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FOR IMMEDIATE RELEASE

U.S. Food and Drug Administration Approves BioMarin's ROCTAVIAN™ (valoctocogene roxaparvovec-rvox), the First and Only Gene Therapy for Adults with Severe Hemophilia A

ROCTAVIAN is a One-Time, Single-Dose Treatment Available for Adults with Severe Hemophilia A to Control Bleeds

ROCTAVIAN's Approval was Based on Durability, Efficacy and Safety Results from the Largest and Longest Phase 3 Study for a Gene Therapy for Hemophilia

Majority of Patients Continued to Respond to ROCTAVIAN Treatment over 3 Years

Conference Call and Webcast to be Held at 4:30 p.m. Eastern Time on Thursday, June 29, 2023

SAN RAFAEL, Calif., June 29, 2023 – BioMarin Pharmaceutical Inc. (Nasdaq: BMRN), a global biotechnology company dedicated to transforming lives through genetic discovery, today announced that the United States Food and Drug Administration (FDA) approved ROCTAVIAN™ (valoctocogene roxaparvovec-rvox) gene therapy for the treatment of adults with severe hemophilia A (congenital factor VIII (FVIII) deficiency with FVIII activity < 1 IU/dL) without antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test.

The one-time, single-dose infusion is the first approved gene therapy for severe hemophilia A in the U.S. ROCTAVIAN was first approved by the European Medicines Agency in August 2022.

"Adults with severe hemophilia A face a lifelong burden, with frequent infusions and a high risk of health complications, including uncontrolled bleeding and irreversible joint damage," said Dr. Steven Pipe, professor of pediatrics and pathology at the University of Michigan and an investigator in the Phase 3 study. "The approval of ROCTAVIAN, as the first gene therapy for severe hemophilia A, has the potential to transform the way we treat adults based on years of bleed control following a single, one-time infusion."

Hemophilia A is a lifelong, genetic condition caused by a mutation in the gene responsible for producing a protein called FVIII, which is necessary for blood clotting. When severely deficient in amount, the condition puts people with hemophilia A at risk for painful and potentially life-threatening bleeds, which can occur spontaneously. With the current standard of care, individuals undergo lifelong preventative therapy, receiving infusions or injections at burdensome routine intervals to maintain enough clotting factor in the bloodstream to prevent bleeds. ROCTAVIAN is designed to replace the function of the mutated gene, allowing people with severe hemophilia A to produce their own FVIII and thereby limit bleeding episodes.

“Today’s approval of ROCTAVIAN builds on BioMarin’s proven track record of advancing treatments that target the underlying cause of life-threatening genetic conditions, which has produced eight best- or first-in-class commercial therapies,” said Jean-Jacques Bienaimé, chairman and chief executive officer of BioMarin. “We are proud to now offer adults with severe hemophilia A, a one-time, single-dose treatment option. We are especially grateful to the bleeding disorders community for its support of this program, and to all the patients and healthcare providers who participated in our clinical trials.”

ROCTAVIAN is manufactured at the company’s facility in Novato, California. The BioMarin-owned site is one of the largest gene therapy manufacturing facilities of its kind and will allow the company to meet commercial demand throughout its product lifecycle.

Largest Phase 3 Gene Therapy Study in Hemophilia to Report More Than Three Years of Data

The FDA approval is based on data from the global Phase 3 GENEr8-1 study, the largest Phase 3 trial of any gene therapy in hemophilia. Of the 134 patients who received ROCTAVIAN in the study, 112 patients had baseline annualized bleeding rate (ABR) data prospectively collected during a period of at least six months on FVIII prophylaxis prior to receiving ROCTAVIAN. The remaining 22 patients had baseline ABR collected retrospectively. All patients were followed for at least 3 years.

As reported in the FDA-approved labeling for ROCTAVIAN, the 112 patients in whom 6-month baseline ABR was collected prospectively experienced a mean ABR reduction of 52% after receiving ROCTAVIAN (2.6 bleeds/year) through end of follow-up (median of three years) compared to their baseline ABR while receiving routine FVIII prophylaxis (5.4 bleeds/year). This result was based on an FDA analysis that imputed an ABR of 35 in 13 patients for the periods when these patients were on prophylaxis. These patients also reported a substantial reduction in the rate of spontaneous bleeds and joint bleeds following treatment with ROCTAVIAN (observed mean ABR of 0.5 bleeds/year for spontaneous bleeds and 0.6 bleeds/year for joint bleeds) compared to their baseline rate while receiving routine FVIII prophylaxis (observed mean ABR of 2.3 bleeds/year for spontaneous bleeds and 3.1 bleeds/year for joint bleeds).

The majority of study participants continued to respond to treatment through year three and beyond, without supplemental use of regular prophylaxis.

BioMarin will continue to monitor the long-term effects of treatment with an extension study that will follow all clinical trial participants for up to 15 years, as well as post-approval studies to follow those dosed in a real-world setting for 15 years or more.

Data Presented at the International Society on Thrombosis and Haemostasis (ISTH) 2023 Congress

Additionally, results from the three-year analysis of the Phase 3 GENEr8-1 study that were presented on Sunday at ISTH showed that study participants had an 82.9% reduction in treated bleeds overall compared with baseline. The study also found ROCTAVIAN led to a 96.8% reduction in FVIII usage overall compared with baseline.

Ensuring Access: Hemophilia Treatment Centers (HTCs) and Outcomes-Based Warranty Program

BioMarin will begin educating physicians and patients about ROCTAVIAN immediately to ensure the hemophilia community is aware of this new treatment option.

As part of the development of ROCTAVIAN, BioMarin has worked with private and public payers in the U.S. in parallel to enable access, with the goal of ensuring that every eligible adult interested in ROCTAVIAN is able to receive treatment.

A key component of the company's approach to access is the outcomes-based warranty, which will be offered to all U.S. insurers. The warranty will reimburse government and commercial payers up to 100% of wholesale acquisition cost in the event that a person does not respond to ROCTAVIAN. If an individual treated with ROCTAVIAN loses response at any time in the first four years after dosing, BioMarin will reimburse payers on a prorated basis for the cost of treatment.

Most people with hemophilia receive care at HTCs. The company is working closely with the leading U.S. HTCs to ensure that the centers are prepared to administer ROCTAVIAN following today's approval.

"Our teams have been working for many months to ensure that the people who are eligible for ROCTAVIAN have access to this first-in-class medicine," said Jeff Ajer, executive vice president and chief commercial officer of BioMarin. "We appreciate the close partnership with health insurers, hemophilia treatment centers and the hemophilia community to ensure the greatest access for people with severe hemophilia A."

It is estimated that there are approximately 6,500 adults living with severe hemophilia A in the U.S. BioMarin expects approximately 2,500 of those adults to be eligible to receive ROCTAVIAN with this initial approval.

Investor Conference Call and Webcast to be Held at 4:30 p.m. Eastern Time on Thursday, June 29, 2023

BioMarin will host a conference call and webcast to discuss the approval at 4:30 p.m., Thursday, June 29, 2023. This event can be accessed in the investor section of the BioMarin website at https://investors.biomarin.com/events-presentations.

U.S./Canada Dial-in Number: 888-330-3073	Replay Dial-in Number: 800-770-2030
International Dial-in Number: 646-960-0683	Replay International Dial-in Number: 647-362-9199
Conference ID: 1816377	Conference ID: 1816377

Robust Ongoing Clinical Program

BioMarin has multiple clinical studies underway in its comprehensive gene therapy program for the treatment of severe hemophilia A. In addition to the global Phase 1/2 and Phase 3 GENEr8-

1 studies, the company is also conducting a single-arm, open-label study to evaluate the efficacy and safety of ROCTAVIAN at a dose of 6e13 vg/kg with prophylactic corticosteroids in people with severe hemophilia A (Study 270-303). There is also an ongoing study with the 6e13 vg/kg dose of ROCTAVIAN in people with severe hemophilia A with pre-existing AAV5 antibodies (Study 270-203) and a study with the 6e13 vg/kg dose of ROCTAVIAN in people with severe hemophilia A with active or prior FVIII inhibitors (Study 270-205).

Safety Summary

Safety results for 134 patients have been reported through three years, demonstrating that ROCTAVIAN was well-tolerated.

The Prescribing Information includes Warnings and Precautions for infusion-related reactions, hepatotoxicity, thromboembolic events and theoretical risk of hepatocellular carcinoma.

Patients with detectable pre-existing antibodies to AAV5, active infections, history of thrombosis, immunosuppressive disorders and liver dysfunction were excluded. All patients had a median follow-up of 162 weeks (range: 66 to 255 weeks). The most common adverse reactions (≥ 5%) to ROCTAVIAN were nausea, fatigue, headache, infusion-related reactions, vomiting, and abdominal pain. ROCTAVIAN is contraindicated for patients with active infections, either acute or uncontrolled chronic, known significant hepatic fibrosis (stage 3 or 4), or cirrhosis and a known hypersensitivity to mannitol.

The most common laboratory abnormalities were alanine transaminase (ALT), aspartate transaminase (AST), lactate dehydrogenase (LDH), creatine kinase (CPK), factor VIII activity levels, gamma-glutamyl transferase (GGT) and bilirubin > upper levels of normal (ULN). The majority of patients in the clinical trial required corticosteroids for ALT elevation (median duration of corticosteroid use was 35 weeks). See additional safety information in the Prescribing Information and Important Safety Information below.

Patient Support for Accessing ROCTAVIAN

To reach a BioMarin RareConnections® case manager, please call 1-833-ROCTAVIAN (1-833-762-8284), or email roctaviansupport@biomarin-rareconnections.com. For more information about ROCTAVIAN, please visit BioMarin.com or contact BioMarin Medical Information at medinfo@bmrn.com.

About Hemophilia A

Hemophilia A, also called FVIII deficiency or classic hemophilia, is an X-linked genetic disorder caused by missing or defective FVIII, a clotting protein. Although it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous mutation, a new mutation that was not inherited. Approximately 1 in 10,000 people have hemophilia A.

People living with hemophilia A lack sufficient functioning FVIII protein to help their blood clot and are at risk for painful and/or potentially life-threatening bleeds from even modest injuries. Additionally, people with the most severe form of hemophilia A (FVIII levels <1%) often experience painful, spontaneous bleeds into their muscles or joints. Individuals with the most severe form of hemophilia A make up approximately 50% of the hemophilia A population. People with hemophilia A with moderate (FVIII levels 1-5%) or mild (FVIII levels 5-40%) disease show a much-reduced

propensity to bleed. Individuals with severe hemophilia A are treated with a prophylactic regimen of intravenous FVIII infusions administered 2-3 times per week (100-150 infusions per year) or a bispecific monoclonal antibody that mimics the activity of FVIII administered 1-4 times per month (12-48 injections or shots per year). Despite these regimens, many people continue to experience breakthrough bleeds, resulting in progressive and debilitating joint damage, which can have a major impact on their quality of life.

About ROCTAVIAN

ROCTAVIAN is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity < 1 IU/dL) without antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test. The one-time infusion works by delivering a functional gene that is designed to enable the body to produce FVIII on its own, reducing the need for ongoing prophylaxis.

The European Commission (EC) granted conditional marketing authorization to ROCTAVIAN on August 24, 2022.

Important Safety Information

Contraindications: Patients with active infections, either acute (such as acute respiratory infections or acute hepatitis) or uncontrolled chronic (such as chronic active hepatitis B). Patients with known significant hepatic fibrosis (stage 3 or 4 on the Batts-Ludwig scale or equivalent), or cirrhosis, and patients with known hypersensitivity to mannitol.

Infusion-related reactions including hypersensitivity reactions and anaphylaxis, have occurred. Monitor during and for at least 3 hours after ROCTAVIAN administration. Administer ROCTAVIAN in a setting where personnel and equipment are immediately available to treat infusion-related reactions. Discontinue infusion for anaphylaxis.

Hepatotoxicity: The safety and effectiveness of ROCTAVIAN in patients with hepatic impairment has not been established. Perform liver health assessments prior to administration. The majority of patients treated with ROCTAVIAN experienced ALT elevations and required corticosteroids for ALT elevation. Assess patient's ability to receive corticosteroids and/or other immunosuppressive therapy that may be required for an extended period. Live vaccines should not be administered to patients while on immunosuppressive therapy.

Monitor ALT weekly for at least 26 weeks and as clinically indicated, during corticosteroid therapy and institute corticosteroid treatment in response to ALT elevations as required. Continue to monitor ALT until it returns to baseline. Monitor factor VIII activity levels since ALT elevation may be accompanied by a decrease in factor VIII activity. One case of autoimmune hepatitis was reported during third year follow-up in a patient with history of hepatitis C and steatohepatitis.

It is recommended that patients abstain from consuming alcohol for at least 1 year after administration and thereafter limit alcohol use. Concomitant medications may cause hepatotoxicity, decrease factor VIII activity, or change plasma corticosteroid levels which may impact liver enzyme elevation and/or factor VIII activity or decrease the efficacy of the corticosteroid regimen or increase their side effects. Closely monitor concomitant medication use

including herbal products and nutritional supplements and consider alternative medications in case of potential drug interactions.

Thromboembolic events: Factor VIII activity above ULN has been reported following ROCTAVIAN infusion. Thromboembolic events may occur in the setting of elevated factor VIII activity above ULN. Evaluate patients for risk of thrombosis including general cardiovascular risk factors before and after administration of ROCTAVIAN. Advise patients on their individual risk of thrombosis in relation to their factor VIII activity levels above ULN and consider prophylactic anticoagulation. Advise patients to seek immediate medical attention for signs or symptoms indicative of a thrombotic event.

Factor VIII inhibitors and Monitoring for inhibitors. The safety and effectiveness of ROCTAVIAN in patients with prior or active factor VIII inhibitors have not been established. Patients with active factor VIII inhibitors should not take ROCTAVIAN. Following administration, monitor patients for factor VIII inhibitors (neutralizing antibodies to factor VIII). Test for factor VIII inhibitors especially if bleeding is not controlled, or plasma factor VIII activity levels decrease.

Monitor Factor VIII using the same schedule for ALT monitoring. It may take several weeks after ROCTAVIAN infusion before ROCTAVIAN-derived factor VIII activity rises to a level sufficient for prevention of spontaneous bleeding episodes. Exogenous factor VIII or other hemostatic products may also be required in case of surgery, invasive procedures, trauma, or bleeds. Consider more frequent monitoring in patients with factor VIII activity levels ≤ 5 IU/dL and evidence of bleeding, taking into account the stability of factor VIII levels since the previous measurement.

Factor VIII activity produced by ROCTAVIAN in human plasma is higher if measured with one-stage clotting assays compared to chromogenic substrate assays. When switching from hemostatic products prior to ROCTAVIAN treatment, physicians should refer to the relevant prescribing information to avoid the potential for factor VIII activity assay interference during the transition period.

Malignancy: The integration of liver-targeting AAV vector DNA into the genome may carry the theoretical risk of hepatocellular carcinoma development. ROCTAVIAN can also insert into the DNA of other human body cells. Monitor patients with risk factors for hepatocellular carcinoma (e.g., hepatitis B or C, non-alcoholic fatty liver disease, chronic alcohol consumption, non-alcoholic steatohepatitis, advanced age) with regular liver ultrasound (e.g., annually) and alpha-fetoprotein testing for 5 years following ROCTAVIAN administration. In the event that any malignancy occurs after treatment with ROCTAVIAN, contact BioMarin Pharmaceutical Inc. at 1-866-906-6100.

Most Common Adverse Reactions: Most common adverse reactions (incidence ≥ 5%) were nausea, fatigue, headache, infusion-related reactions, vomiting, and abdominal pain. Most common laboratory abnormalities (incidence ≥ 10%) were ALT, AST, LDH, CPK, factor VIII activity levels, GGT and bilirubin > ULN. Patients also experienced adverse reactions from corticosteroid use.

Isotretinoin, Efavirenz, and HIV Positive Patients. Isotretinoin is not recommended in patients who are benefiting from ROCTAVIAN. Efavirenz is not recommended in patients treated with

ROCTAVIAN. Clinical studies of ROCTAVIAN did not include sufficient numbers of patients with HIV to determine whether the efficacy and safety differs compared to patients without HIV infection.

Females and Males of Reproductive Potential. ROCTAVIAN is not intended for administration in women. There are no data on the use of ROCTAVIAN in pregnant women or regarding lactation. For 6 months after administration of ROCTAVIAN men of reproductive potential and their female partners must prevent or postpone pregnancy using an effective form of contraception, and men must not donate semen.

You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to BioMarin at 1-866-906-6100.

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

About BioMarin

Founded in 1997, BioMarin is a global biotechnology company dedicated to transforming lives through genetic discovery. The company develops and commercializes targeted therapies that address the root cause of the genetic conditions. BioMarin's unparalleled research and development capabilities have resulted in eight transformational commercial therapies for patients with rare genetic disorders. The company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. For additional information, please visit BioMarin.com.

Forward-Looking Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the potential benefits and impact of ROCTAVIAN for treating adults with severe hemophilia A, including (i) the potential for ROCTAVIAN to transform treatment for such patients by providing years of bleed control following a single infusion of ROCTAVIAN and (ii) allowing such patients to produce their own FVIII and thereby limit bleeding episodes; the clinical development of ROCTAVIAN, including (i) BioMarin's continued monitoring of the long-term effects of treatment with an extension study as well as post-approval studies and the duration of such monitoring and (ii) clinical studies regarding potential product expansion opportunities for ROCTAVIAN; the commercialization of ROCTAVIAN, including (i) BioMarin's ability to manufacture sufficient ROCTAVIAN to meet commercial demand throughout the product's lifecycle, (ii) BioMarin's plans to immediately educate physicians and patients about ROCTAVIAN, (iii) BioMarin's goal to ensure that every eligible adult interested in ROCTAVIAN is able to receive treatment, (iv) BioMarin's plans to offer outcomes-based warranties for ROCTAVIAN to all U.S. insurers, and (v) BioMarin's goal that HTCs are prepared to administer ROCTAVIAN; the expectation that ROCTAVIAN will offer significant savings to society; the expectation that BioMarin's outcomes-based warranties will further enhance the value of ROCTAVIAN to payers; and BioMarin's expectations regarding the number of adult patients with severe hemophilia in the U.S. who will be eligible to receive ROCTAVIAN with the initial FDA approval. 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: BioMarin's success in the commercialization of ROCTAVIAN, including achieving adequate market share and reimbursement levels; whether ROCTAVIAN will have the impacts and benefits as anticipated; the results and timing of current and planned preclinical studies and clinical trials of ROCTAVIAN and the release of data from those trials, including continued monitoring of the participants in the clinical trials and post-approval studies; BioMarin's ability to successfully manufacture ROCTAVIAN for the clinical trials and commercially; the content and timing of decisions by the FDA, EU health authorities and other regulatory authorities regarding ROCTAVIAN; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the quarter year ended March 31, 2023, as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc., and ROCTAVIAN™ is a trademark of BioMarin Pharmaceutical Inc.

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