ISTH GENE THERAPY in Hemophilia

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 x 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

Rose S, et al. *Blood*. 2020;136(22):2524-2534

Timeline

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