Rhythm Pharmaceuticals Reports Third Quarter 2018 Financial Results

November 9, 2018

-- Presented Updated Data from Phase 2 Basket Studies in Bardet-Biedl Syndrome (BBS) and Alström Syndrome at the 57th Annual European Society for Pediatric Endocrinology (ESPE) Meeting --

-- Launched TEMPO Registry for Rare Genetic Disorders of Obesity --

-- On-Track to Initiate and Enroll First Patients in Combined Pivotal Phase 3 Trial in BBS and Alström Syndrome by Year-End --

BOSTON, Nov. 09, 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 third quarter ended September 30, 2018.

"Our recent progress provides further compelling evidence of setmelanotide's potential as a replacement therapy for patients with MC4R pathway disorders, and demonstrates our commitment to building an integrated community to support people living with rare genetic forms of obesity," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "In addition to presenting updated data from our Phase 2 basket studies in patients with BBS and Alström Syndrome, which showed continued reductions in body weight and decreased appetite, we were pleased to launch our TEMPO registry in the third quarter. TEMPO represents a landmark effort to collect longitudinal data on patients with rare genetic disorders of obesity, in hopes of providing key stakeholders with the information and resources to better identify and treat people living with these conditions. We look forward to continuing to work together with physicians and the broader community to improve our understanding of rare genetic disorders of obesity, while also advancing our broad development program for setmelanotide with the initiation of our third pivotal trial expected by year-end."

Third Quarter and Recent Business Highlights:

Pipeline:

- In September 2018, Rhythm presented updated clinical data from its Phase 2 basket studies evaluating setmelanotide in BBS and Alström Syndrome at the 57th Annual ESPE Meeting. The updated results demonstrated that treatment with setmelanotide led to marked reductions in body weight and decreased appetite, with patients continuing to experience sustained improvements in both measures over time. Safety data presented at ESPE were consistent with previous clinical studies of setmelanotide. Rhythm's basket studies are a designed to evaluate setmelanotide response in multiple patient cohorts for different diseases of the MC4R pathway.
- In September 2018, Rhythm announced the launch of a new, non-interventional, patient registry, called *Tracing the Effect of the MC4R Pathway in Obesity* (TEMPO), designed to measure the prevalence of rare genetic disorders of obesity, collect data on the burden of these diseases, and inform future research aimed at identifying effective strategies to improve patient care and management. Rhythm also presented the TEMPO registry protocol at the 57th Annual ESPE Meeting.

Corporate:

- In October 2018, Rhythm announced the appointment of Murray W. Stewart, M.D., as Chief Medical Officer.

Upcoming Milestones:

- 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 pro-opiomelanocortin (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 leptin receptor (LEPR) deficiency obesity in the third quarter of 2019. The Company then plans to submit concurrent New Drug Application (NDA) 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.

Third Quarter 2018 Financial Results:

- **Cash Position:** As of September 30, 2018, cash, cash equivalents and short-term investments were $272.4 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 $10.7 million in the third quarter of 2018 as compared to $6.0 million in the third quarter of 2017. The increase was primarily due to $1.3 million in employee related costs due to the hiring of additional personnel during the second half of 2017 and throughout 2018, an increase of $1.2 million related to genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies as well as supplemental work related to the potential NDAs for setmelanotide, a $0.9 million increase related to an increase in patient visits and the number of investigator sites for new and existing clinical trials and a $0.6 million increase in travel and sponsorship related expenses for conferences attended during the three months ended September 30, 2018.
- **S,G&A Expenses:** S,G&A expenses were $8.5 million for the third quarter of 2018 as compared to $2.3 million for the third quarter of 2017. The increase was primarily due to a $1.4 million increase of employee related costs attributable to hiring additional personnel to enable the Company's growth, an increase of $4.0 million for patient identification programs, and consulting services supporting Rhythm's disease awareness activities, as well as increased professional and consulting fees associated with being a public company.
- **Net Loss:** Net loss was $17.7 million for the third quarter of 2018, or a net loss per basic and diluted share of $0.52, as compared to a net loss of $11.4 million for the third quarter of 2017, or a net loss per basic and diluted share of $1.78.

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 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. 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 September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	$ 10,705	$ 5,971	$ 31,575	$ 16,241
Selling, general, and administrative	8,539	2,315	19,691	5,188
Total operating expenses	19,244	8,286	51,266	21,429
Loss from operations	(19,244)	(8,286)	(51,266)	(21,429)
Other income (expense):				
Revaluation of Series A Investor Instrument and Series A Investor Right/Obligation	—	(1,781)	—	(1,863)
Interest income, net	1,558	51	2,709	114
Total other income (expense):	1,558	(1,730)	2,709	(1,749)
Net loss and comprehensive loss	$ (17,686)	$ (10,016)	$ (48,557)	$ (23,178)
Net loss attributable to common stockholders	$ (17,686)	$ (11,429)	$ (48,557)	$ (26,963)

Net loss attributable to common stockholders per common share, basic and diluted	$ (0.52)	$ (1.78)	$ (1.63)	$ (3.02)
Weighted average common shares outstanding, basic and diluted	34,256,519	6,404,254	29,859,314	8,918,389

Rhythm Pharmaceuticals, Inc.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

	(Unaudited) September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 58,247	$ 34,236
Short-term investments	214,139	113,846
Prepaid expenses and other current assets	4,152	2,589
Total current assets	276,538	150,671
Property, plant and equipment, net	939	840
Restricted cash	251	225
Total assets	$ 277,728	$ 151,736
Liabilities, convertible preferred stock and stockholders' equity		
Current liabilities:		
Accounts payable	$ 208	$ 2,427
Deferred rent	—	83
Accrued expenses and other current liabilities	7,595	4,210
Total current liabilities	7,803	6,720
Long-term liabilities:		
Deferred rent	288	228
Total liabilities	8,091	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 September 30, 2018 and December 31, 2017, respectively	—	—
Stockholders' equity:		
Common stock, $0.001 par value: 120,000,000 shares authorized; 34,382,525 and 27,284,140 shares issued and outstanding September 30, 2018 and December 31, 2017, respectively	34	27
Additional paid-in capital	428,698	255,013
Accumulated deficit	(159,095)	(110,252)
Total stockholders' equity	269,637	144,788
Total liabilities, convertible preferred stock and stockholders' equity	$ 277,728	$ 151,736

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