Rhythm Pharmaceuticals Provides Updates on Leadership Transition and Business Operations

March 30, 2020

Hunter Smith, Chief Financial Officer, appointed Interim Chief Executive Officer following completion of NDA submission to FDA

Precautionary measures in place to mitigate impact of COVID-19 on clinical programs

BOSTON, March 30, 2020 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM), a late-stage biopharmaceutical company aimed at developing and commercializing therapies for the treatment of rare genetic disorders of obesity, today announced that Hunter Smith, the Company's Chief Financial Officer, has been named to the additional role of Interim Chief Executive Officer, effective immediately. Mr. Smith succeeds Keith Gottesdiener, M.D. whose planned departure was announced by the Company in January 2020. Dr. Gottesdiener stepped down from his roles as CEO, President and member of the Board of Directors following the Company's completion of its New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA).

"On behalf of the Board, I am pleased that Hunter and the Rhythm management team will continue their excellent stewardship of the Company and dedication to transforming the care of people living with rare genetic disorders of obesity while we continue our search for a permanent CEO," said David Meeker, M.D., Chairman of the Board of Directors. "With Murray Stewart, Chief Medical Officer, leading our clinical development and regulatory strategy and Nithya Desikan, Chief Commercial Officer, and her team making tremendous strides in community building, we believe that Rhythm is well positioned to change the paradigm for rare genetic obesity."

As previously disclosed, the Company's Board of Directors has formed a search committee and retained an executive search firm to assist in identifying Dr. Gottesdiener's permanent successor. The Board has made significant progress in the search process and is considering a number of highly qualified candidates.

"I look forward to working closely with the Board, Murray and Nithya, our colleagues on the management team, and the entire Rhythm team to advance setmelanotide," Mr. Smith said. "We are committed to working with patients and the community to build greater awareness and understanding of rare genetic disorders of obesity so we can maximize our impact and transform the care of people living with these conditions."

COVID-19 and Business Continuity

To help protect the health and safety of the patients, caregivers and healthcare professionals involved in its ongoing clinical trials of setmelanotide, as well as its employees, in response to the novel coronavirus (COVID-19) pandemic, Rhythm has implemented a number of precautionary clinical and operational measures to protect patient well-being and ensure consistent and appropriate clinical trial conduct.

With many clinical trial sites already closing down in response to COVID-19-related country- and state-level guidelines and more closures expected, Rhythm and study investigators and staff remain focused on the safety, treatment and monitoring of patients currently enrolled in these trials. Rhythm has introduced measures to ensure patients in ongoing clinical trials continue to be monitored as scheduled and receive their study drug.

Mr. Smith continued, "We are acutely aware of the unprecedented crisis unfolding across the globe as a result of the COVID-19 pandemic. Over the past several weeks, we have put measures in place to protect the health and safety of patients and staff participating in our clinical trials, as well as our employees and their families, and to seek to ensure that our ongoing studies can continue with minimal interruption."

POMC Deficiency Obesity and LEPR Deficiency Obesity

Today, Rhythm announced that it has completed its rolling submission of an NDA to the FDA for setmelanotide for the treatment of pro-opiomelanocortin (POMC) deficiency obesity and leptin receptor (LEPR) deficiency obesity. The FDA typically has a 60-day filing review period to determine whether the NDA is complete and acceptable for filing. Rhythm has requested priority review for the application which, if granted, could provide a target FDA review period of six months from the application filing date. Rhythm continues to anticipate that it will submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the second quarter of 2020. At this time, Rhythm is continuing its regular interactions with the FDA and EMA and based on current information, the Company does not anticipate COVID-19 to materially affect its timelines.

Ongoing Clinical Trials of Setmelanotide

Rhythm continues to expect to meet disclosed timelines for reporting data from its pivotal Phase 3 trial in Bardet-Biedl Syndrome (BBS) and Alström Syndrome and Phase 2 Basket Study. The Company anticipates announcing topline data in the fourth quarter of 2020 or early in the first quarter of 2021 from the Phase 3 BBS and Alström syndrome trial, which completed enrollment in December 2019. Rhythm also anticipates announcing additional data in high-impact heterozygous (HET) obesity and additional data from one or more of its other ongoing indications in 2020, based on current enrollment levels in the Phase 2 Basket Study.

Rhythm currently believes there will be no disruption of clinical supply of setmelanotide. The Company's contract manufacturers have indicated that they have appropriate plans and procedures in place to ensure uninterrupted future supply of clinical and commercial-grade setmelanotide, subject to potential limitations on their operations due to COVID-19.

Corporate Operations

Consistent with guidelines from the Centers for Disease Control (CDC) and the Commonwealth of Massachusetts, Rhythm has also implemented measures to help keep the Company's employees, families, and local communities healthy and safe. All employees are working remotely and all

business travel has been restricted.

About Rhythm

Rhythm is a late-stage biopharmaceutical company focused on the development and commercialization of therapies for the treatment of rare genetic disorders of obesity. In August 2019, the company announced positive topline results from pivotal Phase 3 clinical trials of setmelanotide, its MC4R agonist, in people living with POMC deficiency obesity and LEPR deficiency obesity and, in March 2020, completed its first rolling NDA submission to the FDA. Rhythm is also evaluating setmelanotide for reduction in hunger and body weight in a pivotal Phase 3 trial in people living with Bardet-Biedl and Alström syndromes, with topline data from this trial expected in the fourth quarter of 2020 or early in the first quarter of 2021. Rhythm is leveraging the Rhythm Engine -- comprised of its Phase 2 basket study, TEMPO Registry, GO-ID genotyping study and Uncovering Rare Obesity program -- to improve the understanding, diagnosis and potentially the treatment of rare genetic disorders of obesity. 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.

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, including the anticipated timing for enrollment and design of clinical trials, the timing for approval of an NDA and for submission of an MAA, the impact of COVID-19 on the Company's clinical trials, regulatory submissions and supply of setmelanotide, the ongoing CEO search, and expectations surrounding future guidance, the release of results of clinical trials and updates on patient enrollment. Statements using words such as "expect", "anticipate", "believe", "may", "will" and similar terms are also forward-looking statements. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the impact of COVID-19 and other global economic factors, 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, and other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2019 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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