted share of (\$0.92).

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:
 - SG&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 March 31, 2024 along with the proceeds from the sale of \$150 million in preferred stock, 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 first 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 live 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 proopiomelanocortin (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, and regulatory and clinical design or progress, potential regulatory submissions, approvals and timing thereof of setmelanotide and LB54640, including our Phase 3 trial of setmelanotide for patients with hypothalamic obesity and Phase 2 SIGNAL trial, the potential benefits of setmelanotide for patients with hypothalamic obesity, our expectations surrounding potential regulatory submissions, approvals and timing thereof, including the sNDA for the label expansion of IMCIVREE, the Company's business strategy and plans, including regarding commercialization of setmelanotide, our anticipated financial performance and financial position, 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. 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, 2023 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 depends on various factors, some of which are outside of Rhythm's control.

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

	Three months ended March 31,	
	2024	2023
Revenues:		
Product revenue, net	\$ 25,967	\$ 11,469
License revenue	—	—
Total revenues	<u>25,967</u>	<u>11,469</u>
Costs and expenses:		
Cost of sales	2,807	1,421
Research and development	128,665	37,945
Selling, general, and administrative	34,382	24,634
Total costs and expenses	<u>165,854</u>	<u>64,000</u>
Loss from operations	(139,887)	(52,531)
Other income (expense):		
Other income (expense), net	524	(27)
Interest expense	(4,755)	(3,061)
Interest income	3,046	3,440
Total other income (expense), net	<u>(1,185)</u>	<u>352</u>
Loss before income taxes	(141,072)	(52,179)
Provision for income taxes	<u>300</u>	<u>—</u>

Net loss	\$	(141,372)	\$	(52,179)
Net loss per share, basic and diluted	\$	(2.35)	\$	(0.92)
Weighted-average common shares outstanding, basic and diluted		60,143,558		56,708,975
Other comprehensive loss:				
Net loss	\$	(141,372)	\$	(52,179)
Foreign currency translation adjustment		(71)		21
Unrealized gain (loss), net on marketable securities		(244)		65
Comprehensive loss	\$	(141,687)	\$	(52,093)

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

	<u>March 31,</u>	<u>December 31,</u>
	<u>2024</u>	<u>2023</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 53,428	\$ 60,081
Short-term investments	147,771	215,765
Accounts receivable, net	14,695	14,867
Inventory	8,507	8,624
Prepaid expenses and other current assets	11,352	8,931
Total current assets	<u>235,753</u>	<u>308,268</u>
Property and equipment, net	1,149	1,341
Right-of-use asset	670	781
Intangible assets, net	6,815	7,028
Restricted cash	460	328
Other long-term assets	13,804	14,999
Total assets	<u>\$ 258,651</u>	<u>\$ 332,745</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 7,550	\$ 4,885
Accrued expenses and other current liabilities	44,545	48,262
Deferred revenue	1,286	1,286
Lease liability	793	770
Total current liabilities	<u>54,174</u>	<u>55,203</u>
Long-term liabilities:		
Deferred royalty obligation	107,355	106,143
Lease liability, non-current	284	490
Derivative liability	660	1,150
Other Long Term Liabilities	34,597	—
Total liabilities	<u>197,070</u>	<u>162,986</u>
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at March 31, 2024 and December 31, 2023	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 60,964,468 and 59,426,559 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	60	59
Additional paid-in capital	1,097,810	1,064,302
Accumulated other comprehensive income (loss)	(181)	134

Accumulated deficit	<u>(1,036,108)</u>	<u>(894,736)</u>
Total stockholders' equity	<u>61,581</u>	<u>169,759</u>
Total liabilities and stockholders' equity	\$ 258,651	\$ 332,745



Source: Rhythm Pharmaceuticals, Inc.