A Study to Evaluate the Efficacy and Safety of Factor IX Gene Therapy With PF-06838435 in Adult Males With Moderately Severe to Severe Hemophilia B

Clinical Trials.Gov: NCT03861273
www.clinicaltrials.gov/ct2/show/NCT03861273

Basic Information

**Sponsor:** Pfizer, PF-06838435 (previously developed by Spark Therapeutics, SPK-9001)

**Vector:** rAAV-Spark100-hFIX-Padua (fidanacogene elaparvovec)

**Data:** From phase 1/2 for up to 4 months

**Estimated enrollment:** 55

Study Population

**Congenital hemophilia B:**
- Moderately severe to severe disease (residual FIX activity ≤ 2%)
- Completed 6 months of FIX prophylaxis therapy during lead-in study

**Age:** 18–65 years

**No history of FIX inhibitors**

**No AAV-Spark100 neutralizing antibodies**

Intervention

**Single intravenous infusion of rAAV-Spark100-hFIX-Padua (PF-06838435)**

**Dosing:** NA (phase 1/2 doses were 5 x 10¹¹, 1 x 10¹², and 2 x 10¹² vg/kg)

Outcomes

**Primary Outcome Measures**
- Annualized bleeding rate (ABR) [Time Frame: 12 months]
- Vector derived FIX:C level [Time Frame: Week 12 to 12 months post PF 06838435 infusion]

**Secondary Outcome Measures**
- Annualized infusion rate (AIR) of exogenous Factor IX activity [Time Frame: First 12 months post study drug infusion]
- Annualized Factor IX activity consumption [Time Frame: 12 months post study drug infusion]
  - + 16 others

Published Results

Not available

Timeline

- **July 29, 2019** Study Starts
- **April 23, 2022** Estimated Primary Completion
- **April 21, 2027** Estimated Study Completion