GENE THERAPY in Hemophilia

Single-Arm Study to Evaluate the Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at a Dose of 4E13 vg/kg (BMN 270-302)

Clinical Trials.Gov: NCT03392974

www.clinicaltrials.gov/ct2/show/NCT03392974

Basic Information

Sponsor: BioMarin Pharmaceutical

Multi-center

Multi-national

Active trial

x 40 Estimated 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)

Low dose: 4 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

In preparation

Timeline

March 14, 2018	December 1, 2022	March 1, 2024
Study Starts	Estimated Primary Completion	Estimated Study Completion