Striving to challenge the inevitability of genetic disease by discovering, developing and delivering treatments in ways unimaginable – until now.

The development of investigational LUXTURNA™ (voretigene neparvovec)

Investigational LUXTURNA is currently under Priority Review with the U.S. Food and Drug Administration (FDA) for the treatment of patients with vision loss due to confirmed biallelic *RPE65* mutation-associated inherited retinal disease. LUXTURNA is also under review with the European Medicines Agency (EMA) for the treatment of patients with vision loss due to Leber congenital amaurosis (LCA) or retinitis pigmentosa caused by confirmed biallelic *RPE65* mutations.

April 2012
Orphan drug designation
received from the European Medicines Agency (EMA) for LCA

November 2012
Pivotal Phase 3 clinical trial initiated

March 2013
Spark Therapeutics
founded

November 2014
Breakthrough therapy designation
received from U.S. FDA

October 2015
Announcement of successful completion of the first randomized, controlled Phase 3 trial
of a gene therapy for a genetic disease

July 2016
Safety and efficacy data
in contralateral eye from a Phase 1 study published in *The Lancet*

October 2016
Two-year efficacy and safety data
from Phase 3 study presented at the American Academy of Ophthalmology 2016 Annual Meeting

January 2017
Four-year efficacy and safety data
shown from a Phase 1 follow-on study

January 2017
Amended FDA orphan drug designation announced for "the treatment of inherited retinal dystrophy due to biallelic *RPE65* mutations"

July 2017
Pivotal Phase 3 clinical trial
data published
in *The Lancet*

July 2017
FDA accepts for filing
Biologics License Application (BLA) and grants Priority Review with Prescription Drug User Fee Act (PDUFA) date of January 12, 2018

August 2017
EMA validates
submitted Marketing Authorization Application (MAA)

August 2017
Study published
in *Clinical and Experimental Ophthalmology* confirming multi-luminance mobility test's construct and content validity, reliability and ability to detect change in functional vision.

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