BioMarin's Gene Therapy for Adults with Severe Hemophilia A, ROCTAVIAN™ (valoctocogene roxaparvovec), Assessed to Provide Substantial Cost Savings Per Patient in a Preliminary Independent Report

Institute for Clinical and Economic Review (ICER) Base-Case Model Report Results in $4 Million Savings Versus Emicizumab Prophylaxis Per Patient Over a Lifetime

Biologics License Application (BLA) Resubmission to FDA on Track for End of September 2022

SAN RAFAEL, Calif., Sept. 15, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that the Institute for Clinical and Economic Review (ICER) released a Draft Evidence Report updating the previous Hemophilia A assessment of the comparative clinical effectiveness and value of ROCTAVIAN ™ (valoctocogene roxaparvovec).

In the Draft Evidence Report, ICER finds Roctavian is a dominant treatment at an assumed place holder price of $2.5 million, providing substantial cost savings and projected gains in quality adjusted life years. These findings were robust to numerous sensitivity analyses and scenario analyses. Marking an important milestone in ICER's eight-month process of assessing this treatment, the Draft Evidence Report is now open for public comment, with a final report expected near the end of the year.

In the Draft Evidence Report, ICER calculated the lifetime cost of managing hemophilia A among clinically eligible patients using one-time administration with Roctavian versus emicizumab prophylaxis. Total costs in the model include treatment, treatment-related adverse events, treatment for bleeding episodes, arthropathy, surgery, and non-drug costs. ICER assumed annual cost of emicizumab to be $640K per year and one-time Roctavian price to be $2.5M. ICER modelled the effect of Roctavian to last 12 years (before patients were assumed to switch back to prophylaxis) for the entire cohort post infusion. ICER arrived at greater than $4M cost saving per patient over a lifetime with projected improvement in quality of life. Importantly, ICER incorporated an outcomes-based warranty agreement in its base-case economic model, an innovative approach that BioMarin plans to offer that will allow effective risk sharing for the period of four years and have it ready to implement with payers at launch.

While Roctavian is approved in the European Union, it is still an investigational therapy in

the U.S. and therefore does not have a price.

"BioMarin is pleased that ICER recognizes the potentially transformative impact of Roctavian as possibly the first gene therapy treatment for severe hemophilia A, and potential to not only deliver profound patient benefit, but also potential long-term healthcare savings," said Jeff Ajer, Executive Vice President, Chief Commercial Officer at BioMarin.

BioMarin remains committed to bringing Roctavian to eligible patients with severe hemophilia A in the United States and is targeting a Biologics License Application (BLA) resubmission for Roctavian by the end of September 2022. Typically, BLA resubmissions are followed by a six-month review procedure. However, the Company anticipates three additional months of review may be necessary based on the number of data read-outs that will emerge during the procedure.

BioMarin expects first commercial sales in Europe in the fourth quarter of 2022.

ICER is an independent non-profit research organization that evaluates the evidence on the effectiveness and economic value of prescription drugs and other medical devices. The Draft Evidence Report will next be evaluated by one of ICER's three independent evidence appraisal committees before the organization issues its final Evidence Report expected near the end of the year.

Safety Summary

Overall, single 6e13 vg/kg dose of Roctavian has been well tolerated with no delayed-onset treatment related adverse events. The most common adverse events (AE) associated with Roctavian occurred early and included transient infusion associated reactions and mild to moderate rise in liver enzymes with no long-lasting clinical sequelae. Alanine aminotransferase (ALT) elevation (113 participants, 80%), a laboratory test of liver function, remained the most common adverse drug reaction. Other adverse reactions included aspartate aminotransferase (AST) elevation (95 participants, 67%), nausea (52 participants, 37%), headache (50 participants, 35%), and fatigue (42 participants, 30%). No participants developed inhibitors to Factor VIII, thromboembolic events or malignancy associated with Roctavian.

About Hemophilia A

People living with hemophilia A lack sufficient functioning Factor VIII 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 (Factor VIII 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 percent of the hemophilia A population. People with hemophilia A with moderate (Factor VIII 1-5%) or mild (Factor VIII 5-40%) disease show a much-reduced propensity to bleed. Individuals with severe hemophilia A are treated with a prophylactic regimen of intravenous Factor VIII infusions administered 2-3 times per week (100-150 infusions per year) or a bispecific monoclonal antibody that mimics the activity of Factor VIII administered 1-4 times per month (12-48 infusions 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.

Hemophilia A, also called Factor VIII deficiency or classic hemophilia, is an X-linked genetic disorder caused by missing or defective Factor VIII, 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.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening genetic diseases and medical conditions. The Company selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's portfolio consists of eight commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases. For additional information, please visit www.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:; BioMarin's plans to re-submit a BLA for Roctavian to the FDA by the end of September 2022; the duration of the FDA's review procedure of BioMarin's BLA resubmission for Roctavian; BioMarin plans to offer an outcomes based warranty agreement that will allow effective risk sharing for the period of four years and have it ready to implement with payers at launch; BioMarin expectations of its first commercial sales of Roctavian in

Europe to occur in the fourth quarter of 2022; and expectations that the Draft Evidence Report is expected to be issued near the end of the year. 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: that the findings included in the Draft Evidence Report may not be ICER's final conclusions, the results and timing of current and planned preclinical studies and clinical trials of Roctavian; additional data from the continuation of the clinical trials of Roctavian, any potential adverse events observed in the continuing monitoring of the participants in the clinical trials; the content and timing of decisions by the FDA, the EC and other regulatory authorities,; the content and timing of decisions by local and central ethics committees regarding the clinical trials; our ability to successfully manufacture Roctavian for the clinical trials and commercially;; and those 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 ended June 30, 2022 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.

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