Rhythm Pharmaceuticals Announces Seven Abstracts Accepted for Presentation at ENDO 2026

May 29, 2026

BOSTON, May 29, 2026 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a global commercial-stage biopharmaceutical company focused on transforming the lives of patients living with rare neuroendocrine diseases, today announced that seven presentations, including four late-breaking abstracts, have been accepted for presentation at The Endocrine Society's Annual Meeting (ENDO 2026) taking place June 13-16, 2026 in Chicago.

Jennifer Miller, M.D., Pediatric Endocrinology, University of Florida College of Medicine, will present results from an ongoing Phase 2 trial of setmelanotide in patients with Prader-Willi syndrome (PWS), evaluating higher doses and longer treatment duration. The poster presentation will include updated results from 17 of 18 patients following six months of setmelanotide therapy.

- **Setmelanotide, a Melanocortin-4 Receptor Agonist, in Prader-Willi Syndrome: Initial Phase 2 Results**[1]
  Saturday, June 13, 2026, 12:15 PM – 1:45 p.m. CT

Dr. Vidhu Thaker, M.D., Pediatric Endocrinology, Columbia University, will present data on weight reduction outcomes in patients with acquired hypothalamic obesity (HO) after 1 year of treatment with bivamelagon, an oral MC4R agonist.

- **Weight Reduction After 1 Year of Oral Bivamelagon in Acquired Hypothalamic Obesity**
  Monday, June 15, 2026, 12:00 – 1:00 p.m. CT

Christian Roth, M.D., Seattle Children's Research Institute, will provide an oral presentation with data from the Phase 2 trial and long-term extension evaluating setmelanotide in patients with acquired HO, including outcomes after 2.5 years of treatment.

- **Long-Term Efficacy with Setmelanotide in Patients with Acquired Hypothalamic Obesity**
  Monday, June 15, 2026, 10:00 AM - 10:15 a.m. CT

Jennifer Miller, M.D., Pediatric Endocrinology, University of Florida College of Medicine, will present data from the Phase 3 trial of setmelanotide in patients with acquired HO, focusing on outcomes in participants with a history of bariatric surgery.

- **Setmelanotide Treatment in Patients with Acquired Hypothalamic Obesity and Previous Weight Loss Surgery**
  Saturday, June 13, 2026, 12:15 PM – 1:45 p.m. CT

Ashley Shoemaker, M.D., MSCI, Associate Professor of Pediatrics, Pediatric Endocrinology at Vanderbilt Health, will present results from the Phase 3 trial of setmelanotide in patients with acquired hypothalamic obesity (aHO), including changes in weight category after one year of treatment.

- **Weight Category Improvement Following Setmelanotide in Patients with Acquired Hypothalamic Obesity**
  Sunday, June 14, 2026, 12:00 – 1:00 p.m. CT

After 17 years at Vanderbilt University Medical Center, Dr. Shoemaker is set to join Rhythm as a medical director effective June 1, 2026.

Caroline Huber, Director of Value & Evidence at Rhythm Pharmaceuticals, will present two late-breaking posters of real-word evidence in Bardet-Biedl syndrome.

- **Transforming the Burden of Hyperphagia in Bardet-Biedl Syndrome: 6-Month Real-World Outcomes from the RESTORE Stud**y
  Sunday, June 14, 2026, 12:00 – 1:30 p.m. CT

- **Real-Word Weight and Healthcare Utilization Outcomes with Setmelanotide in US Patients with Bardet-Biedl Syndrome**

Saturday, June 13, 2026, 12:15 – 1:45 p.m. CT

The presentations from ENDO 2026 will be available following the conference at: https://hcp.rhythmtx.com/publications-presentations/

In addition, Rhythm will host its inaugural MOMENTUM meeting on June 12 ahead of ENDO, convening leading U.S. experts and healthcare professionals to discuss advances in understanding and managing 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 rare neuroendocrine diseases. Rhythm's lead asset, IMCIVREE ® (setmelanotide), an MC4R agonist designed to treat hyperphagia and severe obesity, is approved by the U.S. Food and Drug Administration (FDA) to reduce excess body weight and maintain weight reduction long term in adult and pediatric patients aged 4 years and older with acquired hypothalamic obesity, adult and pediatric patients 2 years of age and older with syndromic or monogenic obesity due to Bardet-Biedl syndrome (BBS) or genetically confirmed pro-opiomelanocortin (POMC), including proprotein convertase subtilisin/kexin type 1 (PCSK1), deficiency or leptin receptor (LEPR) deficiency. The European Commission (EC) has authorized setmelanotide for the treatment of obesity and control of hunger in patients 4 years of age and above with acquired hypothalamic obesity; and both the EC and the UK's Medicines & Healthcare Products Regulatory Agency (MHRA) have authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 2 years of age and above. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare diseases, as well as investigational MC4R agonists bivamelagon and RM-718, and a preclinical suite of small molecules for the treatment of congenital hyperinsulinism. Rhythm's headquarters is in Boston, MA.

Setmelanotide Indication
In the United States, setmelanotide is indicated to reduce excess body weight and maintain weight reduction long term in adults and pediatric patients aged 4 years and older with acquired hypothalamic obesity, in adult and pediatric patients aged 2 years and older with syndromic or monogenic obesity due to Bardet-Biedl syndrome (BBS) or Pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency confirmed by genetic testing demonstrating variants in POMC, PCSK1, or LEPR genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS).

In the European Union and the United Kingdom, setmelanotide is indicated for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 2 years of age and above. In the European Union and the United Kingdom, setmelanotide should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

Limitations of Use

Setmelanotide is not indicated for the treatment of patients with the following conditions as setmelanotide 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 acquired HO, BBS, or POMC, PCSK1 or LEPR deficiency, including obesity associated with other genetic syndromes and general (polygenic) obesity.

Important Safety Information

CONTRAINDICATIONS

Prior serious hypersensitivity to setmelanotide or any of the excipients in IMCIVREE. Serious hypersensitivity reactions (e.g., anaphylaxis) have been reported.

WARNINGS AND PRECAUTIONS

Disturbance in Sexual Arousal: Spontaneous penile erections and increased frequency of penile erections in males have occurred. Inform patients that these events may occur and instruct patients who have an erection lasting longer than 4 hours to seek emergency medical attention.

Depression and Suicidal Ideation: Depression and suicidal ideation have occurred. Monitor patients for new onset or worsening depression or suicidal thoughts or behaviors. Consider discontinuing IMCIVREE if patients experience suicidal thoughts or behaviors, or clinically significant or persistent depression symptoms occur.

Hypersensitivity Reactions: Serious hypersensitivity reactions (e.g., anaphylaxis) have been reported. If suspected, advise patients to promptly seek medical attention and discontinue IMCIVREE.

Skin Hyperpigmentation, Darkening of Pre-existing Nevi, and Development of New Melanocytic Nevi: Generalized or focal increases in skin pigmentation occurred in the majority of IMCIVREE-treated patients. IMCIVREE may also cause development of new melanocytic nevi or darkening of pre-existing nevi. Perform a full body skin examination prior to initiation and periodically during treatment to monitor pre-existing and new pigmented lesions.

Acute Adrenal Insufficiency with Acquired HO: Patients with acquired HO and secondary adrenal insufficiency reported serious adverse reactions related to acute adrenal insufficiency in 5% of IMCIVREE-treated patients and no placebo-treated patients. In patients with secondary adrenal insufficiency, monitor for clinical signs of acute adrenal insufficiency.

Sodium Imbalance in Patients with Acquired HO and Central Diabetes Insipidus: Patients with acquired HO and concomitant central diabetes insipidus (DI)/arginine vasopressin (AVP) deficiency reported hyponatremia in 6% of IMCIVREE-treated patients and 2% of placebo-treated patients and hypernatremia in 5% of IMCIVREE-treated patients and 4% of placebo-treated patients. Monitor serum sodium levels with changes in fluid intake and hydration status. Adjust the doses of concomitant therapies for DI/AVP deficiency as needed.

ADVERSE REACTIONS

Most common adverse reactions (incidence ≥20% in at least 1 indication) included skin hyperpigmentation, injection site reactions, nausea, headache, diarrhea, abdominal pain, vomiting, depression, and spontaneous penile erection.

USE IN SPECIFIC POPULATIONS

Treatment with IMCIVREE is not recommended when breastfeeding. Discontinue IMCIVREE when pregnancy is recognized unless the benefits of therapy outweigh the potential risks to the fetus.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337 or FDA at 1-800-FDA-1088 or http://www.fda.gov/medwatch. See section 4.8 of the Summary of Product Characteristics for information on reporting suspected adverse reactions in Europe.

Please see the full Prescribing Information for additional Important Safety Information.

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 safety, efficacy, potential benefits of, and clinical design or progress, potential regulatory submissions, approvals and timing thereof for any of our products or product candidates at any dosage or in any indication; the presentation of clinical data and results from our trials, including the ongoing Phase 2 trial of setmelanotide in patients with PWS, clinical and real-world efficacy and safety data related to the use of setmelanotide and bivamelagon in patients with acquired hypothalamic obesity BBS and our participation in upcoming events and presentations, including at ENDO and the content, date and timing of any of the foregoing. 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, 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, 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 successfully commercialize setmelanotide, our liquidity and expenses, our ability to retain our key employees and consultants, and to attract, retain and motivate qualified personnel, and general economic conditions, and other important factors, including those discussed under the caption “Risk Factors” in Rhythm’s Quarterly Report on Form 10-Q for the three months ended March 31, 2026, 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.

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

Kate Walsh
Director, Corporate Communications
Rhythm Pharmaceuticals, Inc.
(857) 264-4280
kwalsh@rhythmtx.com

_____

[1] The abstract reflects data available at the time of submission and includes results from 5 of 18 patients with 6 months of follow-up. Updated data will be presented at the meeting.