Rhythm Pharmaceuticals Reports Second Quarter 2018 Financial Results

August 8, 2018

-- Completed Pivotal Enrollment in Two Ongoing Phase 3 Clinical Trials Evaluating Setmelanotide in Pro-opiomelanocortin (POMC) and Leptin Receptor (LEPR) Deficiency Obesity --

-- Announced New Topline Clinical Data from Ongoing Phase 2 Basket Studies Evaluating Setmelanotide in Rare Genetic Disorders of Obesity, Including Updated Data in Bardet-Biedl Syndrome (BBS) and Proof-of-Concept Data in Alström Syndrome --

-- Granted PRIority MEdicines (PRIME) Designation by the European Medicines Agency (EMA) for Setmelanotide --

-- Successfully Completed $174 Million Public Offering --

BOSTON, Aug. 08, 2018 (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, 2018.

"Rhythm is entering a transformative period of development, as we continue to execute on our mission of providing new therapies for people living with rare genetic disorders of obesity," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "In recent months, we announced major clinical and regulatory advancements across our setmelanotide development program, including the completion of pivotal enrollment in our two ongoing Phase 3 trials in POMC and LEPR deficiency obesity, positive topline results from our ongoing Phase 2 basket studies in several additional rare genetic disorders of obesity, and receipt of PRIME designation from the EMA. Together, these accomplishments support our belief in setmelanotide as a new therapeutic option that has the potential to effectively address the excess hunger and weight associated with rare genetic disorders of obesity, and provide us with a clear and efficient path for further development. Following our successful follow-on offering, we are well capitalized, and believe we have sufficient resources to advance our ongoing clinical and market development efforts into the second half of 2020."

Second Quarter and Recent Business Highlights:

Pipeline:

- In July 2018, Rhythm announced that the EMA granted setmelanotide PRIME designation for the treatment of obesity and the control of hunger associated with deficiency disorders of the melanocortin-4 receptor (MC4R) pathway.
- In June 2018, Rhythm announced new topline clinical data from its ongoing Phase 2 basket studies of setmelanotide, including updated data in patients with BBS, initial proof-of-concept data in Alström Syndrome, and promising, preliminary data in POMC and other MC4R pathway heterozygous deficiency obesities, as well as in POMC epigenetic disorders. Across all these rare genetic disorders of obesity, setmelanotide was observed to be well-tolerated.
- In June 2018, Rhythm completed enrollment of the pivotal cohorts of 10 patients in two separate, ongoing, registration-enabling Phase 3 clinical trials evaluating setmelanotide in POMC and LEPR deficiency obesity.

Corporate:

- In June 2018, Rhythm completed a public offering of 6,591,800 shares of its common stock at a public offering price of $26.42 per share, for aggregate gross proceeds of approximately $174 million, before underwriting discounts, commissions, and offering expenses.

Upcoming Milestones:

- Rhythm plans to present results from its Phase 2 studies of setmelanotide in BBS and Alström Syndrome at the 57th Annual European Society for Pediatric Endocrinology Meeting (ESPE), September 27-29, 2018 in Athens, Greece.
- Rhythm plans to initiate and enroll the first patients in a combined pivotal Phase 3 trial evaluating setmelanotide in patients with BBS and Alström Syndrome by the end of 2018.
- Rhythm expects to announce updated data from a larger number of patients in its ongoing Phase 2 basket studies of setmelanotide in patients with POMC and other MC4R pathway heterozygous deficiency obesities and POMC epigenetic disorders in the first quarter of 2019.
- Rhythm expects to announce initial data from both of its pivotal Phase 3 trials of setmelanotide in POMC and LEPR

deficiency obesity in the third quarter of 2019. The Company then plans to submit concurrent New Drug Application filings to the U.S. Food and Drug Administration for setmelanotide in patients with these indications based on one-year data from the respective pivotal cohorts of 10 patients.

Second Quarter 2018 Financial Results:

- **Cash Position:** As of June 30, 2018, cash, cash equivalents and short-term investments were $287.6 million, as compared to $148.1 million as of December 31, 2017. This increase reflects net proceeds of $163.0 million from Rhythm's sale of additional stock in June 2018, partially offset by cash used to fund operating activities in the first half of 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 $8.6 million in second quarter of 2018 as compared to $5.4 million in the second quarter of 2017. The increase was primarily due to $1.5 million in employee-related costs due to the hiring of additional personnel during the past year, an increase of $1.3 million related to the creation of Rhythm's U.S. Medical Science Liaison field force and a $0.3 million increase in manufacturing expenses.
- **S,G&A Expenses:** S,G&A expenses were $6.4 million for the second quarter of 2018 as compared to $1.4 million for the second quarter of 2017. The increase was primarily due to a $1.3 million increase in employee-related costs attributable to hiring additional personnel to support Rhythm's growing organization, an increase of $2.1 million related to the development and building of Rhythm's commercial organization to drive patient identification, as well as increased professional and consulting fees associated with being a public company.
- **Net Loss:** Net loss was $14.4 million for the second quarter of 2018, or a net loss per basic and diluted share of $0.52, as compared to a net loss of $8.0 million for the second quarter of 2017, or a net loss per basic and diluted share of $0.79.

Year to Date 2018 Financial Results:

- **R&D Expenses:** Research and development expenses were $20.9 million for the six months ended June 30, 2018, as compared to $10.3 million for the six months ended June 30, 2017. The increase was primarily due to a $4.4 million non-cash expense related to the license acquired from Takeda for RM-853, a $1.0 million milestone expense associated with the license agreement with Ipsen and a $2.6 million increase in employee-related costs due to the hiring of additional clinical and development personnel during the second half of 2017.
- **S,G&A Expenses**: S,G&A expenses were $11.2 million for the six months ended June 30, 2018, as compared to $2.9 million for the six months ended June 30, 2017. The increase was primarily due to a $2.4 million increase in employee-related costs attributable to hiring additional support personnel, an increase of $3.2 million related to the development and building of Rhythm's commercial organization to drive patient identification, as well as increased professional and consulting fees associated with being a public company.
- **Net Loss:** Net loss was $30.9 million for the six months ended June 30, 2018, or a net loss per basic and diluted share of $1.12, as compared to a net loss of $15.5 million for the six months ended June 30, 2017, or a net loss per basic and diluted share of $1.52.

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 melanocortin-4 receptor (MC4R) agonist, in Phase 3 studies in patients with pro-opiomelanocortin (POMC) deficiency obesity (which includes deficiencies in both the POMC and PCSK1 genes) and leptin receptor (LEPR) deficiency obesity. Rhythm also supports The Genetic Obesity Project (www.GeneticObesity.com), which is dedicated to improving the understanding of severe obesity that results from specific genetic disorders. 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 expectations regarding its anticipated timing for filing of an NDA, its anticipated timing of initiation and enrollment for clinical trials and announcement of data, and expectations regarding the 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 outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, our use of cash 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)

(Unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2018	**2017**	**2018**	**2017**
Operating expenses:				
Research and development	$ 8,584	$ 5,397	$ 20,870	$ 10,270
Selling, general, and administrative	6,437	1,357	11,152	2,873
Total operating expenses	15,021	6,754	32,022	13,143
Loss from operations	(15,021)	(6,754)	(32,022)	(13,143)
Other income (expense):				
Revaluation of Series A Investor Instrument and Series A Investor Right/Obligation	—	(82)	—	(82)
Interest income, net	609	34	1,151	63
Total other income (expense):	609	(48)	1,151	(19)
Net loss and comprehensive loss	$ (14,412)	$ (6,802)	$ (30,871)	$ (13,162)
Net loss attributable to common stockholders	$ (14,412)	$ (8,008)	$ (30,871)	$ (15,534)
Net loss attributable to common stockholders per common share, basic and diluted	$ (0.52)	$ (0.79)	$ (1.12)	$ (1.52)
Weighted average common shares outstanding, basic and diluted	27,960,664	10,196,292	27,624,271	10,196,292

Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

	(Unaudited) June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 210,484	$ 34,236
Short-term investments	77,070	113,846
Prepaid expenses and other current assets	3,217	2,589
Total current assets	290,771	150,671
Property, plant and equipment, net	728	840
Restricted cash	250	225
Total assets	$ 291,749	$ 151,736
Liabilities, convertible preferred stock and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,312	$ 2,427
Deferred rent	86	83
Accrued expenses and other current liabilities	3,849	4,210
Total current liabilities	7,247	6,720
Long-term liabilities:		
Deferred rent	185	228
Total liabilities	7,432	6,948
Commitments and contingencies		
Preferred stock:		
Convertible Preferred Stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively	—	—

Stockholders' equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized; 34,173,137 and 27,284,140 shares issued and outstanding June 30, 2018 and December 31, 2017, respectively	34	27
Additional paid-in capital	425,406	255,013
Accumulated deficit	(141,123)	(110,252)
Total stockholders' equity	284,317	144,788
Total liabilities, convertible preferred stock and stockholders' equity	$ 291,749	$ 151,736

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