

IN FOCUS

A PUBLICATION FOR MEMBERS OF THE FOUNDATION FIGHTING BLINDNESS

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“Optimism is the faith that leads to achievement. Nothing can be done without hope and confidence.”

Helen Keller

Approval of First Gene Therapy for the Eye Is Historic Progress in the Advancement of IRD Cures

The December 2017 U.S. Food and Drug Administration approval of LUXTURNA™, the first approved gene therapy for the eye or for any inherited disease, is a groundbreaking milestone for people with vision loss due to mutations in the RPE65 gene. The approval also provides significant momentum to the entire inherited retinal disease (IRD) field, according to leading researchers and community leaders.

LUXTURNA restores vision by delivering working copies of the RPE65 gene directly into the retina thereby compensating for the nonfunctional, mutated copies.

“This new gene treatment will be life-changing for people with vision loss due to mutations in the RPE65 gene; it is also a critical proof of concept for gene therapy and will create momentum for other gene-based treatments — for the eye and other diseases — now in the clinic,” said FFB CEO Ben Yerxa, PhD.

LUXTURNA has already changed lives for people like:

Katelyn Corey, almost gave up her life-long dream of obtaining a degree in science because of declining vision.

But Corey’s future changed dramatically when she enrolled in the Spark Therapeutics Phase 3 clinical trial that demonstrated the effectiveness of



LUXTURNA. Since treatment, Corey completed her degree program and earned a master’s in epidemiology. She currently works as a research analyst.

Christian Guardino, a 17-year-old from Long Island, New York, born with Leber congenital amaurosis (LCA). By his teenage years, vision loss was restricting his school activities and his social life. He had no night vision and had to be home before dark. Guardino enrolled in the LUXTURNA clinical trial when he was 12. He now enjoys 75–80 percent improvement

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>> **Help end blindness. Use the enclosed envelope to make a life-changing gift.**

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