

BIOMARIN'S MANUFACTURING APPROACH

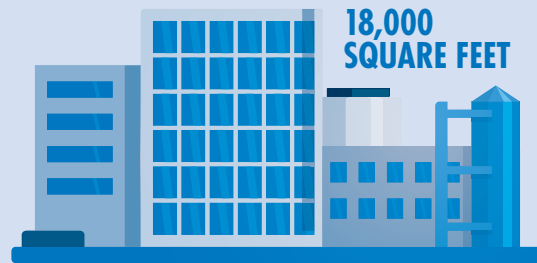
Headquartered in San Rafael, CA, BioMarin is a world leader in providing therapies to patients with rare and ultra-rare conditions and has seven commercialized products that are the first and only therapies for the diseases they treat. With a diverse and exciting pipeline of clinical and pre-clinical candidates, including gene therapy, the company remains focused on bringing treatments to people with serious and life-threatening rare disorders.

SPOTLIGHT ON GENE THERAPY

BioMarin's valoctocogene roxaparvovec is a gene therapy undergoing clinical trials in hemophilia A. It is designed to correct the underlying genetic defect of the disease.

The goal of gene therapy being researched is to provide patients with the genetic code that will allow the body to produce Factor VIII using its own native cellular machinery, mitigating the need for factor replacement. BioMarin has a robust clinical study program for valoctocogene roxaparvovec in severe hemophilia A.

BIOMARIN HAS CONSTRUCTED ONE OF THE FIRST GENE THERAPY MANUFACTURING FACILITIES OF ITS KIND






MEETING CLINICAL & COMMERCIAL DEMAND

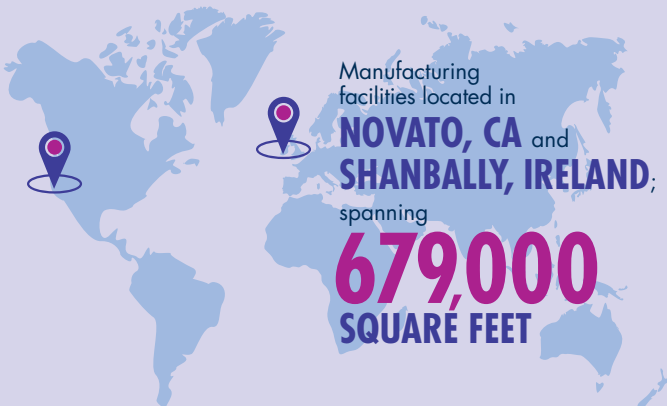
Ability to produce

4,000 DOSES / YEAR OF VALOCTOGENE ROXAPARVOVEC

OWNED MANUFACTURING APPROACH ALLOWS:

-  **CONTROL OF SCHEDULING**
-  **QUALITY**
-  **RAPID PRODUCTION**

MANUFACTURING INVESTMENT & PRODUCTION



MORE THAN \$545 MILLION

invested in manufacturing & R&D facilities since 2013

OVER 20 YEARS OF EXPERIENCE

>15 MILLION



UNITS OF PRODUCT

manufactured in 2017