



Rhythm Pharmaceuticals Reports Second Quarter 2019 Financial Results

July 29, 2019

-- Launched Uncovering Rare Obesity to facilitate diagnosis of rare genetic disorders of obesity --

-- Announced positive opinion by EMA on Orphan Drug Designation for setmelanotide for the treatment of patients with BBS --

-- Topline data from pivotal Phase 3 trials evaluating setmelanotide in POMC and LEPR deficiency obesity expected in the third quarter of 2019, expected to be followed by NDA submission --

BOSTON, July 29, 2019 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a biopharmaceutical company focused on the development and commercialization of therapeutics 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, 2019.

"In recent months, we have made great strides toward achieving our foundational goals of changing the treatment paradigm for rare genetic disorders of obesity and delivering setmelanotide as the first-approved therapy for people living with these conditions," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "We look forward to building on this momentum in the months ahead. We are on track to announce topline data from our two pivotal trials in POMC and LEPR deficiency obesity in the third quarter of 2019, and we are actively preparing for our first new drug application."

Dr. Gottesdiener continued, "We are making significant progress across the Rhythm Engine. Our GO-ID genotyping study is enrolling patients ahead of schedule, with several new sites coming online in the second quarter. We are receiving additional sequencing data through new collaborations with multiple biobanks, and our recently launched Uncovering Rare Obesity program is supporting patient identification and helping identify underlying genetic causes of obesity. As we expand our efforts to educate physicians and patient advocacy groups on these conditions and the potential benefits of genetic sequencing, we are building a new community of patients, families and health care providers focused on rare genetic disorders of obesity in both the United States and Europe."

Second Quarter and Recent Business Highlights:

- In July 2019, Rhythm announced the launch of Uncovering Rare Obesity, a Rhythm-sponsored genetic testing program designed to broaden access to genetic testing and to determine the underlying genetic cause of severe obesity. This program may help identify patients eligible for participation in Rhythm's ongoing clinical research programs.
- In July 2019, Rhythm announced that the European Medicines Agency's Committee for Orphan Medicinal Products issued a positive opinion recommending setmelanotide for designation as an orphan medicinal product for the treatment of patients with Bardet-Biedl syndrome (BBS).
- Rhythm recently added two new members to its Board of Directors. In July 2019, the Company announced the appointment of Stuart Arbuckle, Executive Vice President and Chief Commercial Officer of Vertex Pharmaceuticals, to the Board. In June 2019, Jennifer Good, Co-founder, President and Chief Executive Officer of Trevi Therapeutics, Inc., was elected to the Board at the Company's annual general meeting of stockholders. Neil Exter, a partner at Third Rock Ventures and member of the Company's Board of Directors since April 2014, stepped down from the board as he did not seek re-election.

Upcoming Milestones:

- Rhythm expects to announce topline data from its two pivotal Phase 3 trials of setmelanotide in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesities in the third quarter of 2019. Pending positive results, the Company plans to submit one new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for setmelanotide in patients with these indications at the end of 2019 or early 2020.
- Rhythm remains on track to complete pivotal enrollment in its combined Phase 3 trial evaluating setmelanotide in BBS and Alström syndrome in the second half of 2019, with topline data expected in 2020.
- Rhythm expects to provide an update on ongoing efforts to increase patient identification in the fourth quarter of 2019.
- Rhythm expects to announce an expansion of its ongoing Phase 2 basket studies with additional MC4R pathway disorders in the fourth quarter of 2019.
- Rhythm expects to announce additional data from its ongoing Phase 2 basket study of setmelanotide in HET obesity in 2020.
- Rhythm expects to submit an investigational new drug (IND) application for RM-853, its ghrelin o-acyltransferase (GOAT) inhibitor for the treatment of Prader-Willi Syndrome, to the FDA in 2020.

Second Quarter 2019 Financial Results:

- **Cash Position:** As of June 30, 2019, cash, cash equivalents and short-term investments were \$195.2 million, as compared to \$252.1 million as of December 31, 2018. This decrease reflects cash used to fund operating activities in the first half of 2019. 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 into the fourth quarter of 2020.

- **R&D Expenses:** R&D expenses were \$35.3 million for the second quarter of 2019, as compared to \$8.6 million for the second quarter of 2018. The increase was primarily due to an increase in setmelanotide clinical trial activity of \$12.5 million, primarily due to the expansions of the GO-ID genotyping study and Phase 2 basket study with new trial sites for both studies, as well as ongoing enrollment in the Phase 3 study of setmelanotide in patients with BBS and Alström syndrome. Additional drivers of the R&D increase were: an increase of \$4.6 million related to translational research and genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies; an increase of \$4.8 million primarily related to the purchases of setmelanotide active pharmaceutical ingredient (API) for clinical trials and commercial scale up and pre-IND work for RM-853; and an increase of \$2.3 million due to the hiring of additional personnel related to community building and education efforts for physicians, care providers and patients who are facing rare genetic disorders of obesity.
- **S,G&A Expenses:** S,G&A expenses were \$8.8 million for the second quarter of 2019, as compared to \$6.4 million for the second quarter of 2018. The increase was primarily due to an increase of \$1.9 million in headcount-related expenses and an increase of \$0.2 million in efforts to drive disease awareness about rare genetic causes of obesity and prepare for the potential commercial launch of setmelanotide in the U.S.
- **Net Loss:** Net loss was \$42.8 million for the second quarter of 2019, or a net loss per basic and diluted share of \$1.24, as compared to a net loss of \$14.4 million for the second quarter of 2018, or a net loss per basic and diluted share of \$0.52.

Year to Date Financial Results:

- **R&D Expenses:** R&D expenses were \$58.1 million for the six months ended June 30, 2019, as compared to \$20.9 million for the six months ended June 30, 2018. The increase was primarily due to an increase in setmelanotide clinical trial activity of \$19.8 million, primarily due to the expansions of the GO-ID genotyping study and Phase 2 basket study, as well as ongoing enrollment in the Phase 3 study of setmelanotide in patients with BBS and Alström syndrome. Additional drivers of the R&D increase were: an increase of \$7.5 million related to translational research and genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies; an increase of \$6.0 million primarily related to purchases of setmelanotide API for clinical trials and commercial scale up and pre-IND work for RM-853; and an increase of \$4.7 million in employee-related costs due to the hiring of additional personnel; an increase of \$1.8 million in consulting and professional services associated with the creation of Rhythm's EU Medical Science Liaison field force and various medical communication programs. The above increases were partially offset by a decrease of \$4.4 million due to the non-cash expense related to the license acquired from Takeda for RM-853 in March 2018.
- **S,G&A Expenses:** S,G&A expenses were \$16.6 million for the six months ended June 30, 2019, as compared to \$11.2 million for the six months ended June 30, 2018. The increase was primarily due to an increase of \$3.7 million in headcount-related expenses and an increase of \$1.5 million in efforts to drive disease awareness about rare genetic causes of obesity and prepare for the potential commercial launch of setmelanotide in the U.S.
- **Net Loss:** Net loss was \$71.8 million for the six months ended June 30, 2019, or a net loss per basic and diluted share of \$2.08, as compared to a net loss of \$30.9 million for the six months ended June 30, 2018, or a net loss per basic and diluted share of \$1.12.

About Rhythm Pharmaceuticals

Rhythm is a biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. Rhythm is currently evaluating the efficacy and safety of setmelanotide, the company's first-in-class MC4R agonist, in Phase 3 studies in patients with Pro-opiomelanocortin (POMC) deficiency obesity, Leptin receptor (LEPR) deficiency obesity, Bardet-Biedl syndrome, and Alström syndrome. The company 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 certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties, including statements regarding Rhythm's anticipated timing for enrollment and design of clinical trials, the timing for filing of a new drug application, its ongoing efforts related to patient identification, the release of results of clinical trials, and its sufficiency of cash. 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, 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, and expenses, and other risks as may be detailed from time to time in our Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other reports we file 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.

(in thousands, except share and per share data)

	Three months ended June 30,		Six months ended June 30,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 35,308	\$ 8,584	\$ 58,069	\$ 20,870
Selling, general, and administrative	8,841	6,437	16,600	11,152
Total operating expenses	44,149	15,021	74,669	32,022
Loss from operations	(44,149)	(15,021)	(74,669)	(32,022)
Other income (expense):				
Interest income, net	1,353	609	2,899	1,151
Total other income:	1,353	609	2,899	1,151
Net loss and comprehensive loss	\$ (42,796)	\$ (14,412)	\$ (71,770)	\$ (30,871)
Net loss attributable to common stockholders	\$ (42,796)	\$ (14,412)	\$ (71,770)	\$ (30,871)
Net loss per share attributable to common stockholders, basic and diluted	\$ (1.24)	\$ (0.52)	\$ (2.08)	\$ (1.12)
Weighted average common shares outstanding, basic and diluted	34,452,661	27,960,664	34,435,023	27,624,271

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

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 63,118	\$ 49,542
Short-term investments	132,049	202,519
Prepaid expenses and other current assets	8,334	6,628
Total current assets	203,501	258,689
Property and equipment, net	3,901	1,120
Right-of-use asset	3,085	—
Restricted cash	401	401
Total assets	<u>\$ 210,888</u>	<u>\$ 260,210</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 6,565	\$ 7,640
Accrued expenses and other current liabilities	19,146	5,942
Lease liability	379	—
Total current liabilities	26,090	13,582
Long-term liabilities:		
Lease liability	3,331	—
Deferred rent	—	372
Total liabilities	29,421	13,954
Commitments and contingencies		

Stockholders' equity:

Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 34,497,542 and 34,410,725 shares issued and outstanding June 30, 2019 and December 31, 2018, respectively	34	34
Additional paid-in capital	437,805	430,824
Accumulated deficit	(256,372)	(184,602)
Total stockholders' equity	<u>181,467</u>	<u>246,256</u>
Total liabilities and stockholders' equity	<u>\$ 210,888</u>	<u>\$ 260,210</u>

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