



Spark Therapeutics is excited to continue its commitment to the hemophilia community with the announcement that its **Phase 1/2 trial for the investigation of gene therapy for hemophilia A (factor VIII deficiency) has begun**. This trial will initially be enrolling participants at the Children's Hospital of Philadelphia (CHOP) with additional sites expected to open in the coming months.

Spark Therapeutics is striving to challenge the inevitability of genetic disease by discovering, developing, and delivering gene therapies. Spark Therapeutics has two clinical research programs in hemophilia. *SPK-9001* is an investigational gene therapy product for hemophilia B being developed in collaboration with Pfizer, and *SPK-8011*, an investigational gene therapy product for hemophilia A, is now in a Phase 1/2 trial.

As you may know, Spark Therapeutics and Pfizer recently shared preliminary data from the hemophilia B Phase 1/2 trial at the 2016 American Society of Hematology conference. Spark is encouraged by the initial data from this group of nine participants, and will continue to provide updates as they become available. For a detailed description of these data, please see the companies' press release: <http://ir.sparktx.com/phoenix.zhtml?c=253900&p=irol-newsArticle&ID=2227316>.

Similar to the hemophilia B program, Spark Therapeutics' hemophilia A program's goal is to investigate the safety of providing a one-time intravenous (IV) infusion of investigational *SPK-8011* in an effort to provide adequate levels of factor VIII. For more information about the study, including inclusion and exclusion criteria, please see the study listing on www.clinicaltrials.gov (NCT ID#: NCT03003533). You may also wish to connect directly with CHOP; study coordinator Aline Galvao, MS, RN, can be reached via email at galvaoAM@email.chop.edu or by phone at 267-425-5843.

Should you wish to have more information about Spark Therapeutics and its programs, or have further questions please visit <http://sparktx.com/contact-us/> or email patients@sparktx.com.