FDA approves first gene therapy for a genetic disease—onetime cure for rare blindness

The first gene therapy to restore sight to individuals who suffer from a rare inherited genetic blindness was approved by the U.S. Food and Drug Administration Tuesday [Dec. 19].

The treatment, developed by Philadelphia drug maker Spark Therapeutics and researchers at Children's Hospital of Philadelphia, represents the first gene therapy for a genetic disease in the United States.

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The approval culminates "decades of research that has resulted in three gene therapy approvals this year for patients with serious and rare diseases," the FDA said.

The other gene therapies, which bio-engineer immune system T cells to attack certain blood cancers, are Novartis' Kymriah and Kite Pharmaceuticals' Yescarta. Kymriah's technology was developed at Penn and CHOP.

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Spark's treatment, to be known by the brand name Luxturna, is intended to be a onetime treatment.

Analysts expect the cost will be $500,000 per eye, or $1 million per patient.

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In October, an FDA advisory committee unanimously recommended approval of Luxturna. At that hearing, Joan O'Brien, chair of the University of Pennsylvania's department of ophthalmology and director of Scheie Eye Institute, told the FDA panel, "My prediction is this work will ultimately transform the lives of individuals, perhaps millions, who are facing a life of blindness."

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This first approval will propel further research into gene therapy treatments for other inherited retinal problems associated with other genes, O'Brien said.

Read full, original post: FDA approves Spark's gene therapy for rare blindness pioneered at CHOP