GENE THERAPY in Hemophilia

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)

x 55 Estimated enrollment

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

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

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 10^{11}, 1 x 10^{12}, and 2 x 10^{12} vg/kg)

Outcomes

1 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]

2 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	April 23, 2022	April 21, 2027
Study Starts	Estimated Primary Completion	Estimated Study Completion