



Rhythm Pharmaceuticals Completes Pivotal Enrollment in Two Ongoing Phase 3 Clinical Trials Evaluating Setmelanotide in Rare Genetic Disorders of Obesity

June 14, 2018

One-Year Data from Cohorts of 10 Enrolled Patients to Support Concurrent New Drug Application (NDA) Filings in POMC and LEPR Deficiency Obesity

BOSTON, June 14, 2018 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (NASDAQ:RYTM), a biopharmaceutical company focused on the development and commercialization of therapeutics for the treatment of rare genetic disorders of obesity, today announced that it has completed enrollment of the pivotal cohorts of 10 patients in two separate, ongoing, registration-enabling Phase 3 clinical trials evaluating setmelanotide in pro-opiomelanocortin (POMC) and leptin receptor (LEPR) deficiency obesity. POMC and LEPR deficiency obesity are two ultra-rare genetic disorders that result in excess hunger, also known as hyperphagia, and severe, early-onset obesity. Setmelanotide is a first-in-class melanocortin-4 receptor (MC4R) agonist, for which the U.S. Food and Drug Administration (FDA) has granted both Breakthrough Therapy Designation and Orphan Drug Designation in POMC and LEPR deficiency obesity.

Rhythm expects to report initial data from the Phase 3 trials of both POMC and LEPR deficiency obesity in the third quarter of 2019. Rhythm then plans to submit concurrent New Drug Application (NDA) filings to the FDA for setmelanotide in patients with these indications based on one-year data from these pivotal cohorts of 10 patients. In addition, the Company plans to continue enrolling supplemental patients in both trials who may not complete one year of treatment at the time of NDA filing, including patients between six and 11 years of age, to provide additional data regarding the use of setmelanotide in people living with POMC and LEPR deficiency obesity.

"Patients with MC4R pathway deficiencies represent an underdiagnosed population, whose early-onset obesity and excess hunger impairs their lives," said Keith Gottesdiener, M.D., Chief Executive Officer of Rhythm. "Completing enrollment in both of our pivotal Phase 3 trials in POMC and LEPR deficiency obesity marks a key milestone for Rhythm and for people living with these rare genetic disorders, as it brings us one step closer to our goal of providing setmelanotide as a first-in-class therapy that has the potential to reestablish both weight and appetite control. We are particularly encouraged to have completed enrollment ahead of schedule in our LEPR deficiency obesity trial, which speaks to the significant need for a new, disease-modifying therapy, and which we anticipate will allow us to submit concurrent NDA filings in POMC and LEPR deficiency obesity and potentially to accelerate the availability of setmelanotide for LEPR patients. We look forward to further evaluating setmelanotide's therapeutic potential as we progress our ongoing studies and work to more broadly understand the benefits of setmelanotide, including in the pediatric setting."

The open-label, single-arm, multinational Phase 3 trials evaluating the safety and efficacy of setmelanotide in POMC and LEPR deficiency obesity share the same trial design. The primary endpoint in both trials is a responder analysis for weight, defined as patients achieving a 10 percent change from baseline. The first secondary endpoint in both trials is the mean percentage change in weight. Hunger scores are also key secondary endpoints.

Rhythm is also evaluating setmelanotide in four additional rare genetic disorders of obesity. Rhythm expects to initiate a Phase 3 study evaluating setmelanotide in Bardet-Biedl Syndrome in 2018. The Company has treated a number of the first patients in a Phase 2 proof-of-concept basket study evaluating setmelanotide in Alström Syndrome, POMC epigenetic disorders and POMC heterozygous deficiency obesity and expects to announce initial data in each indication in the second quarter of 2018. In addition, Rhythm has launched efforts to build a patient registry, Tracing the Effect of the MC4R Pathway in Obesity (TEMPO), and is supporting [The Genetic Obesity Project](#) and the [Go-ID Genotyping Study](#).

About Rhythm Pharmaceuticals

Rhythm is a biopharmaceutical company focused on the development and commercialization of therapeutics for the treatment of rare genetic disorders of obesity. Rhythm is currently evaluating the efficacy and safety of setmelanotide, the Company's first-in-class melanocortin-4 receptor (MC4R) agonist, in Phase 3 studies in patients with pro-opiomelanocortin (POMC) deficiency obesity (which includes deficiencies in both the POMC and PCSK1 genes) and leptin receptor (LEPR) deficiency obesity. Rhythm also supports The Genetic Obesity Project (www.GeneticObesity.com), which is dedicated to improving the understanding of severe obesity that results from specific genetic disorders. 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 expectations regarding its preparation of an NDA filing with the FDA, its plans and timing regarding patient enrollment and announcement of data in clinical trials, and the potential acceleration of availability of setmelanotide, and related statements. Statements using words such as "expect", "anticipate", "believe", "may", "will", "plan", "goal" 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 outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, our use of cash 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.

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