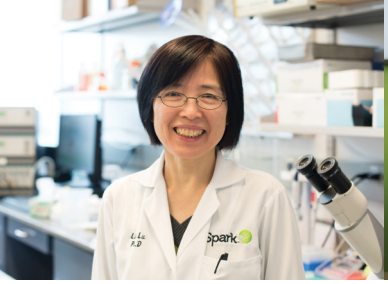


Spark™ THERAPEUTICS

Company Overview

At Spark Therapeutics, a fully integrated, commercial company committed to discovering, developing and delivering gene therapies, we challenge the inevitability of genetic diseases, including blindness, hemophilia, lysosomal storage disorders and neurodegenerative diseases. 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 vision is a world where no life is limited by genetic disease.

Our approach to gene therapy is to investigate treatments that target 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.

We are the first biotechnology company that successfully commercialized a gene therapy for a genetic disease in the U.S., bringing a one-time treatment to market. We have created unique competencies in the discovery, development and delivery of genetic medicines which are unmatched across the value chain, including target selection and AAV vector optimization, commercial and scalable AAV manufacturing, regulatory innovation and precedent-setting approvals and gene therapy market development and access.

Spark Therapeutics was founded in March 2013 as a result of the technology and know-how developed at Children's Hospital of Philadelphia (CHOP). Spark's founders were 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 is comprised of cross-functional professionals with deep experience in research and development activities, manufacturing and commercializing complex and novel biotechnology products. Spark Therapeutics is headquartered in Philadelphia, where our state-of-the-art current good manufacturing practices (cGMP) manufacturing facility, the first AAV commercial manufacturing facility for a U.S. Food and Drug Administration (FDA) approved gene therapy for a genetic disease, is located.

In 2019, Spark received the Prix Galien USA Award for Best Biotechnology Product. Our company was also named to *Science Magazine's* Top Employer list 2019 and 2020, ranking in the top ten. We received recognition from *MIT Technology Review* as a "50 Smartest Companies" and to *Bloomberg Businessweek* as one of their "50 Companies to Watch." We have also been recognized as one of the World's Most Innovative Companies by *Fast Company* magazine, and as one of the "Best Places to Work" for four years in a row (2017-2020) by the *Philadelphia Business Journal*.

In December 2019, Spark Therapeutics was acquired by the Roche Group and maintains its headquarters in Philadelphia.

Our Products

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

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

Our Clinical Programs

We are putting our unique competencies to use to evaluate and select a portfolio of potential gene therapies targeting three target tissues – the retina, liver and CNS – across multiple therapeutic areas by moving investigational assets into the clinic to optimize our success at developing and delivering medicines to patients with unmet medical needs.

INHERITED RETINAL DISEASES (IRDs)

LIVER-DIRECTED DISEASES

NEURODEGENERATIVE DISEASES

We continue to advance our portfolio of investigational gene therapies for hemophilia A, or factor VIII deficiency. We have initiated the Phase 3 study for *SPK-8011* for the hemophilia A non-inhibitor patient population. The Phase 1/2 dose-finding study for *SPK-8016* for the hemophilia A inhibitor patient population will initially evaluate safety, efficacy and tolerability in non-inhibitor patients with clinically severe hemophilia A. 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* and *SPK-8016* programs for hemophilia A.

Fidanacogene elaparvovec, previously *SPK-9001*, is an investigational bio-engineered AAV vector utilizing a high-activity *F9* transgene for hemophilia B, or factor IX deficiency. 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 is conducting the Phase 3 trial. 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-3006*, an investigational gene therapy for the potential treatment of Pompe disease. Spark has initiated the RESOLUTE℠ trial, a Phase 1/2, dose-escalation gene transfer study being conducted to evaluate the safety, tolerability, and efficacy of a single intravenous infusion of *SPK-3006* in adults with clinically moderate late-onset Pompe disease (LOPD) currently receiving enzyme replacement therapy. 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 alphaglucosidase (GAA). The initial construct for *SPK-3006* was in-licensed from Genethon in 2017, and Spark retains global commercialization rights.

SPK-1001 is an investigational central nervous system (CNS)-directed AAV gene therapy that has demonstrated preclinical proof-of-concept in a naturally occurring model of tripeptidyl peptidase 1 (TPP1) enzyme deficiency, or CLN2 (a form of Batten disease). Batten disease is a fatal neurological disorder that begins in early childhood and is characterized by mutations of the *TPP1* gene. We have obtained orphan drug designation from the U.S. FDA for *SPK-1001* for the treatment of CLN2 disease caused by TPP1 deficiency and Spark retains global rights.

Spark Therapeutics is advancing an open-label, dose-escalating Phase 1/2 trial designed to assess the safety and preliminary efficacy of subretinal administration of investigational *SPK-7001*. Choroideremia (CHM) is an X-linked IRD that usually manifests in affected males during childhood as night blindness and a reduction of visual field, followed by progressive constriction of visual field, ultimately leading to complete blindness.

Other preclinical programs in our pipeline include investigational gene therapies for Stargardt disease, Hereditary angiodema (HAE), and a Huntington's disease candidate that we have in-licensed.

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