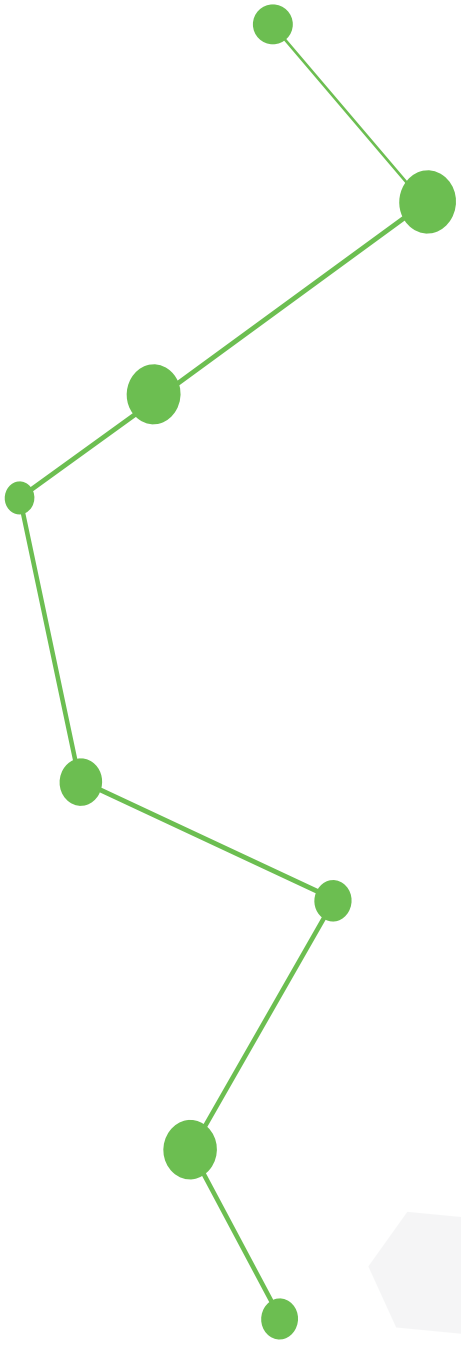
INVESTIGATIONAL GENE THERAPY RESEARCH STUDIES FOR HEMOPHILIA

Spark™ THERAPEUTICS

At Spark Therapeutics, we're striving to challenge genetic disease by **discovering, developing and delivering gene therapies** that address a range of inherited diseases.

Spark Therapeutics is focused solely on developing gene therapies and we have progressed **three investigational gene therapies into clinical trials for hemophilia.**



OUR COMMITMENT TO THE HEMOPHILIA COMMUNITY

We believe gene therapy has the potential to be transformative in the treatment of hemophilia and we understand the importance of developing gene therapies that meet the needs of the hemophilia community. Our priority is the safety and well being of trial participants.

We began our clinical studies of gene therapy in hemophilia B (this program is now in Phase 3 development with Pfizer, Inc.). Our enrolling investigational programs are currently aimed at studying gene therapy in people with hemophilia A.

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OUR ONGOING CLINICAL STUDIES IN HEMOPHILIA A

Spark Therapeutics has two clinical studies of investigational gene therapies for hemophilia A and one observational study (without therapeutic intervention), which is collecting real-time data about people living with hemophilia A.

A Gene Transfer Study of *SPK-8011* for Hemophilia A. This clinical research study is being conducted by Spark Therapeutics, Inc. to determine the safety and efficacy of the factor VIII gene transfer treatment with *SPK-8011* in individuals with hemophilia A. **(Trial identifier NCT03003533)**

Dose-finding Study of *SPK-8016* Gene Therapy in Patients with Hemophilia A to Support Evaluation in Individuals with FVIII Inhibitors. *SPK-8016* is in development for the treatment of patients with inhibitors to FVIII. This Phase 1/2, open-label, non-randomized, dose-finding study is part one of a planned two part study of *SPK-8016*. Part one will evaluate the safety, efficacy and tolerability of *SPK-8016* in adult males with clinically severe hemophilia A and no measurable inhibitor against FVIII. Data obtained from Part 1 will inform the study design and dose selection for Part 2 in patients with FVIII inhibitors. **(Trial identifier NCT03734588)**

Lead-in Study to Collect Prospective Efficacy and Safety Data of Current FVIII Prophylaxis Replacement Therapy in Adult Hemophilia A Participants. The aim of this prospective, observational study is to establish a dataset on the frequency of bleeding events, as well as other characteristics of bleeding events and FVIII infusions, in patients with clinically severe hemophilia A receiving prophylactic FVIII replacement therapy as standard of care. The data collected from this study may assist in providing baseline information for comparison to Spark's investigational hemophilia A gene therapy in future Phase 3 studies. **(Trial identifier NCT03876301)**

WANT TO FIND OUT MORE INFORMATION ABOUT OUR ONGOING CLINICAL TRIALS?

- Speak with your physician
- Visit www.clinicaltrials.gov and search for the Trial Identifier or NCT number

IF YOU HAVE OTHER QUESTIONS FOR SPARK THERAPEUTICS, CONTACT THE SPARK PATIENT ADVOCACY TEAM AT PATIENTS@SPARKTX.COM.