Rhythm Pharmaceuticals Receives Positive CADTH Reimbursement Recommendation for IMCIVREE® (setmelanotide)

November 2, 2023

-- CADTH recommends reimbursement for weight management in adult and pediatric patients six years of age and older with obesity due to Bardet-Biedl syndrome (BBS) --

-- Recommendation based on demonstrated clinical benefit in Phase 3 trial --

BOSTON, Nov. 02, 2023 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a global 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 that the Canadian Agency for Drugs and Technologies in Health (CADTH) has recommended that IMCIVREE® (setmelanotide) be reimbursed by CADTH-participating public drug plans, with conditions, for weight management in adult and pediatric patients six years of age and older with obesity due to Bardet-Biedl syndrome (BBS).

“This positive CADTH recommendation will be welcome news for the patients and families living with hyperphagia, which is a pathological and insatiable hunger, and the severe obesity that is often associated with BBS,” said Prof. Andrea M. Haqq, M.D., Department of Pediatrics, Faculty of Medicine & Dentistry, University of Alberta. “This important recommendation further recognizes the unmet needs of people with BBS who otherwise have no targeted precision treatment for weight management.”

CADTH’s recommendation is based on results from Rhythm’s Phase 3 trial that demonstrated treatment with setmelanotide resulted in meaningful clinical benefit in patients 6 years of age and older with obesity due to BBS. Data showed that treatment with setmelanotide for 52 weeks resulted in a clinically meaningful reduction in weight-related parameters, such as total body weight and body mass index (BMI), and the results were supported by the exploratory comparative 14-week placebo-controlled outcomes. Consistent with prior clinical experience, setmelanotide was generally well tolerated. Treatment-emergent adverse events (TEAEs) included mild injection site reactions and nausea.

BBS is a rare genetic disease with an estimated prevalence of 1 in 100,000 to 160,000 in the populations of North America and Europe with an estimated 300-400 people living with the disease in Canada. People living with BBS may experience insatiable hunger, also known as hyperphagia, and severe obesity beginning early in life. BBS may also be associated with cognitive impairment, polydactyly, renal dysfunction, hypogonadism, and visual impairment.

IMCIVREE® (setmelanotide) is the first and only therapy approved in Canada for weight management in adult and pediatric patients 6 years of age and older with obesity due to BBS. IMCIVREE® (setmelanotide) was given a Priority Review by Health Canada.

“This recommendation represents a significant milestone for Canadian patients living with obesity due to BBS. We appreciate CADTH’s recommendation and their recognition of the rare genetic nature of BBS, the unmet needs of these patients and that IMCIVREE is the only available therapy that targets the underlying disease mechanism of BBS,” said Carol Stiff, General Manager of Rhythm Pharmaceuticals Canada Inc.

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 lead asset, IMCIVREE (setmelanotide) is approved by the U.S. Food and Drug Administration (FDA), and authorized by the European Commission (EC) 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.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337. 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 (https://www.canada.ca/en/health-canada/services/drugs-health-products/medeffect-canada.html) for information on how to report online, by mail or by fax; or calling toll-free at 1-866-234-2345.

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 potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, the potential benefits of and our expectations surrounding the CADTH reimbursement recommendation, and our business strategy and plans, including regarding commercialization of IMCIVREE in Canada and other international regions. 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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Source: Rhythm Pharmaceuticals, Inc.