



FINANCIAL TEAR SHEET

Corporate Profile

Rhythm is a biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity.

Our lead product candidate, setmelanotide, is an investigational, first-in-class melanocortin-4 receptor (MC4R) agonist in development for the treatment of rare genetic disorders of obesity. We believe that setmelanotide, for which we have exclusive worldwide rights, has the potential to serve as replacement therapy that may restore lost activity in the MC4 pathway, a key biological pathway in humans that regulates weight by increasing energy expenditure and reducing appetite. Variants in genes within the MC4 pathway result in the disruption of satiety signals and energy homeostasis in the body, which leads to unrelenting hunger, known as hyperphagia, and to severe, early-onset obesity.

We are initially focused on 6 rare genetic disorders of obesity for which there are currently no effective or approved treatments:

- Pro-opiomelanocortin (POMC) deficiency obesity
- Leptin receptor (LEPR) deficiency obesity
- Bardet-Biedl syndrome
- Alström syndrome
- POMC heterozygous deficiency obesity
- POMC epigenetic disorders

We have demonstrated proof of concept in Phase 2 clinical trials in POMC deficiency obesity, LEPR deficiency obesity, Bardet-Biedl Syndrome, and Alström Syndrome in which setmelanotide demonstrated reductions in both weight and hunger. Setmelanotide has been granted Breakthrough Therapy Designation for the treatment of obesity associated with genetic defects upstream of the MC4 receptor in the leptin-melanocortin pathway, which includes pro-opiomelanocortin (POMC) deficiency obesity, leptin receptor (LEPR) deficiency obesity, Bardet-Biedl Syndrome, and Alström Syndrome.

Setmelanotide has also been granted PRiority MEDicines (PRIME) designation for the treatment of obesity associated with pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity by the European Medicines Agency (EMA). The PRIME program was launched by the EMA in 2016 to provide early and enhanced support to optimize the development of eligible medicines, speed up their evaluation, and contribute to timely patient access.

Setmelanotide is currently in Phase 3 clinical trials for POMC deficiency obesity and LEPR deficiency obesity. We have also initiated a Phase 2 proof of concept basket study in Alström syndrome, POMC heterozygous deficiency obesity, and POMC epigenetic disorders. Additionally, we are evaluating another study for Prader-Willi syndrome.

We are currently assessing opportunities to further evaluate setmelanotide in Prader-Willi syndrome and plan to pursue these in parallel with the development of RM-853. Additionally, given the distinct mechanisms of action for setmelanotide and RM-853, we will explore opportunities to evaluate the two compounds in combination,

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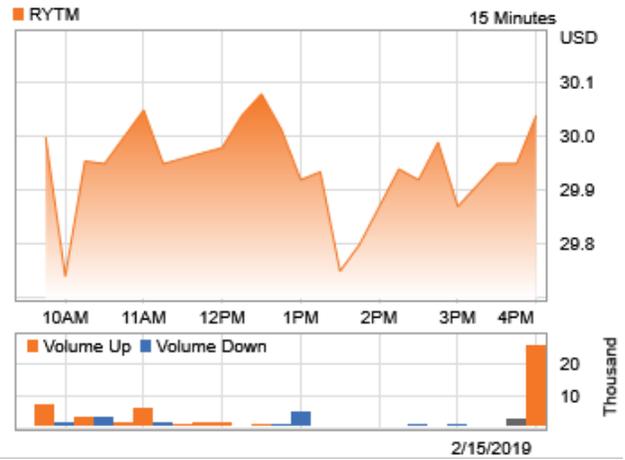
as there may be complementary effects.

Stock Performance

RYTM (Common Stock)

Exchange	NASDAQ (US Dollar)
Price	\$30.04
Change (%)	▲ 0.33 (1.11%)
Volume	85,269
52 Week Low	\$16.80
Market Cap	\$1,033,698,179
Rolling EPS	-2.05
PE Ratio	0
Shares Outstanding	34,410,725

Data as of 02/15/19 4:00 p.m. ET



Recent Headlines & Events

01/04/19

Rhythm Pharmaceuticals to Outline Expected 2019 Milestones and Review Updated Clinical Data in Bardet-Biedl Syndrome at 37th Annual J.P. Morgan Healthcare Conference

12/13/18

Rhythm Pharmaceuticals Announces First Patient Enrolled in Pivotal Phase 3 Clinical Trial Evaluating Setmelanotide in Bardet-Biedl and Alström Syndromes

12/06/18

Rhythm Pharmaceuticals to Host Key Opinion Leader Symposium on MC4R Pathway and Patient Experience with Rare Genetic Disorders of Obesity, Including Bardet-Biedl Syndrome

There are currently no events scheduled.

SEC Filings

Filing Date	Form
02/15/19	4
02/15/19	4
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Data provided by Nasdaq. Minimum 15 minutes delayed.