Rhythm Pharmaceuticals Reports First Quarter 2019 Financial Results

May 3, 2019

- Announced updated clinical data from Phase 2 basket study of setmelanotide in MC4R pathway heterozygous (HET) obesity, demonstrating consistent weight and hunger responses in patients with high-impact loss-of-function (LOF) variants -

- Announced strategy for further development in HET obesity with patients stratified into cohorts based on LOF variant -

- Topline data from pivotal Phase 3 trials evaluating setmelanotide in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity expected in the third quarter of 2019, followed by New Drug Application (NDA) filings -

BOSTON, May 03, 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 first quarter ended March 31, 2019.

"In the first quarter, we announced preliminary data and an updated clinical strategy in patients with HET obesity, which represents key progress as we seek to expand our pipeline into additional MC4R pathway deficiency obesities that may be setmelanotide-responsive. In parallel, we detailed ongoing efforts to leverage the Rhythm Engine -- comprised of our Phase 2 basket study, TEMPO Registry and GO-ID genotyping study -- to accelerate patient identification and develop a better understanding of the burden in people living with rare genetic disorders of obesity," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "We are now preparing for topline data readouts from our two ongoing pivotal Phase 3 trials of setmelanotide in POMC and LEPR deficiency obesities and the potential filing of our first NDAs with the U.S. Food and Drug Administration (FDA). In parallel, we are broadening our community engagement to support the potential launch of setmelanotide. We expect 2019 to be a transformative year for Rhythm, as we progress closer to our vision of delivering the first approved therapeutic for people living with rare genetic disorders of obesity."

First Quarter and Recent Business Highlights:

Pipeline:

- In March 2019, Rhythm announced updated interim data from its ongoing Phase 2 basket study of setmelanotide in HET obesity and its plans to continue evaluating setmelanotide in this patient population, with patients enrolled into cohorts based on the impact of their LOF variants. Preliminary data in 13 patients with HET obesity demonstrated a more consistent treatment benefit in patients with high-impact LOF variants compared to patients with other variants. All four patients with high-impact LOF variants demonstrated positive weight and hunger responses and remain on therapy. In contrast, more variable responses in weight and hunger were observed in nine patients with other LOF variants, five of whom remain on therapy. Consistent with prior clinical experience, setmelanotide continues to be generally well-tolerated in patients with HET obesity.

Upcoming Milestones:

- Rhythm expects to announce topline data from its two pivotal Phase 3 trials of setmelanotide in POMC and LEPR deficiency obesities in the third quarter of 2019. Pending positive results, the Company plans to submit concurrent NDA filings to the FDA for setmelanotide in patients with these indications in late 2019 or early 2020.
- Rhythm expects to complete pivotal enrollment in its combined Phase 3 trial evaluating setmelanotide in Bardet-Biedl syndrome (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 2019.
- Rhythm expects to expand its ongoing Phase 2 basket studies into additional MC4R pathway disorders in 2019.
- Rhythm expects to announce additional data from its ongoing Phase 2 basket study of setmelanotide in HET obesity in 2020.

First Quarter 2019 Financial Results:

- **Cash Position:** As of March 31, 2019, cash, cash equivalents and short-term investments were $221.5 million, as compared to $252.1 million as of December 31, 2018. This decrease reflects cash used to fund operating activities in the first quarter 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 second half of 2020.
- **R&D Expenses:** R&D expenses were $22.8 million for the first quarter of 2019 as compared to $12.3 million for the first quarter of 2018. The increase was primarily due to an increase in setmelanotide clinical trial activity of $7.3 million, primarily due to the expansions of the Phase 2 basket study and GO-ID genotyping 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 $2.5 million in employee-related costs due to the hiring of additional personnel; an increase of $2.4 million related to genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies;

and an increase of $1.3 million related to the continued manufacturing of setmelanotide for clinical trials and of RM-853 for pre-clinical studies. These increases were offset by a $4.4 million reduction in R&D expenses due to the license acquired from Takeda for RM-853 in March 2018.

- **S,G&A Expenses:** S,G&A expenses were $7.8 million for the first quarter of 2019 as compared to $4.7 million for the first quarter of 2018. The increase was primarily due to an increase of $1.8 million in headcount-related expenses and an increase of $0.5 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 $29.0 million for the first quarter of 2019, or a net loss per basic and diluted share of $0.84, as compared to a net loss of $16.5 million for the first quarter of 2018, or a net loss per basic and diluted share of $0.60.

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 POMC deficiency obesity, LEPR deficiency obesity, BBS, and Alström syndrome. Rhythm is dedicated to improving the understanding of severe obesity that results from specific genetic disorders. 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 business strategy, its anticipated timing for enrollment and design of clinical trials, the timing for filing of new drug applications, the timing of information regarding efforts to identify patients, the release of results of clinical trials, and its expectations regarding the sufficiency to fund its operations. Statements using word such as "expect", "anticipate", "believe", "may", "will", "plan" 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.

Rhythm Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Three months ended March 31,	
	2019	**2018**
Operating expenses:		
Research and development	$ 22,761	$ 12,286
Selling, general, and administrative	7,759	4,715
Total operating expenses	30,520	17,001
Loss from operations	(30,520)	(17,001)
Other income (expense):		
Interest income, net	1,546	542
Total other income (expense):	1,546	542
Net loss and comprehensive loss	$ (28,974)	$ (16,459)
Net loss attributable to common stockholders	$ (28,974)	$ (16,459)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.84)	$ (0.60)
Weighted average common shares outstanding, basic and diluted	34,417,189	27,284,140

Rhythm Pharmaceuticals, Inc.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

	(Unaudited) March 31, 2019	December 31, 2018

Assets		
Current assets:		
Cash and cash equivalents	$ 42,020	$ 49,542
Short-term investments	179,503	202,519
Prepaid expenses and other current assets	8,646	6,628
Total current assets	230,169	258,689
Property and equipment, net	2,757	1,120
Right-of-use asset	3,174	—
Restricted cash	402	401
Total assets	$ 236,502	$ 260,210
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,992	$ 7,640
Accrued expenses and other current liabilities	8,373	5,942
Lease liability	202	—
Total current liabilities	12,567	13,582
Long-term liabilities:		
Lease liability	3,446	—
Deferred rent	—	372
Total liabilities	16,013	13,954
Commitments and contingencies		
Stockholders' equity:		
Preferred Stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 34,430,641 and 34,410,725 shares issued and outstanding March 31, 2019 and December 31, 2018, respectively	34	34
Additional paid-in capital	434,031	430,824
Accumulated deficit	(213,576)	(184,602)
Total stockholders' equity	220,489	246,256
Total liabilities and stockholders' equity	$ 236,502	$ 260,210

Investor Contact:
Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200
hannah.deresiewicz@sternir.com

Media Contact:
Adam Daley
Berry & Company Public Relations
212-253-8881
adaley@berrypr.com

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