



## Rhythm Pharmaceuticals Reports Fourth Quarter and Full Year 2019 Financial Results

March 2, 2020

*-- On track to complete rolling NDA submission to the FDA for setmelanotide in POMC and LEPR deficiency obesities in the first quarter of 2020 --*

*-- Granted orphan drug designation by the European Medicines Agency for setmelanotide in Alström syndrome --*

*-- Completed full enrollment in Phase 3 trial of setmelanotide in Bardet-Biedl and Alström syndromes in December; topline data expected in the fourth quarter of 2020 or early in the first quarter of 2021 --*

*--Rhythm Phase 2 Basket study actively enrolling multiple cohorts across five rare MC4R pathway-related disorders with potential U.S. prevalence greater than 80,000 patients; data updates expected in 2020 --*

*-- Ongoing genetic sequencing efforts have sequenced more than 25,000 people with early-onset, severe obesity --*

BOSTON, March 02, 2020 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a late-stage biopharmaceutical company aimed at developing and commercializing therapies 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, 2019.

"We believe Rhythm is entering 2020 in a position of strength as we continue to make significant progress in the science and understanding of rare genetic disorders of obesity and prepare to deliver setmelanotide as the first therapeutic option for people living with these conditions," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm.

Murray Stewart, M.D., Chief Medical Officer, added, "We expect to submit our first New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the coming weeks. In parallel, we are focused on advancing the development of setmelanotide in five additional melanocortin-4 receptor (MC4R) pathway-related disorders in our Phase 2 Basket study. We are also working to build a robust community of patients, caregivers and healthcare providers in order to drive patient identification, increase genetic testing and improve the understanding of these conditions. These efforts are critically important to our progress in Bardet-Biedl syndrome (BBS) and Alström syndrome, for which we expect topline pivotal data in the fourth quarter of 2020 or early in the first quarter of 2021. Lastly, our ongoing sequencing efforts continue to demonstrate that a significant percentage of severely obese individuals may be suffering from MC4R-related disorders of severe obesity and unrelenting hunger, indicating a need for a therapy like setmelanotide that has the potential to treat people living with these conditions."

### Fourth Quarter and Recent Business Highlights:

#### Pipeline and Recent Developments:

- Today, Rhythm announced that its ongoing genetic sequencing programs have now sequenced more than 25,000 people with early-onset, severe obesity. Rhythm's sequencing efforts seek to uncover more rare genetic disorders of obesity and develop a better understanding of those disorders currently under study in its pivotal trials and Phase 2 Basket Study. Rhythm expects to provide an update on its genetic sequencing efforts in 2020.
- Today, Rhythm announced that the European Commission adopted the European Medicines Agency's (EMA's) Committee for Orphan Medicinal Products' positive opinion and designated setmelanotide as an orphan medicinal product for the treatment of patients with Alström syndrome.
- In December 2019, Rhythm announced the completion of enrollment in its pivotal Phase 3 clinical trial evaluating setmelanotide in BBS and Alström syndrome. The Company enrolled 32 individuals with BBS and six individuals with Alström syndrome in the pivotal cohort.
- In November 2019, Rhythm presented late-breaking data from its two pivotal Phase 3 trials evaluating setmelanotide in POMC deficiency obesity and LEPR deficiency obesity at ObesityWeek 2019<sup>®</sup>, which highlighted the effect of setmelanotide on body mass index scores and certain cardiovascular parameters.

#### Corporate:

- In January 2020, Rhythm announced that Dr. Gottesdiener intends to step down as Chief Executive Officer, President and a member of the Company's Board of Directors, following the NDA submission for POMC deficiency obesity and LEPR

deficiency obesity, expected in the first quarter of 2020. Rhythm's Board of Directors has initiated a search for a new chief executive officer.

- 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 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 first quarter of 2020.
- Rhythm expects to submit a Marketing Authorization Application (MAA) to the EMA for setmelanotide in patients with POMC deficiency obesity and LEPR deficiency obesity in the second quarter of 2020.
- Rhythm expects to report topline data from its combined Phase 3 trial evaluating setmelanotide in BBS and Alström syndrome in the fourth quarter of 2020 or early in the first quarter of 2021.
- Rhythm expects to announce additional data from its ongoing Phase 2 Basket Study of setmelanotide in high-impact heterozygous (HET) obesity and additional data from one or more of its other ongoing Phase 2 Basket Study indications in 2020.
- Rhythm expects to provide a clinical development update for its once-weekly formulation of setmelanotide 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.
- Rhythm expects to provide an update on its genetic sequencing efforts in 2020.

#### Fourth Quarter and Full Year 2019 Financial Results:

- **Cash Position:** As of December 31, 2019, cash, cash equivalents and short-term investments were \$292.5 million, as compared to \$252.1 million as of December 31, 2018. This increase reflects net proceeds of \$161.4 million from Rhythm's public offering of common stock in October 2019, partially offset by cash used to fund operating activities in 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 operations through at least the end of 2021.
- **R&D Expenses:** R&D expenses were \$24.8 million in the fourth quarter of 2019 and \$109.5 million for the year ended December 31, 2019, as compared to \$18.8 million in the fourth quarter of 2018 and \$50.3 million for the year ended December 31, 2018. The year-over-year increase was primarily due to an increase of \$34.6 million related to Rhythm's clinical trials, including an expansion of enrollment and opening of new trial sites for the ongoing Phase 3 study of setmelanotide in patients with BBS and Alström syndrome, the Phase 2 Basket Study, and the GO-ID genotyping study; as well as an increase of \$11.8 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 \$6.8 million due to the hiring of additional personnel in Medical Affairs as well as research and development. This increase was partially offset by a decrease of \$4.4 million due to the 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 \$9.4 million for the fourth quarter of 2019 and \$36.6 million for the year ended December 31, 2019, as compared to \$8.4 million for the fourth quarter of 2018 and \$28.1 million for the year ended December 31, 2018. The year-over-year increase was primarily due to an increase of \$6.9 million in employee-related costs in connection with the full-year impact of employees hired in 2018, as well as new personnel in 2019, to support planned commercial activities, operations and finance.
- **Net Loss:** Net loss was \$33.0 million for the fourth quarter of 2019 and \$140.7 million for the year ended December 31, 2019, or a net loss per basic and diluted share of \$0.78 and \$3.86, respectively, as compared to a net loss of \$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.

#### About Rhythm Pharmaceuticals

Rhythm is a late-stage 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 people living with POMC deficiency obesity and LEPR deficiency obesity, and plans to complete its first rolling NDA submission to the FDA in the first quarter of 2020. Rhythm is also evaluating setmelanotide in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected in the fourth quarter of 2020 or early in the first quarter of 2021. Rhythm 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 an NDA, submission of an investigational new drug application and submission of an MAA, its ongoing efforts related to*

patient identification and genetic sequencing, the release of results of clinical trials and updates on patient enrollment, 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.

**Consolidated Statements of Operations**  
(in thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 24,810	\$ 18,763	\$ 109,450	\$ 50,337
Selling, general, and administrative	9,414	8,388	36,550	28,080
Total operating expenses	34,224	27,151	146,000	78,417
Loss from operations	(34,224)	(27,151)	(146,000)	(78,417)
Other income (expense):				
Interest income, net	1,268	1,644	5,271	4,353
Total other income:	1,268	1,644	5,271	4,353
Net loss	\$ (32,956)	\$ (25,507)	\$ (140,729)	\$ (74,064)
Net loss per common share, basic and diluted	\$ (0.78)	\$ (0.74)	\$ (3.86)	\$ (2.39)
Weighted average common shares outstanding, basic and diluted	42,213,180	34,400,916	36,422,450	31,004,047

**Consolidated Balance Sheets**  
(in thousands, except share and per share data)

	December 31, 2019	December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 62,294	\$ 49,542
Short-term investments	230,165	202,519
Prepaid expenses and other current assets	9,945	6,628
Total current assets	302,404	258,689
Property and equipment, net	3,671	1,120
Right-of-use asset	2,045	—
Restricted cash	403	401
Total assets	\$ 308,523	\$ 260,210
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 10,415	\$ 7,640
Accrued expenses and other current liabilities	13,530	5,942
Lease liability	472	—
Total current liabilities	24,417	13,582
Long-term liabilities:		
Lease liability	3,086	—

Deferred rent	—	372
Total liabilities	<u>27,503</u>	<u>13,954</u>
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at December 31, 2019 and 2018	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 43,996,753 and 34,410,725 shares issued and outstanding December 31, 2019 and 2018, respectively	44	34
Additional paid-in capital	606,307	430,824
Accumulated deficit	<u>(325,331)</u>	<u>(184,602)</u>
Total stockholders' equity	<u>281,020</u>	<u>246,256</u>
Total liabilities and stockholders' equity	<u>\$ 308,523</u>	<u>\$ 260,210</u>

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