Single-Arm Study to Evaluate the Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients (BMN 270-301)

Clinical Trials.Gov: NCT03370913
www.clinicaltrials.gov/ct2/show/NCT03370913

Basic Information

- **Sponsor:** BioMarin Pharmaceutical
- **Multi-center**
- **Multi-national**
- **x 134** Actual enrollment
- **Vector:** AAV5
- **Data available:** 156–208 weeks

Study Population

- **Congenital hemophilia A:**
  - Residual FVIII activity ≤ 1 IU/dL
  - On FVIII prophylaxis for at least 12 months prior to study entry
- **Age:** ≥ 18 years
- **No history of FVIII inhibitors**
- **No detectable pre-existing antibodies to AAV5 capsid**

Intervention

- **Single intravenous infusion of AAV5-huFVIII-SQ (BMN 270)**
- **High dose:** $6 \times 10^{13}$ vg/kg

Outcomes

1. **Primary Outcome Measure**
   - [Time Frame: 52 weeks]
   - Change in median FVIII activity levels

2. **Secondary Outcome Measures**
   - [Time Frame: 52 weeks]
   - Change in the annualized utilization (IU/kg) of exogenous FVIII replacement therapy
   - Change in the annualized number of bleeding episodes requiring exogenous FVIII replacement treatment

Published Results


Timeline

- **December 19, 2017**
  - Study Starts
- **December 2020**
  - Estimated Primary Completion
- **September 2023**
  - Estimated Study Completion