



Rhythm Pharmaceuticals Reports Second Quarter 2020 Financial Results

August 3, 2020

-- Appointed David Meeker, M.D., as President and Chief Executive Officer --

-- FDA granted rare pediatric disease designations for setmelanotide for the treatment of POMC and LEPR deficiency obesities --

-- Submitted MAA to EMA for setmelanotide in POMC and LEPR deficiency obesities --

-- Received FDA acceptance of NDA for setmelanotide for POMC and LEPR deficiency obesities for filing; assigned PDUFA goal date of November 27, 2020 --

-- On track to announce topline data from pivotal Phase 3 trial of setmelanotide in BBS and Alström syndrome in fourth quarter of 2020 or early in the first quarter of 2021 --

BOSTON, Aug. 03, 2020 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a late-stage biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity, today reported financial results and provided a business update for the second quarter ended June 30, 2020.

"Rhythm has made tremendous regulatory and clinical progress on its path to bring the first approved therapy to individuals living with rare genetic disorders of obesity," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. "The potential approval of setmelanotide for the treatment of pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesities will validate the melanocortin-4 receptor (MC4R) pathway as an important therapeutic target, and we look forward to furthering our ongoing efforts, with pivotal data from our Phase 3 clinical trial in Bardet-Biedl syndrome (BBS) and Alström syndromes expected in the fourth quarter or early in 2021 and the continued expansion of our ongoing Phase 2 Basket study."

Dr. Meeker continued, "We are energized by the opportunity to help individuals living with rare genetic disorders of obesity, who currently have no meaningful treatment options for their severe obesity or insatiable hunger. Looking ahead, we are eager to complete our transformation into an integrated, patient-focused organization while continuing to advance our community building and patient engagement efforts."

Second Quarter 2020 and Recent Business Highlights:

Pipeline and Recent Developments:

POMC and LEPR Deficiency Obesities

- In July, Rhythm announced the submission of its Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity. In conjunction with this submission, Rhythm announced additional positive data from eight supplemental patients, including four pediatric patients, enrolled in its two pivotal Phase 3 clinical trials for POMC and LEPR deficiency obesities, as well as updated data from its long-term extension study of setmelanotide in patients with POMC or LEPR deficiency obesity. Rhythm included these data in its MAA submission package to the EMA.
 - As announced in July, all eight supplemental patients achieved the primary endpoint of 10 percent or greater weight loss at 52 weeks on setmelanotide therapy, as calculated under the same statistical analysis plan used in the pivotal trials.
 - Setmelanotide was well-tolerated in the long-term extension study, with continued clinical benefit and durable weight loss observed in patients at up to three years on therapy.
- In July, Rhythm announced that the U.S. Food and Drug Administration (FDA) granted rare pediatric disease designations for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity. Subject to FDA approval, Rhythm would be eligible to receive one priority review voucher, which could then be redeemed to receive priority review for any subsequent marketing application or sold or transferred to another company.
- In May, Rhythm announced that the FDA accepted its NDA for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity. The FDA granted Priority Review of the NDA and assigned a Prescription Drug User Fee Act (PDUFA) goal date of November 27, 2020.

Additional Development Pipeline Updates

- In June, Rhythm announced positive results from a Phase 2 study evaluating a once-weekly formulation of setmelanotide

in healthy obese volunteers. Healthy obese people treated with the weekly formulation of setmelanotide achieved comparable weight loss to those treated with the daily formulation, and both weekly and daily formulations of setmelanotide were observed to be safe and well-tolerated. PK analyses showed similar trough drug concentrations for the daily and weekly formulations over the duration of therapy. Rhythm plans to discuss next steps towards registration with the FDA.

- In July, Rhythm announced the publication of results from its Phase 2 study evaluating setmelanotide in people living with BBS in the peer-reviewed journal *Diabetes, Obesity and Metabolism*. As previously reported, data from the study demonstrated that treatment with setmelanotide reduced body weight and hunger in individuals with BBS.
- Rhythm today announced that it is re-evaluating potential indications for RM-853, its ghrelin o-acyltransferase (GOAT) inhibitor, and that the Company will provide an update in the fourth quarter of 2020.

Corporate:

- In July, Rhythm announced the appointment of David Meeker, M.D., the Chair of Rhythm's Board of Directors, as the President and Chief Executive Officer of the Company.
- In July, Rhythm announced the appointment of Joseph Shulman as the Company's Senior Vice President of Technical Operations.

Upcoming Milestones:

- Rhythm is on track to report topline data from its combined pivotal Phase 3 trial evaluating setmelanotide in BBS and Alström syndrome in the fourth quarter of 2020 or early in the first quarter of 2021.
- Rhythm expects to announce additional data in 2020 from its ongoing Phase 2 Basket Study of setmelanotide in high-impact heterozygous (HET) obesity and additional data from one or more of the other disorders being studied in its Phase 2 Basket Study.
- Rhythm expects to provide an update on its genetic sequencing efforts in 2020.

Second Quarter 2020 Financial Results:

- **Cash Position:** As of June 30, 2020, cash, cash equivalents and short-term investments were \$228.6 million, as compared to \$292.5 million as of December 31, 2019. This decrease reflects \$63.9 million of cash used to fund operating activities in the first half of 2020. Based on its current clinical development plans, Rhythm expects that its existing cash and cash equivalents and short-term investments will enable it to fund its operations at least through the end of 2021.
- **R&D Expenses:** R&D expenses were \$22.9 million for the second quarter of 2020 as compared to \$35.3 million for the second quarter of 2019. The decrease in R&D spending was primarily attributed to a \$6.3 million reduction in clinical trial expenses associated with the Company's GO-ID genotyping study and the once-weekly formulation study incurred with site initiations in 2019, a decrease of \$4.0 million related to genetic sequencing with expected lower volume in the same study, and a \$3.4 million decrease in expenses from purchases of setmelanotide API in the same quarter last year. These decreases were partially offset by an increase of \$1.7 million in spending related to the Phase 2 Basket Study and a \$2.0 million milestone payment associated with the license agreement with Ipsen on filing the NDA for setmelanotide for the treatment of POMC and LEPR deficiency obesities.
- **S,G&A Expenses:** S,G&A expenses were \$8.9 million for the second quarter of 2020 as compared to \$8.8 million for the second quarter of 2019. This modest increase was primarily due to increased consulting and professional services fees.
- **Net Loss:** Net loss was \$31.1 million for the second quarter of 2020, or a net loss per basic and diluted share of \$0.71, as compared to a net loss of \$42.8 million for the second quarter of 2019, or a net loss per basic and diluted share of \$1.24.

Year to Date 2020 Financial Results:

- **R&D Expenses:** R&D expenses were \$45.5 million for the six months ended June 30, 2020, as compared to \$58.1 million for the six months ended June 30, 2019. The decrease was primarily due to a decrease of \$8.7 million related to the GO-ID genotyping study, which is nearing its completion, and a decrease of \$5.2 million related to genetic sequencing efforts from expected lower volumes in the same study, as well as a decrease of \$2.5 million related to purchases of setmelanotide API. These decreases were partially offset by an increase of \$2.0 million in spending related to the Phase 2 Basket Study and a \$2.0 million milestone payment associated with the license agreement with Ipsen on filing the NDA for setmelanotide for the treatment of POMC and LEPR deficiency obesities.
- **S,G&A Expenses:** S,G&A expenses were \$21.7 million for the six months ended June 30, 2020, as compared to \$16.6 million for the six months ended June 30, 2019. The increase was primarily due to an accounting charge of \$3.5 million related to the separation agreement and modification of stock options for the Company's former chief executive officer upon his departure on March 27, 2020, as well as an increase of \$0.8 million related to patient engagement and disease awareness efforts and an increase of \$0.8 million in various consulting and professional services related to legal and IT support costs.
- **Net Loss:** Net loss was \$65.3 million for the six months ended June 30, 2020, or a net loss per basic and diluted share of \$1.48, as compared to a net loss of \$71.8 million for the six months ended June 30, 2019, or a net loss per basic and diluted share of \$2.08.

About Rhythm Pharmaceuticals

Rhythm is a late-stage biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. The FDA has accepted for filing an NDA for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity with Priority Review and assigned a PDUFA goal date of November 27,

2020. Rhythm also submitted an MAA for setmelanotide to treat individuals living with POMC deficiency obesity or LEPR deficiency obesity to the EMA in June 2020. Rhythm is also evaluating setmelanotide for reduction in hunger and body weight in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected in the fourth quarter of 2020 or early in the first quarter of 2021. Rhythm is leveraging the Rhythm Engine -- comprised of its Phase 2 basket study, TEMPO Registry, GO-ID genotyping study and Uncovering Rare Obesity program -- to improve the understanding, diagnosis and potentially the treatment of rare genetic disorders of obesity. For healthcare professionals, visit www.UNcommonObesity.com for more information. For patients and caregivers, visit www.LEADforRareObesity.com for more information. The company is based in Boston, MA.

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 potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, expectations regarding regulatory approval, the anticipated timing for release of clinical trial data, our ongoing efforts related to patient identification and genetic sequencing and timing thereof, and the and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations. Statements using word such as “expect”, “anticipate”, “believe”, “may”, “will” and similar terms are also forward-looking statements. Such statements are subject to numerous risks and uncertainties, including, but not limited to, the impact of our management transition, our ability to enroll patients in clinical trials, the design and outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, risks associated with data analysis and reporting, our liquidity and expenses, the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and general economic conditions, and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2020 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 release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

Rhythm Pharmaceuticals, Inc. Condensed Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

(Unaudited)

	Three months ended		Six months ended June 30,	
	June 30, 2020	2019	2020	2019
Operating expenses:				
Research and development	\$ 22,997	\$ 35,308	\$ 45,501	\$ 58,069
Selling, general, and administrative	8,921	8,841	21,717	16,600
Total operating expenses	<u>31,918</u>	<u>44,149</u>	<u>67,218</u>	<u>74,669</u>
Loss from operations	(31,918)	(44,149)	(67,218)	(74,669)
Other income (expense):				
Interest income, net	801	1,353	1,937	2,899
Total other income, net	<u>801</u>	<u>1,353</u>	<u>1,937</u>	<u>2,899</u>
Net loss	<u>\$ (31,117)</u>	<u>\$ (42,796)</u>	<u>\$ (65,281)</u>	<u>\$ (71,770)</u>
Net loss per share, basic and diluted	<u>\$ (0.71)</u>	<u>\$ (1.24)</u>	<u>\$ (1.48)</u>	<u>\$ (2.08)</u>
Weighted-average common shares outstanding, basic and diluted	<u>44,098,860</u>	<u>34,452,661</u>	<u>44,074,352</u>	<u>34,435,023</u>
Other comprehensive loss:				
Net loss	\$ (31,117)	\$ (42,796)	\$ (65,281)	\$ (71,770)
Unrealized gain on marketable securities	567	—	630	—
Comprehensive loss	<u>\$ (30,550)</u>	<u>\$ (42,796)</u>	<u>\$ (64,651)</u>	<u>\$ (71,770)</u>

Rhythm Pharmaceuticals, Inc. Condensed Consolidated Balance Sheets

(in thousands, except share data)

(Unaudited)

	<u>June 30, 2020</u>	<u>December 31, 2019</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 59,091	\$ 62,294
Short-term investments	169,535	230,165
Prepaid expenses and other current assets	<u>9,193</u>	<u>9,945</u>
Total current assets	237,819	302,404
Property and equipment, net	3,331	3,671
Right-of-use asset	1,932	2,045
Restricted cash	<u>403</u>	<u>403</u>
Total assets	<u>\$ 243,485</u>	<u>\$ 308,523</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 4,761	\$ 10,415
Accrued expenses and other current liabilities	9,640	13,530
Lease liability	<u>503</u>	<u>472</u>
Total current liabilities	14,904	24,417
Long-term liabilities:		
Lease liability	<u>2,828</u>	<u>3,086</u>
Total liabilities	17,732	27,503
Commitments and contingencies		
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at June 30, 2020 and December 31, 2019	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 44,115,612 and 43,996,753 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	44	44
Additional paid-in capital	615,691	606,307
Accumulated other comprehensive income	630	—
Accumulated deficit	<u>(390,612)</u>	<u>(325,331)</u>
Total stockholders' equity	<u>225,753</u>	<u>281,020</u>
Total liabilities and stockholders' equity	<u>\$ 243,485</u>	<u>\$ 308,523</u>

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