

# GENE THERAPY in Hemophilia



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

ClinicalTrials.gov: [NCT03569891](https://clinicaltrials.gov/ct2/show/NCT03569891)  
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### Basic Information

**Sponsor:** UniQure Biopharma B.V.

**PI:** Steven Pipe, University of Michigan

**x 56** Estimated enrollment

**Vector:** AAV5

**Multi-center** **Multi-national**

**Data available:** 52 weeks

### 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

**Phase 2B:** Von Drygalski A, et al. *Blood Adv.* 2019;3:3241-3247.

### Timeline

**June 27, 2018**  
Study Starts

**September 2020**  
Estimated Primary Completion

**August 2024**  
Estimated Study Completion