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)

- **Vector:** AAV2/6

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

- **Estimated enrollment:** 63

**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:** $3 \times 10^{13}$ 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

**Timeline**

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

**Published Results**

Not available