



Rhythm Pharmaceuticals Reports Third Quarter 2020 Financial Results

November 2, 2020

-- NDA for setmelanotide for POMC and LEPR deficiency obesities under review, with PDUFA goal date of November 27, 2020 --

-- Appointed Jennifer Chien and Yann Mazabraud to co-lead global integrated commercial strategies --

-- On track to announce topline data from pivotal Phase 3 trial in Bardet-Biedl and Alström syndromes late in fourth quarter of 2020 or early in first quarter 2021 --

-- Plan to announce data from multiple cohorts in ongoing Phase 2 basket study early in first quarter 2021 --

BOSTON, Nov. 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 third quarter ended September 30, 2020.

"At Rhythm, we are making meaningful progress toward our goal of changing how the world thinks about obesity by focusing attention on the critical role that genetics can play in driving disease," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. "We believe we are now on the cusp of a significant and validating inflection point with the potential approval of setmelanotide as the first-ever FDA-approved therapy for rare genetic disorders of obesity expected later this month. In parallel, we are looking forward to key readouts across our next wave of indications, with topline results from our pivotal Phase 3 trial in Bardet-Biedl and Alström syndromes expected late in the fourth quarter of 2020 or early in the first quarter 2021, and data from our Basket Study now expected early in the first quarter of 2021. We are hopeful that these data will further demonstrate setmelanotide's potential to treat the severe obesity and insatiable hunger that characterize MC4R pathway-driven rare genetic disorders of obesity and enable better care for the patients and families affected by these conditions."

Recent Highlights:

- In October, the Company announced that results from two pivotal Phase 3 studies evaluating setmelanotide in pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity were published in [The Lancet Diabetes & Endocrinology](#). Rhythm believes these data validate setmelanotide's potential to be the first approved therapy for people living with POMC or LEPR deficiency obesities.
- Rhythm recently announced the addition of two senior biopharmaceutical executives to its leadership team to lead global integrated commercial strategies in key markets. In October, the Company announced the appointment of Jennifer Chien as Executive Vice President, Head of North America, effective Nov. 9, and in September, the Company announced the appointment of Yann Mazabraud as Executive Vice President, Head of International.

Upcoming Regulatory Milestones:

- The Prescription Drug User Fee Act (PDUFA) target date is November 27, 2020, which is when the U.S. Food and Drug Administration (FDA) is scheduled to act on the Company's New Drug Application (NDA) for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity. As previously announced, the FDA granted rare pediatric disease designations for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity, which means Rhythm would be eligible to receive one priority review voucher, pending approval.
- Rhythm's Marketing Authorization Application (MAA) for setmelanotide for the treatment of POMC deficiency obesity and LEPR deficiency obesity is currently under review by the European Medicines Agency (EMA). As is frequently the case with filings that initially receive accelerated assessment by the EMA, Rhythm's MAA recently has reverted from an accelerated assessment to a standard review.

Upcoming Clinical Milestones:

- Rhythm will present three abstracts at The Obesity Society's ObesityWeek® 2020, being held virtually from Nov. 2-6. Presentations include full data from the Phase 2 trial of a once-weekly formulation of setmelanotide, which will be presented in an oral presentation at 9:45 a.m. ET on Thursday, Nov. 5, as well as updated data from the long-term extension study of setmelanotide in POMC deficiency obesity and new data showing there is no treatment-related effect of setmelanotide on depression or suicidality, both of which will be presented in poster sessions, beginning at 12 noon ET on Nov. 3.
- Rhythm expects to report topline data from its combined pivotal Phase 3 trial evaluating setmelanotide in BBS and Alström syndrome late in the fourth quarter of 2020 or early in the first quarter of 2021.
- Rhythm now plans to provide an update on its ongoing exploratory Phase 2 Basket Study and genetic sequencing efforts early in the first quarter of 2021. This update will include new data from individuals living with HET obesity due to a loss-of-

function variant in one of two alleles on the *POMC*, *PCSK1* or *LEPR* gene, as well as *SRC1* and *SH2B1* deficiency obesities. The update will also include data from Company's sequencing efforts, which now includes samples from more than 30,000 individuals with severe obesity.

- Rhythm currently is evaluating next steps for the pre-clinical development of RM-853, its ghrelin o-acyltransferase (GOAT) inhibitor.

Third Quarter 2020 Financial Results:

- **Cash Position:** As of September 30, 2020, cash, cash equivalents and short-term investments were \$201.8 million, as compared to \$228.6 million as of June 30, 2020. This decrease reflects \$27.0 million of cash used to fund operating activities in the third quarter 2020. Based on its current clinical development plans, Rhythm expects that its existing cash, cash equivalents and short-term investments will enable it to fund its operations at least through the end of 2021.
- **R&D Expenses:** R&D expenses were \$23.0 million for the third quarter of 2020 as compared to \$26.6 million for the third quarter of 2019. The decrease in R&D spending was primarily attributed to a \$5.2 million reduction in clinical trial expenses associated with the Company's Go-ID genotyping study and the once-weekly formulation study and a decrease of \$3.2 million related to translational research and genetic sequencing efforts. These decreases were partially offset by an increase of \$3.6 million related to purchases of setmelanotide API and an increase of \$1.0 million related to a milestone payment associated with the license agreement with Ipsen on filing the MAA for setmelanotide for the treatment of *POMC* and *LEPR* deficiency obesities.
- **S,G&A Expenses:** S,G&A expenses were \$11.3 million for the third quarter of 2020 as compared to \$10.5 million for the third quarter of 2019. This increase was primarily due to an increase of \$1.3 million in stock compensation expense associated with a grant to the Company's new chief executive officer and other new hire grants during the period as well as a modification of stock options for the Company's former chief commercial officer, partially offset by a decrease of \$0.8 million in consulting services.
- **Net Loss:** Net loss was \$33.8 million for the third quarter of 2020, or a net loss per basic and diluted share of \$0.77, as compared to a net loss of \$36.0 million for the third quarter of 2019, or a net loss per basic and diluted share of \$1.04.

Year to Date Financial Results:

- **Cash Position:** As of September 30, 2020, cash, cash equivalents and short-term investments were \$201.8 million, as compared to \$292.5 million as of December 31, 2019. This decrease reflects \$92.9 million of cash used to fund operating expenses in 2020.
- **R&D Expenses:** R&D expenses were \$68.5 million for the nine months ended September 30, 2020, as compared to \$84.6 million for the nine months ended September 30, 2019. The decrease was primarily due to a decrease of \$16.0 million related to the GO-ID genotyping study, the *POMC* and *LEPR* clinical studies and the once-weekly formulation study, and a decrease of \$7.5 million related to translational research, pathway validation and genetic sequencing efforts. These decreases were partially offset by an increase of \$3.0 million related to a milestone expenses associated with the license agreement with Ipsen on filing the NDA with the FDA and the MAA with the EMA for setmelanotide for the treatment of *POMC* and *LEPR* deficiency obesities, an increase of \$2.5 million in spending related to the Phase 2 Basket Study, and an increase of \$1.2 million related to purchases of setmelanotide API.
- **S,G&A Expenses:** S,G&A expenses were \$33.0 million for the nine months ended September 30, 2020, as compared to \$27.1 million for the nine months ended September 30, 2019. The increase was primarily due to an accounting charge of \$4.0 million related to the separation agreement and modification of stock options for the Company's former chief executive officer and chief commercial officer, and an increase of \$1.0 million in various consulting and professional services related to legal and IT support costs.
- **Net Loss:** Net loss was \$99.1 million for the nine months ended September 30, 2020, or a net loss per basic and diluted share of \$2.25, as compared to a net loss of \$107.8 million for the nine months ended September 30, 2019, or a net loss per basic and diluted share of \$3.13.

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 FDA has accepted for filing an NDA for setmelanotide for the treatment of *POMC* deficiency obesity and *LEPR* deficiency obesity with Priority Review and assigned a PDUFA goal date of November 27, 2020. Rhythm also submitted an MAA for setmelanotide to treat individuals living with *POMC* deficiency obesity or *LEPR* deficiency obesity to the EMA in June 2020. Rhythm is also evaluating setmelanotide for reduction in hunger and body weight in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected late 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 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 forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding the potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, including the anticipated timing for

release of clinical trial data and our expectations surrounding potential regulatory approvals and timing thereof, our business strategy and plans, including regarding commercialization of setmelanotide, management changes, our participation in upcoming events and presentations, and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations. 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, the impact of our management transition, 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, our liquidity and expenses, the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and general economic conditions, and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2020 and our other filings 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.

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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,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	\$ 22,995	\$ 26,572	\$ 68,496	\$ 84,641
Selling, general, and administrative	11,289	10,535	33,006	27,135
Total operating expenses	<u>34,284</u>	<u>37,107</u>	<u>101,502</u>	<u>111,776</u>
Loss from operations	(34,284)	(37,107)	(101,502)	(111,776)
Other income (expense):				
Interest income, net	466	1,104	2,403	4,003
Total other income, net	<u>466</u>	<u>1,104</u>	<u>2,403</u>	<u>4,003</u>
Net loss	<u>\$ (33,818)</u>	<u>\$ (36,003)</u>	<u>\$ (99,099)</u>	<u>\$ (107,773)</u>
Net loss per share, basic and diluted	<u>\$ (0.77)</u>	<u>\$ (1.04)</u>	<u>\$ (2.25)</u>	<u>\$ (3.13)</u>
Weighted-average common shares outstanding, basic and diluted	<u>44,142,334</u>	<u>34,541,765</u>	<u>44,097,178</u>	<u>34,470,995</u>
Other comprehensive loss:				
Net loss	\$ (33,818)	\$ (36,003)	\$ (99,099)	\$ (107,773)
Unrealized (loss) gain on marketable securities	(392)	—	238	—
Comprehensive loss	<u>\$ (34,210)</u>	<u>\$ (36,003)</u>	<u>\$ (98,861)</u>	<u>\$ (107,773)</u>

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

	September 30,	December 31,
	2020	2019
	<hr/>	<hr/>
Assets		
Current assets:		
Cash and cash equivalents	\$ 67,670	\$ 62,294
Short-term investments	134,114	230,165
Prepaid expenses and other current assets	8,130	9,945
Total current assets	<hr/> 209,914	<hr/> 302,404
Property and equipment, net	3,289	3,671
Right-of-use asset	1,871	2,045
Restricted cash	403	403
Total assets	<hr/> \$ 215,477 <hr/>	<hr/> \$ 308,523 <hr/>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 3,793	\$ 10,415
Accrued expenses and other current liabilities	11,536	13,530
Lease liability	519	472
Total current liabilities	<hr/> 15,848	<hr/> 24,417
Long-term liabilities:		
Lease liability	2,692	3,086
Total liabilities	<hr/> 18,540	<hr/> 27,503
Commitments and contingencies		
Stockholders' equity:		
Preferred Stock, \$0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2020 and December 31, 2019	—	—
Common stock, \$0.001 par value: 120,000,000 shares authorized; 44,204,745 and 43,996,753 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	44	44
Additional paid-in capital	621,085	606,307
Accumulated other comprehensive income	238	—
Accumulated deficit	<hr/> (424,430)	<hr/> (325,331)
Total stockholders' equity	<hr/> 196,937	<hr/> 281,020
Total liabilities and stockholders' equity	<hr/> \$ 215,477 <hr/>	<hr/> \$ 308,523 <hr/>



Source: Rhythm Pharmaceuticals, Inc.