HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients

Clinical Trials.Gov: NCT03569891
www.clinicaltrials.gov/ct2/show/NCT03569891

Basic Information

- **Sponsor:** UniQure Biopharma B.V.
- **Vector:** AAV5
- **Pl:** Steven Pipe, University of Michigan
- **Multi-center**
- **Multi-national**
- **Data available:** 52 weeks
- **x 56** Estimated enrollment

Study Population

- **Congenital hemophilia B:**
  - Severe or moderately severe
  - Currently on FIX prophylaxis (>150 days of exposure)
- **Age:** ≥ 18 years
- **No history of FIX inhibitors**

Intervention

- **Single intravenous infusion of AAV5-hFIXco-Padua (AMT-061)**
- **1 dose:** $2 \times 10^{13}$ gc/kg ($n = 3$)

Outcomes

1. **Primary Outcome Measure**
   - FIX activity levels
2. **Secondary Outcome Measures**
   - Annualized bleed rate
   - Use of FIX replacement therapy
   - Adverse events

Published Results


Timeline

- **June 27, 2018** Study Starts
- **September 2020** Estimated Primary Completion
- **August 2024** Estimated Study Completion

GENE THERAPY in Hemophilia