

BioMarin InvestorRoom

BioMarin Extends Gene Therapy Leadership with DiNAQOR in a Preclinical Collaboration and License Agreement to Develop Gene Therapies for Rare Genetic Cardiomyopathies

SAN RAFAEL, Calif., May 3, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that the company has entered into a preclinical collaboration and license agreement with DiNAQOR AG (DiNAQOR), a gene therapy platform company, to develop novel gene therapies to treat rare genetic cardiomyopathies. DiNAQOR will receive an undisclosed upfront payment and is eligible to receive development, regulatory and commercial milestones on product sales in addition to tiered royalties on worldwide sales. The company did not disclose financial terms. BioMarin management reiterated its 2020 GAAP net income guidance of \$20 to \$80 million, inclusive of this collaboration.

The license initially covers DiNAQOR's lead program, DiNA-001 for MYBPC3 hypertrophic cardiomyopathy (HCM). Additionally, the companies will collaborate on several of DiNAQOR's other pipeline programs, and BioMarin has the option to extend the license to include these additional programs on similar terms. Reflecting the long-term commitment to the collaboration, BioMarin is simultaneously investing in DiNAQOR.

"With this agreement, BioMarin is continuing to apply its gene therapy know-how and manufacturing expertise in new areas like cardiology," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer at BioMarin. "This collaboration extends our global leadership position in gene therapy and boosts our potential to transform the lives of patients worldwide with rare genetic cardiomyopathies."

"We are thrilled to collaborate with the researchers at DiNAQOR to conduct this pioneering work on the development of gene therapies for inherited cardiomyopathies," said Lon Cardon, Chief Scientific Strategy Officer and Senior Vice President at BioMarin. "We believe there is tremendous potential in combining our experience in gene therapy research and development with DiNAQOR's in-depth knowledge of genetic heart diseases."

DiNAQOR was founded and is led by several leading pharmaceutical and biotechnology executives and academics with deep cardiology and gene therapy expertise. The company's holistic approach to gene therapy is focused on gene therapies for the heart that deliver a medical solution that can safely deliver gene therapies to the heart muscle, ensure transduction of the cardiac cells, and limit the exposure of the therapy to other organs.

"BioMarin is a global leader in rare disease research, development and commercialization, and their commitment to DiNA-001 is a powerful validation of DiNAQOR's gene therapy platform," said Dr. Johannes Holzmeister, Co-Founder, Chairman and CEO at DiNAQOR. "We believe our platform has many potential applications and this milestone agreement will enable us to invest in expanding our genetic medicine pipeline."

"Momentum for gene therapies continues to build, and BioMarin has demonstrated tremendous scientific, clinical, and manufacturing leadership and expertise in the space," said Thomas Voit, M.D., Ph.D., Co-Founder and Chief Scientific Officer at DiNAQOR and Director of the Biomedical Research Centre at the Great Ormond Street Hospital and the UCL Institute of Child Health, University College London. "We are looking forward to combining our strengths to expand the promise of gene therapy treatments by targeting the heart muscle to treat rare genetic cardiomyopathies."

About HCM and MYBPC3

Hypertrophic cardiomyopathy (HCM) is one of the most common genetic heart diseases, with about 500,000 patients diagnosed with HCM worldwide. Up to 60% of HCM cases have a genetic origin, and it is

estimated that 40% of those have mutations in MYBPC3, the gene that encodes cardiac myosin-binding protein C (MyBP-C).

HCM affects the heart muscle, causing the muscle to enlarge. HCM patients have an increased risk of developing heart failure and life-threatening arrhythmias. There are no approved pharmacological treatment options available that address the underlying disease biology of HCM and invasive surgery or heart transplantation may be the only options available for patients with advanced disease.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for serious and life-threatening rare genetic diseases. The Company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomin.com. Information on BioMarin's website is not incorporated by reference into this press release.

About DiNAQOR

Founded in 2019, DiNAQOR AG is a global gene therapy platform company focused on advancing novel solutions for patients suffering from heart disease. The company's lead preclinical program, DiNA-001 is focused on the treatment of MYBPC3-linked cardiomyopathy. DiNAQOR is headquartered in Pfäffikon, Switzerland, with additional presence in London, England and Boston, Massachusetts (US). For more information visit www.dinaqor.com.

Forward Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: BioMarin's expectations regarding the announced collaboration, the prospects for the lead and follow on pipeline products and its 2020 GAAP profitability. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: results and timing of current and planned preclinical studies and clinical trials; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning the programs; the ability to manufacture the product candidates, BioMarin's revenue for 2020, especially with the possible impact of COVID-19, and those other risks detailed from time to time under the caption "Risk Factors" and elsewhere in BioMarin's Securities and Exchange Commission (SEC) filings, including BioMarin's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, and future filings and reports by BioMarin. BioMarin undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

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