

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, reliability and ability to detect change in functional vision.