ForPatients
by Roche

Hemophilia A

A Gene Therapy Study of SPK-8011QQ in Adults With Severe or Moderately Severe Hemophilia A

Trial Status	Trial Runs In	Trial Identifier
Not yet recruiting		NCT07226206 SPK-8011QQ-201 XO46084

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase 2b, Single-Arm, Open-Label, Multicenter Study of the Safety of SPK-8011QQ in Adults With Severe or Moderately Severe Hemophilia A

Trial Summary:

This study will assess the safety and tolerability of SPK-8011QQ in adult males with moderately severe to severe hemophilia A.

Hoffmann-La Roche	Phase 2
Sponsor	Phase

NCT07226206 SPK-8011QQ-201 XO46084
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Male	#18 Years	No

Inclusion Criteria:

- Signed Informed Consent Form (ICF)
- #18 years of age at the time of signing the ICF
- Male sex assigned at birth
- Severe or moderately severe hemophilia A, defined as endogenous FVIII:C activity levels #3%, as documented (historically or during the Screening Period) by a certified laboratory and where the FVIII:C level is measured more than 96 hours after the prior dose of an extended half-life FVIII replacement product or more than 72 hours after the prior dose of a standard half-life FVIII replacement product

- Have documented treatment for a minimum of 6 months prior to screening with either of the following: plasma coagulation factor VIII (FVIII) prophylaxis, defined as receiving a prescribed dose and frequency of FVIII infusions with the intent to treat continuously for 52 weeks per year; or FVIII on demand, with a history of # 5 breakthrough bleeds in the 6 months prior to screening
- No prior history of hypersensitivity or anaphylaxis associated with the administration of any FVIII product
- Have #150 exposure days to a FVIII protein product such as recombinant, plasma-derived, or extended half-life FVIII product
- Negative screening test for inhibitor against FVIII (i.e., <0.6 BU)
- Candidates with prior FVIII inhibitors who are tolerized having completed successful ITI at least 5 years before screening are eligible provided they have had no evidence of inhibitor recurrence (permanent or temporary) within 5 years prior to screening as may be indicated by detection of an inhibitor, FVIII half-life <6 hours, or FVIII recovery <66% since completing ITI
- Confirmed negative anti-Spark200 antibodies as documented through central laboratory testing of a serum sample
- Acceptable hepatobiliary function according to all of the following criteria: ALT, AST, and ALP #2×ULN and INR <1.4 at the time of screening; No evidence of cirrhosis or advanced liver disease on screening liver ultrasound; Otherwise no laboratory or clinical evidence of liver disease or cirrhosis, per the Investigator's judgement
- Adequate renal function, defined as creatinine clearance #30 mL/min/1.73 m2 by Chronic Kidney Disease Epidemiology Collaboration formula; patients on dialysis are not eligible for the study
- Platelet count #50,0000 cells/µL
- Negative HIV test at screening, with the following exception: Individuals with a positive HIV test at screening are eligible provided they are stable on an antiretroviral treatment regimen, have a cluster of differentiation (CD4) count >200/mm3, and undetectable viral load (<50 gc/mL)
- Negative hepatitis B surface antigen (HBsAg) at screening
- Positive hepatitis surface antibody (HBsAb) at screening, or a negative HBsAb at screening accompanied by either of the following: Negative hepatitis B core antibody (HBcAb); Positive HBcAb and negative hepatitis B virus (HBV) DNA test
- Negative hepatitis C virus (HCV) antibody test at screening, or positive HCV antibody test at screening accompanied by negative HCV RNA test
- Otherwise appropriate medical history and physical and laboratory evaluation that are acceptable for inclusion in this clinical trial
- Are able and willing to comply with scheduled visits, treatment plans, laboratory tests, and other study procedures, including the completion of applicable patient-reported outcome questionnaires
- Agreement to adhere to the contraception requirements described in the protocol

Exclusion Criteria:

- Are currently undergoing antiviral therapy for chronic hepatitis B or chronic hepatitis C
- Have an inherited or acquired bleeding disorder other than hemophilia A
- Have known inherited or acquired thrombophilia, have signs of thromboembolic disease in the Investigator's judgement, or are on current treatment for thromboembolic disease. A history of previous catheter-associated thrombosis for which anti-thrombotic treatment is not currently ongoing is not considered an exclusion criterion
- Have had prior treatment with a vector or gene transfer agent. Nucleic acid-based vaccines, such as the vaccine for coronavirus disease 2019 (COVID-19), are not considered gene transfer agents
- Are receiving an investigational drug concurrently or have received an investigational drug within 30 days or 5 half-lives of the last investigational drug administration, whichever is longer
- Have a major surgical procedure planned in the 15-month period following SPK-8011QQ infusion
- Are unable (or unwilling) to receive blood or blood products (or any standard-of-care treatment for a life-threatening condition)

- Have concurrent disease, treatment, or abnormality in clinical laboratory tests that could interfere with the conduct of the study or that would, in the opinion of the Investigator preclude the candidate's safe participation in and completion of the study, or the interpretation of the study results
- History of malignancy within 5 years prior to screening and up to investigational study drug administration (Day 1) with the following exceptions: Participants with curatively treated basal or squamous cell carcinoma of the skin at any time prior to investigational study drug administration (Day 1) are eligible