



Rhythm Pharmaceuticals Reports Fourth Quarter and Full Year 2018 Financial Results

March 8, 2019

BOSTON, March 08, 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 fourth quarter and full year ended December 31, 2018.

"Our vision at Rhythm is to transform the care of obesity by delivering the first approved therapeutic for people living with rare genetic disorders of obesity due to impairments of the melanocortin-4 receptor (MC4R) pathway and building an integrated community of patients, caregivers, health care providers, and payors to better address these conditions," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "In 2018, we made important progress toward making this vision a reality. In addition to advancing setmelanotide into three pivotal trials, we shared new clinical data demonstrating setmelanotide's ability to have dramatic impacts on weight and hunger in people with different MC4R pathway deficiencies and launched our TEMPO registry to gather critical information on the daily burden of rare genetic disorders of obesity on those affected."

Dr. Gottesdiener continued, "As we look to 2019, we are excited to enter our next stage of growth, with topline data from our pivotal Phase 3 trials in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity expected in the third quarter, and our first new drug applications expected to follow in late 2019 or early 2020. In parallel, we will continue to further evaluate setmelanotide through advancing our ongoing pivotal trial in Bardet-Biedl syndrome (BBS) and Alström syndrome, reading out updated data from our Phase 2 basket study in POMC and other MC4R pathway heterozygous deficiency obesities and POMC epigenetic disorders, and expanding our clinical development pipeline to include patients with additional genetic targets tied to the MC4R pathway."

Fourth Quarter and Recent Business Highlights:

Pipeline:

- In January 2019, Rhythm announced updated clinical data from its Phase 2 basket study evaluating setmelanotide in BBS, including new data from two adolescent patients who previously had only short-term results available. In total, six out of nine BBS patients enrolled in the Phase 2 basket study have now achieved a clinically meaningful weight loss of 10 percent change from baseline. One additional patient with Type-1 diabetes did not lose weight, but did respond with marked improvements in hunger score and blood sugar levels.
- In December 2018, Rhythm announced the enrollment of the first patient in its pivotal Phase 3 clinical trial evaluating setmelanotide in BBS and Alström syndrome. The combined pivotal trial is a multinational study designed to enroll 30 patients aged six years and older, including at least 20 patients with BBS and at least six patients with Alström syndrome. The trial consists of 52 weeks of treatment with setmelanotide administered once daily by subcutaneous injection, including a 14-week placebo-controlled period. The primary endpoint is a responder analysis after approximately 52 weeks of therapy. The company plans to continue enrolling supplemental patients following enrollment of the last pivotal patient to generate additional data regarding the safety and efficacy of setmelanotide in people with BBS and Alström syndrome.
- In November 2018, Rhythm presented updated clinical data from its Phase 2 basket study evaluating setmelanotide in Alström syndrome at ObesityWeek 2018. These updated data from multiple Alström syndrome patients continued to show that treatment with setmelanotide leads to a reduction in body weight and decreased appetite. Safety data in individuals with Alström syndrome were consistent with previous clinical studies of setmelanotide.

Upcoming Milestones:

- Rhythm is continuing to enroll patients in its ongoing Phase 2 basket study of setmelanotide in patients with MC4R heterozygous deficiency obesity and epigenetic disorders. Rhythm is enrolling heterozygous deficiency patients into cohorts based on the degree of loss of function of their genetic variants, in order to assess the relationship between loss of function severity and responsiveness to setmelanotide. Rhythm expects to announce updated interim data from this approach in the near future.
- Rhythm expects to announce topline data from both of its pivotal Phase 3 trials of setmelanotide in POMC and LEPR deficiency obesity in the third quarter of 2019. Pending positive results, the Company plans to submit concurrent new drug application filings to the U.S. Food and Drug Administration 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 BBS and Alström

syndrome in the second half of 2019, and to announce topline data in 2020.

- Rhythm expects to expand its ongoing Phase 2 basket study into additional MC4R pathway disorders in 2019.

Fourth Quarter and Full Year 2018 Financial Results:

- **Cash Position:** As of December 31, 2018, cash, cash equivalents and short-term investments were \$252.1 million, as compared to \$148.1 million as of December 31, 2017. This increase reflects net proceeds of \$163.0 million from Rhythm's public offering of common stock in June 2018, partially offset by cash used to fund operating activities in 2018. 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:** Research and development expenses were \$18.8 million in the fourth quarter of 2018 and \$50.3 million for the year ended December 31, 2018, as compared to \$6.7 million in the fourth quarter of 2017 and \$22.9 million for the year ended December 31, 2017. The year-over-year increase was primarily due to an increase of \$6.5 million in employee-related costs due to the hiring of additional personnel throughout 2018; an increase of \$5.0 million related to ongoing clinical trials of setmelanotide; an increase of \$4.4 million due to the non-cash expense related to the license acquired from Takeda for RM-853; an increase of \$4.2 million in consulting and professional services; an increase of \$3.4 million related to genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies and support the expansion of Rhythm's development programs and discovery efforts; and an increase of \$1.0 million related to the continued manufacturing of setmelanotide for clinical trials and of RM-853 for pre-clinical studies.
- **S,G&A Expenses:** S,G&A expenses were \$8.4 million for the fourth quarter of 2018 and \$28.1 million for the year ended December 31, 2018, as compared to \$4.3 million for the fourth quarter of 2017 and \$9.5 million for the year ended December 31, 2017. The year-over-year increase was primarily due to an increase of \$8.7 million related to expenses incurred to increase awareness of rare genetic obesity disorders among healthcare providers and patients and to prepare for a potential commercial launch of setmelanotide; an increase of \$4.4 million in employee-related costs due to the hiring of additional personnel to support planned commercial activities, operations and the continued build of finance and human resource functions; and an increase of \$1.9 million related to professional fees associated with expanding operations.
- **Net Loss:** Net loss was \$25.5 million for the fourth quarter of 2018 and \$74.1 million for the year ended December 31, 2018, or a net loss per basic and diluted share of \$0.74 and \$2.39, respectively, as compared to a net loss of \$10.5 million for the fourth quarter of 2017 and \$33.7 million for the year ended December 31, 2017, or a net loss per basic and diluted share of \$0.41 and \$2.83, respectively.

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 anticipated timing for enrollment and design of clinical trials, the timing for filing of a new drug application, 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.

Rhythm Pharmaceuticals, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2018	2017	2018	2017
Operating expenses:				

Research and development	\$ 18,763	\$ 6,653	\$ 50,337	\$ 22,894
Selling, general, and administrative	8,388	4,330	28,080	9,518
Total operating expenses	<u>27,151</u>	<u>10,983</u>	<u>78,417</u>	<u>32,412</u>
Loss from operations	(27,151)	(10,983)	(78,417)	(32,412)
Other income (expense):				
Revaluation of Series A Investor Instrument and Series A Investor Right/Obligation	—	—	—	(1,863)
Interest income, net	1,644	452	4,353	566
Total other income (expense):	<u>1,644</u>	<u>452</u>	<u>4,353</u>	<u>(1,297)</u>
Net loss and comprehensive loss	<u>\$ (25,507)</u>	<u>\$ (10,531)</u>	<u>\$ (74,064)</u>	<u>\$ (33,709)</u>
Net loss attributable to common stockholders	<u>\$ (25,507)</u>	<u>\$ (10,619)</u>	<u>\$ (74,064)</u>	<u>\$ (37,582)</u>
Net loss attributable to common stockholders per common share, basic and diluted	<u>\$ (0.74)</u>	<u>\$ (0.41)</u>	<u>\$ (2.39)</u>	<u>\$ (2.83)</u>
Weighted average common shares outstanding, basic and diluted	<u>34,400,916</u>	<u>26,174,843</u>	<u>31,004,047</u>	<u>13,267,960</u>

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

	<u>December 31, 2018</u>	<u>December 31, 2017</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 49,542	\$ 34,236
Short-term investments	202,519	113,846
Prepaid expenses and other current assets	6,628	2,589
Total current assets	<u>258,689</u>	<u>150,671</u>
Property, plant and equipment, net	1,120	840
Restricted cash	401	225
Total assets	<u>\$ 260,210</u>	<u>\$ 151,736</u>
Liabilities, convertible preferred stock and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 7,640	\$ 2,427
Deferred rent	—	83
Accrued expenses and other current liabilities	5,942	4,210
Total current liabilities	<u>13,582</u>	<u>6,720</u>
Long-term liabilities:		
Deferred rent	372	228
Total liabilities	<u>13,954</u>	<u>6,948</u>
Commitments and contingencies		
Preferred stock:		
Convertible Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively	—	—
Stockholders' equity:		
Common stock, \$0.001 par value: 120,000,000 shares authorized; 34,410,725 and 27,284,140 shares issued and outstanding December 31, 2018 and December 31, 2017, respectively	34	27

Additional paid-in capital	430,824	255,013
Accumulated deficit	<u>(184,602)</u>	<u>(110,252)</u>
Total stockholders' equity	246,256	144,788
Total liabilities, convertible preferred stock and stockholders' equity	<u>\$ 260,210</u>	<u>\$ 151,736</u>

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