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 valoctocogene roxaparvovec.

Owned Manufacturing Approach Allows:
- Control of Scheduling
- Quality
- Rapid Production

Manufacturing Investment & Production
Manufacturing facilities located in Novato, CA and Shanbally, Ireland, spanning 679,000 square feet.

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.