Rhythm Pharmaceuticals Announces Health Canada Approval of IMCIVREE® (setmelanotide) for Weight Management in Adult and Pediatric Patients 4 years of Age and Older with Acquired Hypothalamic Obesity

September 22, 2026

-- First targeted therapy approved in Canada for patients four years and older living with acquired hypothalamic obesity (HO) --

BOSTON, Sept. 22, 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 Health Canada has approved IMCIVREE® (setmelanotide) for the additional indication of weight management in adult and pediatric patients 4 years of age and older with acquired hypothalamic obesity (HO). IMCIVREE was first approved by Health Canada in 2023 for weight management in adult and pediatric patients 6 years of age and older with obesity due to Bardet-Biedl syndrome (BBS), or genetically confirmed biallelic pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency due to variants interpreted as pathogenic, likely pathogenic, or of uncertain significance. Please consult the IMCIVREE product monograph available at www.rhythmtx.ca.

"Acquired hypothalamic obesity is a rare, complex MC4R pathway disease that develops following injury to the hypothalamus, resulting in accelerated and sustained weight gain, hyperphagia and significant impacts on quality of life," said Jennifer Lee, Executive Vice President, Head of North America for Rhythm. "The approval of this additional indication for IMCIVREE provides healthcare professionals with an important new treatment option for eligible patients in Canada. We are grateful to the Canadian patient and healthcare community and look forward to working to make IMCIVREE available to patients living with acquired hypothalamic obesity."

IMCIVREE, a melanocortin-4 receptor (MC4R) agonist, is the first targeted therapy approved in Canada to treat acquired HO. The Health Canada approval is based on the positive pivotal Phase 3 TRANSCEND trial of setmelanotide in 142 patients with acquired HO. The global study met its primary endpoint, demonstrating a statistically significant 18.8% placebo-adjusted reduction in body mass index (BMI). Results from the Phase 3 TRANSCEND trial were recently published in The New England Journal of Medicine.

"Patients living with acquired hypothalamic obesity face significant medical and quality-of-life challenges, and treatment options have been extremely limited," said Jill Hamilton, M.D., Paediatric Endocrinologist at The Hospital for Sick Children. “This approval provides a treatment option that addresses the underlying MC4R pathway dysfunction associated with acquired hypothalamic obesity.”

Following this approval, Rhythm will continue with supporting access to IMCIVREE for patients with acquired HO across Canada.

Disclosure footnote: Dr. Jill Hamilton has received institutional support for industry sponsored studies, including setmelanotide trials from Rhythm Pharmaceuticals.

About Acquired Hypothalamic Obesity

Acquired HO is a rare disease characterized by accelerated and sustained weight gain caused by an injury to the hypothalamus. Hypothalamic injury may lead to decreased alpha-melanocyte-stimulating hormone (α-MSH) production and impairment of MC4R pathway signaling. The MC4R pathway is responsible for regulating energy balance and body weight. Acquired HO most frequently follows the growth or treatment of craniopharyngioma, astrocytoma, or other hypothalamic-pituitary tumors. Additional causes of injury may include traumatic brain injury, stroke, infection, or inflammation. Due to impairment of the MC4R pathway, patients experience accelerated and sustained weight gain, often accompanied by hyperphagia and/or decreased energy expenditure. Acquired hypothalamic obesity can occur as early as six months following hypothalamic injury. Acquired HO is a rare disease with limited epidemiologic data specific to Canada.

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. Both the European Commission (EC) and the UK’s Medicines and Healthcare Products

Regulatory Agency (MHRA) have authorized setmelanotide for the treatment of obesity and control of hunger in patients 4 years of age and above with acquired hypothalamic obesity due to hypothalamic injury or impairment; and 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.

Reporting side effects
You can report any suspected side effects associated with the use of health products to Health Canada by:

- Visiting the Web page on Adverse Reaction Reporting (canada.ca/drug-device-reporting) for information on how to report online, by mail or by fax; or
- Calling toll-free at 1-866-234-2345.

NOTE: Contact your healthcare professional if you need information about how to manage your side effects. The Canada Vigilance Program does not provide medical advice.

Please see the Product Monograph at https://rhythmtx.ca/wp-content/uploads/2023/05/IMCIVREE-Product-Monograph-EN.pdf for complete 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 of any of our products or product candidates at any dosage or in any indication, including, setmelanotide, bivamelagon, and RM-718; the use of setmelanotide in patients with acquired HO and the success of our commercial launch; our expectations surrounding potential regulatory submissions, progress, or approvals and timing thereof for any of our product candidates; the commercial growth of IMCIVREE; the estimated market size and addressable population for our drug products, including setmelanotide for the treatment of acquired HO; and the 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 the 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 June 30, 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