

Although LCA typically leads to progressive loss of all vision, new advances in gene therapy offer hope for some patients. Voretigene neparvovec-rzyl (Luxturna(TM)) is the gene therapy product injected underneath the retina, allowing a new, functional copy of the gene to pass into the appropriate cells. Luxturna(TM) requires a common retina surgery procedure called a vitrectomy, and must be done by an ophthalmologist with experience in injecting genes under the retina. This new therapy involves implanting new genes into the abnormal retinal cells to correct the defective gene. A defect in this gene can cause LCA in some patients as well as retinitis pigmentosa (RP) in others. Recently, gene therapy has become available for patients with mutations in both copies of the RPE65 gene. RP is a disease similar to LCA that occurs later in life. It is the first gene therapy approved by the US Food and Drug Administration (FDA) to treat a disease.