



## Rhythm Pharmaceuticals Reports Third Quarter 2019 Financial Results

November 1, 2019

*-- Announced Positive Topline Results from Pivotal Phase 3 Clinical Trials Evaluating Setmelanotide in POMC and LEPR Deficiency Obesity --*

*-- Added Four New MC4R Pathway Obesity Indications to Phase 2 Basket Study and Enrolling Patients --*

*-- Announced Genetic Sequencing Data Supporting Ultra-Rarity of POMC and LEPR Deficiency Obesity and Suggesting U.S. Prevalence for Four New Basket Indications of > 60,000 --*

*-- Successfully Completed \$172.5 Million Public Offering --*

BOSTON, Nov. 01, 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 third quarter ended September 30, 2019.

"We believe Rhythm is well-positioned to make a significant impact on the lives of people living with MC4R pathway-driven disorders of obesity," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "In August, we announced topline data from our first pivotal studies of setmelanotide, demonstrating a statistically significant and clinically meaningful impact on weight loss and hunger in patients with pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity, and we are on track to complete our New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) for these two indications in the fourth quarter of this year or first quarter of 2020."

Dr. Gottesdiener continued, "These milestones mark a critical first step toward our goal of bringing setmelanotide to many patients affected by rare genetic disorders of obesity. In parallel, we are continuing our community-building efforts to raise awareness, increase understanding of, and identify patients with these disorders, all of which are supported by the programs under our Rhythm Engine. We expect to complete enrollment in our pivotal Phase 3 trial evaluating setmelanotide for the treatment of severe obesity and hunger in patients with Bardet-Biedl syndrome (BBS) and Alström syndrome before year-end, and are enrolling patients with additional indications in our Phase 2 Basket Study, including SRC1 deficiency obesity, SH2B1 deficiency obesity, MC4R deficiency obesity and Smith-Magenis syndrome. Following our successful public offering in October, we believe that we are well-capitalized and have sufficient resources to advance our ongoing clinical and commercial efforts through at least the end of 2021."

### Third Quarter and Recent Business Highlights:

#### Pipeline:

- In September 2019, Rhythm announced the expansion of its Phase 2 Basket Study of setmelanotide to include four additional melanocortin-4 receptor (MC4R) pathway obesity disorders: SRC1 deficiency obesity, SH2B1 deficiency obesity, MC4R deficiency obesity and Smith-Magenis syndrome.
- In September 2019, Rhythm announced results from genetic sequencing of 13,567 individuals with severe obesity yields 11.7 percent (1,584 individuals) who have a rare genetic variant within the MC4R pathway eligible for enrollment in the Phase 2 Basket Study. These sequencing data support the ultra-rarity of POMC deficiency obesity and LEPR deficiency obesity and suggest an aggregate U.S. prevalence for the four new indications of approximately greater than 60,000 patients.
- In September 2019, Rhythm shared updated data from patients with BBS and Alström syndrome who are continuing to receive setmelanotide treatment in the Phase 2 Basket Study, demonstrating continued treatment effect in patients with BBS and Alström syndrome following nearly two years, and greater than one year, of treatment, respectively.
- In August 2019, Rhythm announced positive topline results from its two pivotal Phase 3 clinical trials evaluating setmelanotide for the treatment of POMC and LEPR deficiency obesity. Both trials met their primary endpoints and all key secondary endpoints, demonstrating a statistically significant and clinically meaningful effect on weight loss and reductions in insatiable hunger, or hyperphagia, in patients with POMC and LEPR deficiency obesity.

#### Corporate:

- In October 2019, Rhythm completed a public offering of 9,324,324 shares of its common stock at a public offering price of \$18.50 per share, for aggregate gross proceeds of approximately \$172.5 million, before underwriting discounts, commissions, and offering expenses.

#### Upcoming Milestones:

- Rhythm plans to present additional data from its two pivotal Phase 3 trials of setmelanotide in POMC deficiency obesity and LEPR deficiency obesity on November 4, 2019 at The Obesity Society's ObesityWeek in Las Vegas.
- Rhythm remains on track to complete submission of its rolling NDA to the FDA for setmelanotide in patients with POMC

deficiency obesity and LEPR deficiency obesity in the fourth quarter of 2019 or the first quarter of 2020.

- Rhythm expects to report topline data from its combined Phase 3 trial evaluating setmelanotide in BBS and Alström syndrome in 2020.
- Rhythm expects to announce additional data from its ongoing Phase 2 Basket Study of setmelanotide in high-impact heterozygous (HET) obesity and may announce preliminary data from other Phase 2 Basket Study indications 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.

#### Third Quarter 2019 Financial Results:

- **Cash Position:** As of September 30, 2019, cash, cash equivalents and short-term investments were \$162.4 million, as compared to \$195.2 million as of June 30, 2019. This decrease reflects \$33.6 million of cash used to fund operating activities in the third quarter of 2019. Cash, cash equivalents and short-term investments as of September 30, 2019 do not include aggregate gross proceeds of approximately \$172.5 million from the Company's October 2019 public offering.
- Based on its current clinical development plans and taking into account its recent public offering, Rhythm expects that its existing cash, cash equivalents and short-term investments will enable it to fund its operations through at least the end of 2021.
- **R&D Expenses:** R&D expenses were \$26.6 million for the third quarter of 2019, as compared to \$10.7 million for the third quarter of 2018. This increase was primarily due to an increase of \$10.2 million related to Rhythm's clinical trials, including an expansion of the GO-ID genotyping study and the 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; an increase of \$3.4 million related to translational research and genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies and pathway validation efforts; and an increase of \$1.5 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 \$10.5 million for the third quarter of 2019, as compared to \$8.5 million for the third quarter of 2018. This is due primarily to an increase of \$1.9 million in employee related costs in connection with the hiring of additional full-time employees to support planned commercial activities, operations and the continued build of finance and human resource functions.
- **Net Loss:** Net loss was \$36.0 million for the third quarter of 2019, or a net loss per basic and diluted share of \$1.04, as compared to a net loss of \$17.7 million for the third quarter of 2018, or a net loss per basic and diluted share of \$0.52.

#### Year to Date Financial Results:

- **Cash Position:** As of September 30, 2019, cash, cash equivalents and short-term investments were \$162.4 million, as compared to \$252.1 million as of December 31, 2018. This decrease reflects \$90.5 million of cash used to fund operating activities in 2019.
- **R&D Expenses:** R&D expenses were \$84.6 million for the nine months ended September 30, 2019, as compared to \$31.6 million for the nine months ended September 30, 2018. The increase was primarily due to an increase of \$30.0 million related to Rhythm's clinical trials associated with setmelanotide, including an expansion of the GO-ID genotyping study and the 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; an increase of \$11.0 million related to translational research and genetic sequencing efforts designed to improve identification of patients with MC4R pathway deficiencies and pathway validation efforts; an increase of \$6.2 million due to the hiring of additional full-time employees in order to support efforts for community building and education efforts for physicians, care providers and patients who are facing rare genetic disorders of obesity, as well as to support the growth of Rhythm's research and development programs; an increase of \$5.1 million primarily related to purchases of setmelanotide active pharmaceutical ingredient (API) for clinical trials, commercial scale up and pre-IND work for RM-853; and an increase of \$3.0 million in consulting and professional services associated with the creation of Rhythm's EU Medical Science Liaison field force, various medical communication programs and support for Rhythm's NDA filing. These increases were partially offset by a decrease of \$4.4 million due to a 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 \$27.1 million for the nine months ended September 30, 2019, as compared to \$19.7 million for the nine months ended September 30, 2018. The increase was primarily due to an increase of \$5.6 million in employee related costs in connection with the hiring of additional full-time employees to support planned commercial activities, operations and the continued build of finance and human resource functions; and an increase of \$2.1 million in various consulting and professional services related to 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 \$107.8 million for the nine months ended September 30, 2019, or a net loss per basic and diluted share of \$3.13, as compared to a net loss of \$48.6 million for the nine months ended September 30, 2018, or a net loss per basic and diluted share of \$1.63.

## 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. The company recently announced positive topline results from pivotal Phase 3 clinical trials of setmelanotide, its MC4R agonist, in patients with POMC deficiency obesity and LEPR deficiency obesity, and Rhythm expects to share additional data in forthcoming publications and medical meeting presentations. The company plans to complete its first rolling NDA submission to the FDA in the fourth quarter of 2019 or the first quarter of 2020. Rhythm is also evaluating setmelanotide in a pivotal Phase 3 study in patients with BBS and Alström syndrome and expects to complete enrollment in the second half of 2019. 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](http://www.UNcommonObesity.com) for more information. For patients and caregivers, visit [www.LEADforRareObesity.com](http://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 and submission of an investigational 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.*

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

	September 30, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 71,680	\$ 49,542
Short-term investments	90,755	202,519
Prepaid expenses and other current assets	8,442	6,628
Total current assets	170,877	258,689
Property and equipment, net	3,862	1,120
Right-of-use asset	2,097	—
Deferred issuance costs	295	—
Restricted cash	402	401
Total assets	\$ 177,533	\$ 260,210
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 7,745	\$ 7,640
Accrued expenses and other current liabilities	17,005	5,942
Lease liability	457	—
Total current liabilities	25,207	13,582
Long-term liabilities:		
Lease liability	3,211	—
Deferred rent	—	372
Total liabilities	28,418	13,954
Commitments and contingencies		
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2019 and December 31, 2018,	—	—

respectively

Common stock, \$0.001 par value: 120,000,000 shares authorized; 34,578,564 and 34,410,725 shares issued and outstanding September 30, 2019 and December 31, 2018, respectively

Additional paid-in capital	35	34
Accumulated deficit	441,455	430,824
Total stockholders' equity	(292,375)	(184,602)
Total liabilities and stockholders' equity	149,115	246,256
	\$ 177,533	\$ 260,210

**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,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 26,572	\$ 10,705	\$ 84,641	\$ 31,575
Selling, general, and administrative	10,535	8,539	27,135	19,691
Total operating expenses	<u>37,107</u>	<u>19,244</u>	<u>111,776</u>	<u>51,266</u>
Loss from operations	(37,107)	(19,244)	(111,776)	(51,266)
Other income (expense):				
Interest income, net	1,104	1,558	4,003	2,709
Total other income:	<u>1,104</u>	<u>1,558</u>	<u>4,003</u>	<u>2,709</u>
Net loss and comprehensive loss	<u>\$ (36,003)</u>	<u>\$ (17,686)</u>	<u>\$ (107,773)</u>	<u>\$ (48,557)</u>
Net loss attributable to common stockholders	<u>\$ (36,003)</u>	<u>\$ (17,686)</u>	<u>\$ (107,773)</u>	<u>\$ (48,557)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (1.04)</u>	<u>\$ (0.52)</u>	<u>\$ (3.13)</u>	<u>\$ (1.63)</u>
Weighted average common shares outstanding, basic and diluted	34,541,765	34,256,519	34,470,995	29,859,314

**Corporate Contact:**

David Connolly  
Head of Investor Relations and Corporate Communications  
Rhythm Pharmaceuticals, Inc.  
857-264-4280  
[dconnolly@rhythmtx.com](mailto:dconnolly@rhythmtx.com)

**Investor Contact:**

Hannah Deresiewicz  
Stern Investor Relations, Inc.  
212-362-1200  
[hannah.deresiewicz@sternir.com](mailto:hannah.deresiewicz@sternir.com)

**Media Contact:**

Adam Daley  
Berry & Company Public Relations  
212-253-8881  
[adaley@berrypr.com](mailto:adaley@berrypr.com)



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