Gene therapy for blindness may become first ever approved in US

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What could become the first gene therapy to win approval in the United States moved closer to market, with its developer announcing that the medicine had succeeded in a late-stage clinical trial in treating an inherited eye disease that can cause blindness.

The developer, Spark Therapeutics, said the treatment had allowed people with certain so-called inherited retinal dystrophies to more easily maneuver in dimmer light than they could before. The company said it planned to apply to the Food and Drug Administration next year for approval to sell the product.

“We saw substantial restoration of vision in patients who were progressing toward complete blindness,” Dr. Albert M. Maguire, a professor of ophthalmology at the University of Pennsylvania and a lead researcher in the study, said in a news release being issued by Spark.

Dr. Katherine High, Spark’s president and chief scientific officer, said this was the first successful randomized, controlled trial for any gene therapy aimed at an inherited disease.

Gene therapy involves putting healthy genes into the body to take the place of mutated genes that cause disease. There have been hundreds of trials of gene therapy in humans since 1990, and none have resulted in a medicine winning approval from the F.D.A. One gene therapy for an extremely rare disease was approved in Europe in 2012, but there are questions about its effectiveness.

Read full, original post: Eye Treatment Closes In on Being First Gene Therapy Approved In U.S.