Rhythm Pharmaceuticals Announces Late-Breaking Data Presentations at ENDO 2021

March 9, 2021

BOSTON, March 09, 2021 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic diseases of obesity, today announced that three late-breaking abstracts have been accepted for presentation at the 103rd Annual Meeting and Expo of the Endocrine Society (ENDO 2021) to be held virtually March 20-23.

In a live oral presentation, Sadaf Farooqi, M.D., Ph.D., professor at the Wellcome-MRC Institute of Metabolic Science and NIHR Cambridge Biomedical Research Centre, will present clinical data from Rhythm's Phase 2 study evaluating setmelanotide in individuals living with heterozygous (HET) obesity due to genetic variants in one of two alleles of the *POMC*, *PCSK1* or *LEPR* gene.

- **Session OR24 - Emerging Endocrine Therapies Across the Lifespan**
  March 20, 2021, 11:30 a.m. - 11:40 a.m.
  "Effects of Setmelanotide in Patients With POMC, PCSK1, or LEPR Heterozygous Deficiency Obesity in a Phase 2 Study"

In addition, Robert Haws, M.D., Clinical Research Center at the Marshfield Clinic Research Institute, will present an on-demand poster detailing top-line data from a Phase 3 trial that evaluated setmelanotide in patients with Bardet-Biedl Syndrome or Alström Syndrome.

- **Session P02 - Integrated Physiology of Obesity and Metabolic Disease**
  Robert Haws, M.D., Clinical Research Center at the Marshfield Clinic Research Institute
  "A Phase 3 Trial in Participants With Obesity Due to Bardet-Biedl Syndrome or Alström Syndrome: Efficacy and Safety of the Melanocortin 4 Receptor Agonist Setmelanotide"

Also, Karine Clément, M.D., Ph.D., Pitié-Salpêtrière Hospital and Sorbonne Université in Paris, will present an on-demand poster with a further analysis of safety data from two phase 3 trials evaluating setmelanotide in patients with obesity due to POMC or LEPR deficiency.

- **Session P02 - Integrated Physiology of Obesity and Metabolic Disease**
  Karine Clément, M.D., Ph.D., Pitié-Salpêtrière Hospital and Sorbonne Université in Paris
  "Timing of Onset of Adverse Events With Setmelanotide, an MC4R Agonist, in Patients With Severe Obesity Due to LEPR or POMC Deficiency"

The poster presentations will be available for on-demand viewing on the ENDO 2021 website, https://www.endocrine.org/endo2021, beginning at 11 a.m. on Saturday, March 20.

Rhythm will also provide additional information about rare genetic diseases of obesity, genetic testing, and the company's clinical development pipeline at its virtual booth throughout the ENDO 2021 meeting.

About Rhythm Pharmaceuticals
Rhythm is a commercial-stage biopharmaceutical company committed to transforming the treatment paradigm for patients living with rare genetic diseases of obesity. Early-onset severe obesity may result from genetic variants within the melanocortin-4 receptor (MC4R) pathway, a key hypothalamic pathway that regulates hunger, caloric intake, and energy expenditure, consequently affecting body weight. Rhythm is developing setmelanotide for the treatment of rare genetic diseases of obesity that arise due to an impaired pathway, as setmelanotide has shown the potential to restore impaired pathway function.

Setmelanotide was approved by the FDA in late 2020 for chronic weight management in adult and pediatric patients 6 years of age and older with obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency confirmed by genetic testing, under the brand name IMCIVREE ™. In addition, Rhythm is advancing a broad clinical development program with phase 2 and 3 trials evaluating setmelanotide for the treatment of obesity due to a deficiency in one of 36 additional genes associated with the MC4R pathway. Rhythm has created and is leveraging the largest known obesity DNA database - now with approximately 37,500 sequencing samples - to improve the understanding, diagnosis, and care of patients with severe obesity due to certain genetic deficiencies.

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.

IMCIVREE™ (setmelanotide) Indication
IMCIVREE is indicated for chronic weight management in adult and pediatric patients 6 years of age and older with obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency. The condition must be confirmed by genetic testing demonstrating variants in *POMC*, *PCSK1*, or *LEPR* genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS).

Limitations of Use

IMCIVREE is not indicated for the treatment of patients with the following conditions as IMCIVREE would not be expected to be effective:

- Obesity due to suspected POMC, PCSK1, or LEPR deficiency with *POMC*, *PCSK1*, or *LEPR* variants classified as benign or likely benign;
- Other types of obesity not related to POMC, PCSK1 or LEPR deficiency, including obesity associated with other genetic syndromes and general (polygenic) obesity.

Important Safety Information

WARNINGS AND PRECAUTIONS

Disturbance in Sexual Arousal: Sexual adverse reactions may occur in patients treated with IMCIVREE. Spontaneous penile erections in males and sexual adverse reactions in females occurred in clinical studies with IMCIVREE. Instruct patients who have an erection lasting longer than 4 hours to seek emergency medical attention.

Depression and Suicidal Ideation: Some drugs that target the central nervous system, such as IMCIVREE, may cause depression or suicidal ideation. Monitor patients for new onset or worsening of depression. Consider discontinuing IMCIVREE if patients experience suicidal thoughts or behaviors.

Skin Pigmentation and Darkening of Pre-Existing Nevi: IMCIVREE may cause generalized increased skin pigmentation and darkening of pre-existing nevi due to its pharmacologic effect. This effect is reversible upon discontinuation of the drug. Perform a full body skin examination prior to initiation and periodically during treatment with IMCIVREE to monitor pre-existing and new skin pigmentary lesions**.**

Risk of Serious Adverse Reactions Due to Benzyl Alcohol Preservative in Neonates and Low Birth Weight Infants: IMCIVREE is not approved for use in neonates or infants.

ADVERSE REACTIONS

- The most common adverse reactions (incidence ≥23%) were injection site reactions, skin hyperpigmentation, nausea, headache, diarrhea, abdominal pain, back pain, fatigue, vomiting, depression, upper respiratory tract infection, and spontaneous penile erection.

USE IN SPECIFIC POPULATIONS
Discontinue IMCIVREE when pregnancy is recognized unless the benefits of therapy outweigh the potential risks to the fetus.
Treatment with IMCIVREE is not recommended for use while breastfeeding.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

See Full Prescribing Information for IMCIVREE.

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 our business strategy and plans and our participation in upcoming events and presentations. 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 Annual Report on Form 10-K for the year ended December 31, 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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Source: Rhythm Pharmaceuticals, Inc.