A Study to Investigate the Safety and Effectiveness of a Coagulation Factor IX Gene Insertion Therapy (REGV131-LNP1265) in Pediatric, Adolescent and Adult Participants With Hemophilia B

NCT06379789

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Regeneron Pharmaceuticals
Enrollment	130 participants

Key Eligibility Criteria

Inclusion (3)

- Confirmed diagnosis of severe or moderately severe hemophilia B with medical history of FIX functional activity (d2% or \<0.02 IU/mL) or documented genotype known to produce severe hemophilia B
- Currently taking FIX prophylaxis and previous experience with FIX therapy, as defined in the protocol
- Participation in the lead-in period of this interventional study OR a separate lead-in study (R0000-HEMB-2187 \[NCT05568459\]) for at least 6 months for ABR data while taking FIX prophylaxis, as defined in the protocol

Exclusion (10)

- History of FIX inhibitor (clinical or laboratory-based assessment) on 2 or more occasions
- Bethesda inhibitor titer greater than the upper limit of normal (ULN) at screening
- Detectable pre-existing antibodies to the adeno-associated virus serotype 8 (AAV8) capsid; as measured by enzyme-linked immunosorbent assay (ELISA) at prescreening (or final lead-in visit, if applicable).
- Any significant underlying liver disease such as: cholestatic liver disease, liver cirrhosis, portal hypertension, splenomegaly, hepatic encephalopathy
- Evidence of advanced liver fibrosis, as defined in the protocol

... and 5 more (see full listing online)

Locations (40 total)

Orthopaedic Hemophilia Treatment Center, Los Angeles, California, United States
David Geffen School of Medicine at UCLA, Los Angeles, California, United States
Children's Hospital Los Angeles, Los Angeles, California, United States
... and 37 more locations