BioMarin Sells Priority Review Voucher for $110 Million

SAN RAFAEL, Calif., Feb. 9, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that it has entered into a definitive agreement with an undisclosed purchaser to sell the Rare Pediatric Disease Priority Review Voucher (PRV) it obtained in November 2021 for a lump sum payment of $110,000,000. The Company received the voucher under a Food and Drug Administration (FDA) program intended to encourage the development of treatments for rare pediatric diseases. BioMarin was awarded the voucher when it received approval of VOXZOGO™ (vosoritide) for Injection, indicated to increase linear growth in pediatric patients with achondroplasia five years of age and older with open epiphyses (growth plates). The transaction remains subject to customary closing conditions, including anti-trust review.

"We are pleased to announce the sale of the PRV and plan to direct the proceeds from this voucher sale towards additional investment in an already robust pipeline of investigational therapies for people with genetic diseases," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are proud to be able to participate in this program and that this voucher will be accelerating the availability of a therapy for patients."

This is the third Priority Review Voucher that BioMarin has received. The FDA also awarded PRVs to the company when Brineura® (cerliponase alfa) and Vimizim® (elosulfase alfa) were approved. Company Management expects that the net gain on the sale of the PRV, after income taxes, will be incremental to the Company's previously communicated expectation to earn positive GAAP net income in 2022.

About the Rare Pediatric Disease Priority Review Voucher Program

The program is intended to encourage development of new drug and biological products for the prevention and treatment of certain rare pediatric diseases. A PRV is issued to the sponsor of a rare pediatric disease product application and entitles the holder to priority review of a single New Drug Application or Biologics License Application. The sponsor receives the voucher upon approval of the rare pediatric disease product application. PRVs may be sold or transferred, and there is no limit on the number of times a PRV can be transferred.

About FDA Standard Review and Priority Review Designations

Prior to approval, each drug marketed in the United States must go through a detailed FDA review process. In 1992, under the Prescription Drug User Act (PDUFA), FDA agreed to specific goals for improving the drug review time and created a two-tiered system of review times - Standard Review and Priority Review. A Priority Review designation is given to drugs that provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition. The FDA goal for reviewing a drug with Priority Review status is six months from the time the application is filed by the FDA, compared to 10 months under standard review.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates.

For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

Forward-Looking Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the Company's plans to use the PRV sale proceeds towards additional investment in BioMarin's development programs and expectations that the net gain on the sale of the PRV, after income taxes, will be incremental to the Company's previously communicated expectation to earn positive GAAP net income in 2022. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. Additional important factors to be considered in connection with forward-looking statements are 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 September 30, 2021, 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.

Contact:	
Investors:	*Media:*
Traci McCarty	*Debra Charlesworth*
BioMarin Pharmaceutical Inc.	*BioMarin Pharmaceutical Inc.*
(415) 455-7558	(*415) 455-7451*

SOURCE BioMarin Pharmaceutical Inc.
