Rhythm Pharmaceuticals Reports First Quarter 2020 Financial Results

May 4, 2020

-- Completed rolling NDA submission to FDA for setmelanotide in POMC and LEPR deficiency obesities; on track to submit MAA to EMA in the second quarter of 2020 –

-- Received Orphan Drug Designation from FDA for setmelanotide for the treatment of Alström syndrome --

-- Continues to expect topline data from pivotal Phase 3 trial of setmelanotide in Bardet-Biedl and Alström syndromes in fourth quarter of 2020 or early in the first quarter of 2021 --

BOSTON, May 04, 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 first quarter ended March 31, 2020.

"We are excited about the completion of our first New Drug Application (NDA) submission, which marks a critical next step towards bringing a potentially life-changing medicine to individuals living with rare genetic disorders of obesity," said Hunter Smith, interim Chief Executive Officer and Chief Financial Officer of Rhythm. "We are especially proud of how our Rhythm community, including our employees, the clinical staff at our trials sites, and the patients and families involved in our studies, has endured and met the unprecedented challenges brought on by the novel coronavirus pandemic, enabling our ongoing efforts to advance the development of setmelanotide with minimal interruption."

Murray Stewart, M.D., Chief Medical Officer of Rhythm, said, "We are grateful for the support and dedication of the staff at the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) during these trying times, as their commitment to a consistent dialogue during the pandemic has supported the progression of our first NDA submission and our planned submission of a Marketing Authorization Application (MAA). As we move toward the potential commercialization of setmelanotide, our team is focused on identifying patients and advancing key pre-commercial activities across Rhythm. Looking ahead, we anticipate additional data from our Phase 2 Basket Study this year and remain on track to report topline data from our pivotal Phase 3 trial in Bardet-Biedl syndrome (BBS) and Alström syndrome in the fourth quarter of 2020 or early in the first quarter of 2021. As these studies progress, we are simultaneously focused on building greater awareness and understanding of melanocortin-4 receptor (MC4R) pathway obesity disorders among patients, physicians, caregivers and other key stakeholders."

First Quarter 2020 and Recent Business Highlights:

Pipeline and Recent Developments:

- In March, Rhythm announced the completion of its rolling submission of an NDA to the FDA for setmelanotide for the treatment of pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity. Rhythm requested priority review for the application, which, if granted, could result in a six-month review period from the date of acceptance of the application.
- In March, Rhythm announced that the FDA granted orphan drug designation to setmelanotide for the treatment of Alström syndrome.

Corporate:

- In March, Rhythm announced the appointment of Chief Financial Officer Hunter Smith as Interim President and Chief Executive Officer, succeeding Keith Gottesdiener, M.D., whose planned departure was announced previously in January 2020. Dr. Gottesdiener stepped down from his roles as CEO, President and member of the Board of Directors following the Company's completion of its NDA submission to the FDA. As previously disclosed, Rhythm's Board of Directors has formed a search committee and retained an executive search firm to assist in identifying a permanent successor.
- Also in March, to help protect the health and safety of the patients, caregivers and healthcare professionals involved in its ongoing clinical trials of setmelanotide, as well as its employees, in response to the novel coronavirus (COVID-19) pandemic, Rhythm implemented a number of precautionary clinical and operational measures to protect patient well-being and ensure consistent and appropriate clinical trial conduct. Rhythm has introduced measures designed to ensure patients already enrolled in ongoing clinical trials continue to be monitored as scheduled and receive their study drug.

Upcoming Milestones:

- Rhythm remains on track to submit an MAA to the EMA for setmelanotide in patients with POMC deficiency obesity and LEPR deficiency obesity in the second quarter of 2020.
- Rhythm expects 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 from its ongoing Phase 2 Basket Study of setmelanotide in high-impact heterozygous (HET) obesity and additional data from one or more of its other ongoing Phase 2 Basket Study indications in 2020.
- Rhythm expects to provide a clinical development update for its once-weekly formulation of setmelanotide in 2020.
- Rhythm expects to submit an investigational new drug (IND) application for RM-853, its ghrelin o-acyltransferase (GOAT) inhibitor to the FDA in 2020.
- Rhythm expects to provide an update on its genetic sequencing efforts in 2020.

First Quarter 2020 Financial Results:

- **Cash Position:** As of March 31, 2020, cash, cash equivalents and short-term investments were $257.4 million, as compared to $292.5 million as of December 31, 2019. This decrease reflects cash used to fund operating activities in the first quarter 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 $22.5 million for the first quarter of 2020 as compared to $22.8 million for the first quarter of 2019. The modest decrease in R&D spending was attributed to decreased costs associated with the GO-ID genotyping study and partially offset by increased spending related to the submission of the NDA to FDA, expansion of additional studies including the Phase 2 Basket Study, long-term extension study, and the Phase 2 study of a weekly formulation of setmelanotide.
- **S,G&A Expenses**: S,G&A expenses were $12.8 million for the first quarter of 2020 as compared to $7.8 million for the first quarter of 2019. The increase was primarily due to a non-cash, accounting charge of $3.5 million related to the separation agreement and modification of stock options for the former CEO and an increase of $1.2 million for efforts to drive disease awareness about rare genetic disorders of obesity and prepare for the potential commercialization of setmelanotide in the U.S.
- **Net Loss:** Net loss was $34.2 million for the first quarter of 2020, or a net loss per basic and diluted share of $0.78, as compared to a net loss of $29.0 million for the first quarter of 2019, or a net loss per basic and diluted share of $0.84.

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. In August 2019, the company announced positive topline results from pivotal Phase 3 clinical trials of setmelanotide, its MC4R agonist, in people living with POMC deficiency obesity and LEPR deficiency obesity and, in March 2020, completed its first rolling NDA submission to the FDA. 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, the anticipated timing for release of clinical trial data, the timing for submission of an MAA, our ongoing efforts related to patient identification and genetic sequencing and timing thereof, our ongoing search for a Chief Executive Officer, the anticipated impact of the COVID-19 pandemic on our business and operations, 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 March 31, 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.

Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)
(Unaudited)

	Three months ended March 31,	
	2020	2019
Operating expenses:		
Research and development	$ 22,504	$ 22,761
Selling, general, and administrative	12,796	7,759
Total operating expenses	35,300	30,520
Loss from operations	(35,300)	(30,520)
Total other income, net	1,136	1,546
Net loss	$ (34,164)	$ (28,974)
Net loss per share, basic and diluted	$ (0.78)	$ (0.84)
Weighted-average common shares outstanding, basic and diluted	44,049,843	34,417,189

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

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 55,776	$ 62,294
Short-term investments	201,648	230,165
Prepaid expenses and other current assets	10,436	9,945
Total current assets	267,860	302,404
Property and equipment, net	3,504	3,671
Right-of-use asset	1,989	2,045
Restricted cash	403	403
Total assets	$ 273,756	$ 308,523
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,932	$ 10,415
Accrued expenses and other current liabilities	11,277	13,530
Lease liability	487	472
Total current liabilities	17,696	24,417
Long-term liabilities:		
Lease liability	2,959	3,086
Total liabilities	20,655	27,503
Commitments and contingencies		
Stockholders' equity:		
Common stock	44	44
Additional paid-in capital	612,552	606,307
Accumulated deficit	(359,495)	(325,331)
Total stockholders' equity	253,101	281,020
Total liabilities and stockholders' equity	$ 273,756	$ 308,523

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