



Rhythm Pharmaceuticals Reports Third Quarter 2017 Financial Results

November 14, 2017

*- Successfully Completed Upsized Initial Public Offering Raising \$137.8 Million in Gross Proceeds -
- Presented Preliminary Data from Phase 2 Clinical Trial of Setmelanotide in Bardet-Biedl Syndrome (BBS) at ObesityWeek -*

BOSTON, Nov. 14, 2017 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (NASDAQ:RYTM), a biopharmaceutical company developing peptide therapeutics for rare genetic deficiencies that result in life-threatening metabolic disorders, today reported financial results and provided a business update for the third quarter ended September 30, 2017.

“Our third quarter and recent achievements represent meaningful progress toward our goal of developing and commercializing setmelanotide for patients with rare, genetic disorders of obesity,” said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. “We recently announced promising preliminary results from our ongoing Phase 2 proof-of-concept study in patients with Bardet-Biedl syndrome, which build on our prior experiences establishing proof-of-concept with setmelanotide in patients with POMC and LepR deficiency obesity and further support its potential as a replacement therapy for the treatment of MC4 pathway deficiencies. In addition, in October, we completed our upsized initial public offering, providing us the financial strength to advance our clinical and market development efforts into the first half of 2019. We are committed to addressing the unmet needs facing patients with early onset obesity and hyperphagia, and look forward to advancing our broad clinical program for setmelanotide in six monogenic MC4 pathway deficiencies.”

Recent Business Highlights:

- In October 2017, Rhythm presented preliminary data from its ongoing Phase 2 proof-of-concept study evaluating the safety and efficacy of setmelanotide for the treatment of Bardet-Biedl syndrome (BBS) at ObesityWeek 2017 in Washington, D.C. Of five evaluable BBS patients treated with setmelanotide, four experienced substantial weight loss and all five experienced improvements in their hunger scores. The data also showed that setmelanotide was well-tolerated.
- In October 2017, Rhythm completed an upsized initial public offering of common stock at \$17.00 per share, raising net proceeds of approximately \$125.8 million, after deducting underwriting discounts, commissions and estimated offering expenses.
- In August 2017, Rhythm announced the appointments of Hunter Smith as Chief Financial Officer and Nithya Desikan as Chief Commercial Officer.

Selected Third Quarter 2017 Financial Results:

- Cash Position: As of September 30, 2017, cash, cash equivalents and short-term investments were \$30.4 million, as compared to \$10.5 million as of December 31, 2016. Cash, cash equivalents and short-term investments as of September 30, 2017 do not include total net proceeds of approximately \$125.8 million from the Company's initial public offering of common stock, which was completed in October 2017.
- R&D Expenses: R&D expenses were \$6.0 million for the third quarter of 2017, as compared to \$5.4 million for the third quarter of 2016. This increase was primarily due to the initiation of additional new clinical trials in 2017 and an increase in other commercial and development activities associated with setmelanotide, offset by the completion of some preclinical research studies. The Company hired additional personnel in the clinical and market development departments during the third quarter of 2017.
- G&A Expenses: G&A expenses were \$2.3 million for the third quarter of 2017, as compared to \$1.0 million for the third quarter of 2016. This increase was primarily due to stock-based compensation expense related to the revaluation of certain option grants to the former President upon changing from an employee to nonemployee status, the legal costs related to the distribution by Rhythm's prior parent company, Rhythm Holdings LLC, of common shares of Rhythm to its members and general administrative expenses due to the increase in headcount during the third quarter of 2017.
- Net Loss: Net loss was \$10.0 million for the third quarter of 2017, or \$1.78 per basic and diluted share, as compared to a net loss of \$6.4 million for the third quarter of 2016, or \$0.71 per basic and diluted share.

About Rhythm

Rhythm is a biopharmaceutical company focused on the development and commercialization of peptide therapeutics for the treatment of rare genetic deficiencies that result in life-threatening metabolic disorders. Rhythm's lead peptide product candidate is setmelanotide, a first-in-class melanocortin-4 receptor (MC4R) agonist for the treatment of rare genetic disorders of obesity. Rhythm supports The Genetic Obesity Project (www.GeneticObesity.com), which is dedicated to improving the understanding of severe obesity that is caused by specific genetic defects. The company is based in Boston, MA.

Forward-Looking Statements:

This press release contains forward looking statements 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, including statements regarding our expectations about our use of cash, our advancement of our

clinical programs, our goals to develop and commercialize setmelanotide, and other statements identified by words such as “anticipates,” “believes,” “could,” “estimates,” “expects,” “intends,” “may,” “might,” “likely,” “plans,” “potential,” “predicts,” “projects,” “seeks,” “should,” “target,” “will,” “would,” or similar expressions and the negatives of those terms. Forward-looking statements are not promises or guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control, and which could cause actual results to differ materially from those contemplated in such forward-looking statements. These factors include our limited operating history, our ability to obtain necessary funding, our ability to generate positive clinical trial results for setmelanotide, risks associated with the small population of patients we are targeting, and our ability to establish that population and identify patients, changes in laws and regulations to which we are subject, our ability to identify additional product candidates, and other risks set forth under the heading “Risk Factors” of our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017. Our actual results could differ materially from the results described in or implied by such forward-looking statements. Forward-looking statements speak only as of the date hereof, and, except as required by law, we undertake no obligation to update or revise these forward-looking statements.

Rhythm Pharmaceuticals, Inc.

Condensed Balance Sheets

(in thousands, except share and per share data)

	(Unaudited)	
	September 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	\$ 27,914	\$ 6,540
Short-term investments	2,501	3,997
Prepaid expenses and other current assets	1,621	638
Total current assets	32,036	11,175
Property, plant and equipment, net	845	930
Deferred issuance costs	1,698	9
Restricted cash	225	225
Total assets	\$ 34,804	\$ 12,339
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	\$ 2,271	\$ 1,895
Due to related party	—	105
Deferred rent	81	76
Accrued expenses and other current liabilities	3,108	2,655
Total current liabilities	5,460	4,731
Long-term liabilities:		
Deferred rent	250	311
Total liabilities	5,710	5,042
Commitments and contingencies		
Preferred stock:		
Series A Convertible Preferred Stock, \$1.00 par value: 80,950,000 shares authorized; 80,949,999 shares issued and outstanding at September 30, 2017 and 40,000,000 shares issued and outstanding at December 31, 2016; (aggregate liquidation preference of \$88,864 and \$44,129 at September 30, 2017 and December 31, 2016 respectively)	80,950	40,000
Stockholders' equity (deficit):		
Common stock, \$0.001 par value: 29,919,979 shares authorized; 1,770,302 and 10,196,292 shares issued and outstanding and September 30, 2017 and December 31, 2016, respectively	2	10
Series A-1 Convertible Junior Preferred Stock, \$0.001 par value, 78,666,209 shares authorized; 78,666,209 and no shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	79	—
Additional paid-in capital	47,784	43,830

Accumulated deficit	(99,721)	(76,543)
Total stockholders' equity (deficit)	(51,856)	(32,703)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 34,804</u>	<u>\$ 12,339</u>

Rhythm Pharmaceuticals, Inc.

Condensed 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,	
	2017	2016	2017	2016
Operating expenses:				
Research and development	\$ 5,971	\$ 5,419	\$ 16,241	\$ 13,963
General and administrative	2,315	982	5,188	3,567
Total operating expenses	<u>8,286</u>	<u>6,401</u>	<u>21,429</u>	<u>17,530</u>
Loss from operations	(8,286)	(6,401)	(21,429)	(17,530)
Other income (expense):				
Revaluation of Series A Investor Instrument	(1,781)	—	(1,863)	—
Interest income, net	51	10	114	24
Total other income (expense):	<u>(1,730)</u>	<u>10</u>	<u>(1,749)</u>	<u>24</u>
Net loss and comprehensive loss	<u>\$ (10,016)</u>	<u>\$ (6,391)</u>	<u>\$ (23,178)</u>	<u>\$ (17,506)</u>
Net loss attributable to common stockholders	<u>\$ (11,429)</u>	<u>\$ (7,191)</u>	<u>\$ (26,963)</u>	<u>\$ (19,902)</u>
Net loss attributable to common stockholders per common share, basic and diluted	<u>\$ (1.78)</u>	<u>\$ (0.71)</u>	<u>\$ (3.02)</u>	<u>\$ (1.95)</u>
Weighted average common shares outstanding, basic and diluted	<u>6,404,254</u>	<u>10,196,292</u>	<u>8,918,389</u>	<u>10,196,292</u>

Rhythm Pharmaceuticals, Inc.

Condensed Statements of Cash Flows

(in thousands, except share and per share data)

(Unaudited)

	Nine Months Ended September 30,	
	2017	2016
Operating activities		
Net loss	\$ (23,178)	\$ (17,506)
Adjustments to reconcile net loss to cash used in operating activities:		
Stock-based compensation expense	1,569	859
Depreciation and amortization	163	89
Non-cash rent expense	(56)	29
Mark to market revaluation of Series A Investor Instrument	1,863	—

Changes in operating assets and liabilities:

Prepaid expenses and other current assets	(972)	(272)
Deferred issuance costs	(1,689)	(348)
Tenant improvement allowance	—	376
Accounts payable, accrued expenses and other current liabilities	830	(335)
Deferred grant income	—	(71)
Due to related parties	(105)	139
Net cash used in operating activities	<u>(21,575)</u>	<u>(17,040)</u>
Investing activities		
Purchases of short-term investments	(13,021)	(11,211)
Maturities of short-term investments	14,506	7,054
Purchases of property, plant and equipment	(78)	(1,057)
Net cash provided by (used in) investing activities	<u>1,407</u>	<u>(5,214)</u>
Financing activities		
Net proceeds from issuance of Series A Convertible Preferred Stock	40,842	—
Proceeds from the exercise of stock options	700	—
Net cash provided by financing activities	<u>41,542</u>	<u>—</u>
Net increase (decrease) in cash and cash equivalents	21,374	(22,254)
Cash and cash equivalents at beginning of period	6,540	34,869
Cash and cash equivalents at end of period	<u>\$ 27,914</u>	<u>\$ 12,615</u>

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Rhythm Pharmaceuticals, Inc.