



Rhythm Pharmaceuticals Presents New Data at ESPE 2023

September 22, 2023

BOSTON, Sept. 22, 2023 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a commercial-stage biopharmaceutical company focused on transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases, today announced four oral presentations at the 61st Annual European Society for Paediatric Endocrinology (ESPE) Meeting being held September 21-23, 2023 in The Hague, Netherlands.

"We are excited to deliver multiple presentations at ESPE 2023 including statistics that deepen the understanding of the genetics of obesity from our Rare Obesity Advanced Diagnosis™ (ROAD) testing program as we learn more about the importance of genetics in patients with early-onset, severe obesity," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm.

Impact of Setmelanotide on Metabolic Syndrome Risk

Andrea Haqq, M.D., Division of Pediatric Endocrinology, University of Alberta in Canada, and Jesús Argente M.D., Ph.D., Department of Pediatrics and Pediatric Endocrinology, Universidad Autónoma de Madrid in Spain, delivered an oral presentation on the effects of setmelanotide on the metabolic syndrome severity score based on body mass index (MetS-Z-BMI) score in pediatric patients with Bardet-Biedl syndrome (BBS).

In an oral presentation titled, "Impact of Setmelanotide on Metabolic Syndrome Risk in Pediatric Patients with POMC and LEPR Deficiency," researchers led by Martin Wabitsch, M.D., Ph.D., Department of Pediatrics and Adolescent Medicine, University of Ulm in Germany, presented new findings regarding setmelanotide treatment in pediatric patients with pro-opiomelanocortin (POMC) or leptin receptor (LEPR) deficiency.

Analyses from ROAD genetic testing program

In an oral presentation titled, "Frequency of MC4R Pathway Variants in a European Cohort of Individuals with Early-Onset Severe Obesity," researchers led by Anthony Goldstone, M.D., Ph.D., Department of Brain Sciences, Imperial College London, assessed a large European-based cohort of individuals with early-onset severe obesity, finding that 20.4% of individuals sequenced carried a variant in one or more of 11 specific MC4R pathway-related genes, including 5.0% of individuals with variants classified as pathogenic, likely pathogenic or suspected pathogenic.

"Frequency of Obesity-Related Gene Variants in a European Population with Early-Onset, Severe Obesity," as presented orally by Jesús Argente M.D., Ph.D., Department of Pediatrics and Pediatric Endocrinology, Universidad Autónoma de Madrid in Spain, shows that among individuals with early-onset, severe obesity, approximately 31.4% carried variants believed to be associated with obesity.

In addition, Rhythm is hosting a satellite symposium at ESPE 2023, titled, "Hyperphagia and early-onset, severe obesity: The role of precision medicine in the treatment of leptin melanocortin-4 receptor (MC4R) pathway diseases."

About Rhythm Pharmaceuticals

Rhythm is a commercial-stage biopharmaceutical company committed to transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) diseases. Rhythm's product, IMCIVREE (setmelanotide) is approved by the U.S. Food and Drug Administration (FDA) and authorized by the European Medicines Agency (and the UK's Medicines & Healthcare Products Regulatory Agency (MHRA) for use in accordance with product labeling. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare MC4R pathway diseases, as well as a preclinical suite of investigational candidates for the treatment of congenital hyperinsulinism. Rhythm's headquarters is in Boston, MA.

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 the potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, the potential benefits of setmelanotide for patients with cardiovascular diseases and type 2 diabetes mellitus, as well as severe obesity due to certain genetic deficiencies, expectations regarding the prevalence of patients living with MC4R pathway-related gene variants, and our participation in upcoming events and presentations. Statements using words 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, risks relating to our liquidity and expenses, our ability to enroll

patients in clinical trials, the design and outcome of clinical trials, the ability to achieve necessary regulatory approvals, risks associated with data analysis and reporting, failure to identify and develop additional product candidates, unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, risks associated with the laws and regulations governing our international operations and the costs of any related compliance programs, our ability to obtain or maintain orphan drug designations for setmelanotide or to obtain or maintain exclusivity in any use, the impact of competition, risks relating to product liability lawsuits, inability to maintain our collaborations, or the failure of these collaborations, our reliance on third parties, risks relating to intellectual property, our ability to hire and retain necessary personnel, the impact of the COVID-19 pandemic and general economic conditions on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and the other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the three months ended June 30, 2023 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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