

BioMarin's Gene Therapy Manufacturing Facility Recognized with Industry Award

One of the largest gene therapy facilities received Facility of the Year Award for Project Execution from the International Society for Pharmaceutical Engineering

SAN RAFAEL, Calif., March 21, 2018 [/PRNewswire/](#) -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the International Society for Pharmaceutical Engineering (ISPE) selected the company's gene therapy manufacturing facility as the **2018 Facility of the Year Category Winner for Project Execution**. The recognition highlighted the company's successful construction of the facility in Novato, CA, which took less than a year to transform basic infrastructure into one of the first gene manufacturing facilities of its kind in the world. Winners were announced on Tuesday, March 20 during the 2018 Europe Annual Conference in Rome, Italy.

With the company's hemophilia A investigational treatment valoctocogene roxaparvec (formerly BMN 270) in global Phase 3 development and a need to produce material for the clinical studies and potentially for commercial demand, BioMarin converted an office and warehouse building into an 18,000 square foot gene therapy facility. The company proceeded with its project with the goal of releasing the first filled vials of Phase 3 clinical drug product in less than 15 months from project initiation. Beating the goal, the buildout and commissioning for the facility was accomplished in less than a year.



"The buildout of our gene therapy manufacturing facility was a logistical challenge that required leveraging our experience gained during licensure of our Galli and Shanbally biological facilities. This plant provides us with scheduling flexibility allowing for rapid product development and is capable of providing sufficient capacity to meet clinical and projected commercial requirements," said Robert Baffi, Ph.D., Executive Vice President of Technical Operations at BioMarin. "In addition, it creates the opportunity for our engineers and scientists to establish a valuable knowledge base required for manufacturing complex gene therapy products. This project demonstrated how the industry's best people, equipped with the right technology and energized by the right motivation, can make a seemingly impossible project possible."

BioMarin's engineering team, along with its partners Project Architect and Engineer, CRB; and General Contractor, Novo Construction, attributed its success to implementation of a number of best practices and accelerating elements. This included speed of site selection, rapid assembly of critical project teams and engineering resources, co-location of all project teams on site, phased design methodologies, construction and buildout concepts and concurrent commercial process scale-up. As a result of this new gene therapy manufacturing capability, the company is well positioned, with the necessary resources and capacity to undertake the aggressive development program for valoctocogene roxaparvec in severe hemophilia A.

More about the Awards Program

ISPE's Facility of the Year program is the premier global awards program, recognizing innovation and creativity in the pharmaceutical and biotechnology manufacturing industries. Projects selected for recognition set the standard by demonstrating excellence in facility design, construction and operations.

About ISPE

The International Society for Pharmaceutical Engineering (ISPE) is the world's largest not-for-profit association serving its members through leading scientific, technical, and regulatory advancement across the entire pharmaceutical lifecycle. The 18,000 members of ISPE are building solutions in the development and

manufacture of safe, effective pharmaceutical and biologic medicines, and medical delivery devices in more than 90 countries around the world. Founded in 1980, ISPE has its worldwide headquarters in Bethesda, Maryland USA, and an operations and training center in Tampa, Florida USA. Visit [ISPE](#) for more information.

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.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the Company's development program for valoctocogene roxaparvovec in severe hemophilia A, the possible approval and commercialization of BioMarin's product candidate valoctocogene roxaparvovec and the Company's gene therapy manufacturing facility and its ability to support clinical and anticipated commercial demand of valoctocogene roxaparvovec, if approved. 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 clinical trials of valoctocogene roxaparvovec; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; our ability to successfully manufacture the product candidate for the clinical trials; 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 Annual Report on Form 10-K for the year ended December 31, 2017 and future filings and reports by BioMarin. 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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