



Rhythm Pharmaceuticals to Provide an Update on Genetic Sequencing Efforts and Expansion of Phase 2 Basket Study with Four Additional Rare MC4R Pathway-driven Disorders

September 9, 2019

- Plan to expand ongoing Phase 2 Basket Study with four additional rare indications --*
- Sequenced nearly 14,000 people with early-onset, severe obesity; Yield suggests potential significant opportunity for setmelanotide across new indications --*
- Rhythm to host and webcast R&D breakfast event on Wednesday, Sept. 25 --*

BOSTON, Sept. 09, 2019 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq:RYTM), a biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity, today announced plans to expand its Phase 2 Basket Study evaluating setmelanotide to include four additional rare melanocortin-4 receptor (MC4R) pathway-driven disorders. The company will host and webcast a Research & Development (R&D) event to provide an update on genetic sequencing results and the Phase 2 Basket Study on Wednesday, Sept. 25, from 8 to 10 a.m. in New York.

Company management will provide details on:

- Four new indications added to the Phase 2 Basket Study: SRC1 deficiency obesity; SH2B1 deficiency obesity; MC4R deficiency obesity; and Smith-Magenis syndrome;
- Yield from sequencing nearly 14,000 patients with early-onset, severe obesity for POMC, LEPR and MC4R pathway HETs obesities, as well as these four new indications;
- Disease prevalence and epidemiology estimates for patients with genetic variants tied to these indications based on Rhythm's sequencing data; and
- Patient identification efforts for Bardet-Biedl syndrome and Alström syndrome, two indications currently being studied in a pivotal, Phase 3 trial.

"Rhythm set out to transform the care for patients with rare genetic disorders of obesity based on our understanding of how the MC4R pathway regulates hunger and body weight," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "In parallel with our ongoing efforts to progress setmelanotide through late-stage clinical trials, we have been working with our partners to sequence people living with early-onset, severe obesity to uncover more rare genetic disorders of obesity. We look forward to providing additional detail on our patient identification and community building efforts, as well as our next indications for our expanded Basket trial, at this R&D event. We are leveraging our strong scientific foundation to deliver setmelanotide as potentially the first approved therapeutic for MC4R pathway-driven disorders of obesity."

Rhythm remains on track to share the full data from its Phase 3 clinical trials in Pro-opiomelanocortin (POMC) deficiency obesity and Leptin receptor (LEPR) deficiency obesity in a forthcoming publication and in a presentation at an upcoming medical meeting.

A live audio webcast of the R&D event will be available under "Events & Presentations" in the Investors & Media section of the Company's website at www.rhythmtx.com. A replay of the webcast will be available on the Rhythm website for 30 days following the event.

About Rhythm

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. The company plans to complete its first rolling new drug application (NDA) submission to the U.S. Food and Drug Administration 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 Bardet-Biedl syndrome 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 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 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 of patients in clinical trials and submission of an NDA, its ongoing efforts related to patient identification, prevalence and epidemiology estimates, the release of results of clinical trials, and the use of setmelanotide as a treatment for additional indications. 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.

Corporate Contact:

David Connolly
Head of Investor Relations and Corporate Communications
Rhythm Pharmaceuticals, Inc.
857-264-4280
dconnolly@rhythmtx.com

Investor Contact:

Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200
hannah.deresiewicz@sternir.com

Media Contact:

Adam Daley
Berry & Company Public Relations
212-253-8881
adaley@berrypr.com



Source: Rhythm Pharmaceuticals, Inc.