

BioMarin Sells Second Priority Review Voucher for \$125 Million

SAN RAFAEL, Calif., Nov. 27, 2017 /[PRNewswire](#)/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that it has entered into a definitive agreement to sell the Rare Pediatric Disease Priority Review Voucher (PRV) it obtained in April of this year for a lump sum payment of \$125,000,000. The Company received the voucher under a U.S. 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 Brineura®, a new biological product for patients with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency, a form of Batten disease. The transaction remains subject to customary closing conditions, including anti-trust review.

"We are proud to be able to participate in a program that encourages investment in the development of therapies for children with rare diseases. BioMarin will direct the proceeds from this voucher sale towards additional investment in an already robust pipeline of products to treat rare and ultra-rare diseases," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are very pleased that this voucher will be accelerating the availability of a therapy for patients."



This is the second PRV that BioMarin has sold. In July 2014, BioMarin received \$67.5 million from Regeneron Ireland, an indirect, wholly-owned subsidiary of Regeneron Pharmaceuticals, Inc., in exchange for a voucher awarded when it received approval of Vimizim® for patients with the rare disease, Mucopolysaccharidosis type IVA, also known as Morquio A syndrome.

Pediatric Disease Priority Review Voucher Sale Impact to BioMarin Financial Guidance

The sale of the PRV will be recorded as a \$125 million gain on sale of an intangible asset and will also be associated with approximately \$25 million of income tax expense. As a result of the sale, the Company will update its GAAP Net Loss guidance by the \$100 million net after tax gain, and for full-year 2017, the GAAP Net Loss guidance will be reduced to between \$(10) million and \$(30) million. The sale of the PRV is a special item that will be excluded from Non-GAAP Income and consequently the Non-GAAP Income guidance for the full-year 2017 is unchanged at \$60 to \$80 million.

About the Pediatric Disease Priority Review Voucher Program

The PRV is issued to the sponsor of a rare pediatric disease product application that 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 priority review voucher can be transferred.

About Food and Drug Administration 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. Standard Review can be accomplished in a ten-month time frame from the time the application is filed by the FDA, which typically occurs approximately 60-days following submission of the application. A

Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA goal for reviewing a drug with Priority Review status is six months from the time the application is filed by the FDA.

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 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.

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