ISTH GENE THERAPY in Hemophilia

Study to Evaluate the Efficacy and Safety of PF-07055480 in Moderately Severe to Severe Hemophilia A Adults

Clinical Trials.Gov: NCT04370054

www.clinicaltrials.gov/ct2/show/NCT04370054

Basic Information

Sponsor: Pfizer, PF-07055480
(previously developed by Sangamo, SB-525)

x 63 Estimated enrollment

Vector: AAV2/6

Data: From phase 1/2 for up to 14 months

Study Population

Congenital hemophilia A:

- Moderately severe to severe disease (residual FVII activity ≤ 1%)
- Completed 6 month FVIII prophylaxis lead in study
- ≥ 150 exposure days to FVIII

Age: 18–64 years

No history of FVIII inhibitors

No AAV6 neutralizing antibodies

Intervention

Single intravenous infusion of rAAV2/6 hFVIII (PF-07055480)

Dosing: 3 x 10^{13} vg/kg based on phase 1/2 studies

Outcomes

1 Primary Outcome Measure

- Annualized bleeding rate (ABR) [Time Frame: 12 months]

2 Secondary Outcome Measures

- Steady state FVIII activity levels [Time Frame: 12 months]
- Annualized infusion rate (AIR) of exogenous Factor VIII activity [Time Frame: 12 months]
- Annualized FVIII consumption [Time Frame: 12 months]
- Plus others

Published Results

Not available

Timeline

August 18, 2020	August 12, 2022	October 29, 2026
Study Starts	Estimated Primary Completion	Estimated Study Completion