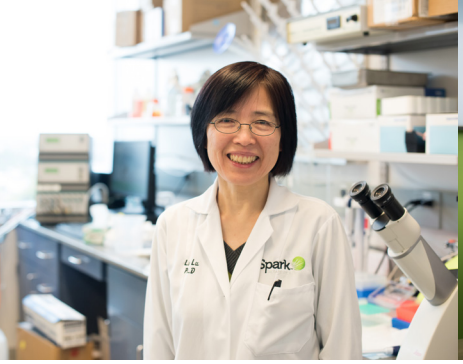




Spark THERAPEUTICS

Company Overview

Spark Therapeutics, a fully integrated, commercial gene therapy company, strives to challenge the inevitability of genetic disease by working to discover, develop and deliver gene therapies that address inherited retinal diseases (IRDs), neurodegenerative diseases, as well as diseases that can be addressed by targeting the liver. We aim to reawaken healthy biologic processes through the potential one-time administration of gene therapies, and spark a transformation for people affected by rare genetic diseases where no, or only palliative, therapies exist.

Our approach to gene therapy is to investigate treatments that go to an inherited disease at its root by augmenting, replacing or suppressing the function of a mutated gene. We engineer investigational gene therapy vectors using a cutting-edge, proprietary adeno-associated viral (AAV) vector platform, developed through vigorous preclinical and clinical testing. Our validated gene therapy platform has delivered human proof-of-concept data in two target tissues and secured breakthrough therapy designations in the retina and liver.

Spark Therapeutics was founded in March 2013 as a result of the technology and know-how developed at Children's Hospital of Philadelphia (CHOP). Members of our founding scientific team have been at the forefront of gene therapy research for more than two decades. They are responsible for numerous development milestones, including the first clinical trials of AAV vectors in skeletal muscle tissue and the liver, the first clinical studies to evaluate AAV administration to the second eye, the first gene therapy trial for a nonlethal disorder that included pediatric participants, and the first approved gene therapy for a genetic disease in the U.S.

The Spark Therapeutics team includes more than 350 industry professionals with deep experience in research and development activities, manufacturing and commercializing complex and novel biotechnology products. We are headquartered in Philadelphia in a 48,000-square foot facility that includes a state-of-the-art cGMP manufacturing facility, the only AAV commercial manufacturing facility for an FDA-approved product in the U.S. With AAV vector GMP manufacturing capabilities in-house, investigational clinical-grade vectors developed and manufactured by our team have been delivered through six routes of administration to participants in more than a dozen clinical trials. These AAV vectors are also used in our approved gene therapy product.

Since our founding, we have secured more than $1 billion in financing to support the growth of our clinical programs and platform. Our company has been named to MIT Technology Review's list of the world's "50 Smartest Companies" in the top 10 for two years in a row (2016, 2017), and to Bloomberg Businessweek's "50 Companies to Watch" in 2018. We have also been recognized as one of the World's Most Innovative Companies by Fast Company magazine.

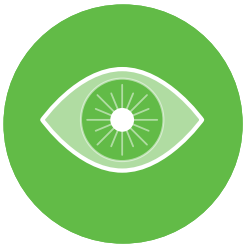
We don't follow footsteps. We create the path.

Our Products

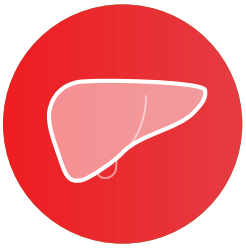
For information about Spark Therapeutics products, visit **www.sparktx.com/products.**

Our Clinical Programs

We have a growing pipeline of nine investigational gene therapies, including three gene therapies currently in clinical trials.



INHERITED RETINAL DISEASES (IRDs)



LIVER-DIRECTED DISEASES

NEURODEGENERATIVE DISEASES

In the U.S., our first such investigational candidate is *SPK-7001* for the potential treatment of choroideremia, a rare IRD that usually manifests in affected males during childhood as night blindness and a reduction of visual field, ultimately leading to complete blindness. An open-label, dose-escalating Phase 1/2 trial is designed to assess the safety and preliminary efficacy of subretinal administration of investigational *SPK-7001* for choroideremia.

We also have initiated the Phase 1/2 trial for *SPK-8011*, an investigational gene therapy for hemophilia A, or factor VIII deficiency. Hemophilia A is a serious and rare inherited hematologic disorder, characterized by mutations in the coagulation factor VIII, or *F8*, gene which lead to deficient blood coagulation and an increased risk of bleeding or hemorrhaging. Spark Therapeutics retains global commercialization rights to its *SPK-8011* program for hemophilia A.

Fidanacogene elaparvovec, previously *SPK-9001*, is an investigational bio-engineered AAV vector utilizing a high-activity factor IX transgene. Hemophilia B is a serious and rare inherited hematologic disorder, characterized by mutations in the coagulation factor IX, or *F9*, gene, which lead to deficient blood coagulation and an increased risk of bleeding or hemorrhaging. Fidanacogene elaparvovec has received both breakthrough therapy and orphan product designations from the U.S. FDA. Spark Therapeutics initiated the ongoing Phase 1/2 clinical trial of fidanacogene elaparvovec that was transitioned to Pfizer in July 2018. At this time, Pfizer announced initiation of a Phase 3 lead-in study. As part of the collaboration, Pfizer assumes sole responsibility for all subsequent pivotal studies, all regulatory activities, manufacturing and potential global commercialization of any products resulting from the hemophilia B gene therapy program.

We are also developing *SPK-GAA*, an investigational gene therapy for the potential treatment of Pompe disease. Pompe disease is an oftentimes fatal lysosomal storage disorder and neuromuscular disease, with systemic, multi-organ manifestations resulting from loss of function mutations in the gene encoding acid alpha-glucosidase (GAA). The initial construct for *SPK-GAA* was in-licensed from Genethon in 2017, and Spark retains global commercialization rights.

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Spark THERAPEUTICS

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