FDA has approved voretigene neparvovec-rzyl, a one-time gene therapy product indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy.

It is the first FDA-approved gene therapy for a genetic disease, the first and only pharmacologic treatment for an inherited retinal disease (IRD), and the first adeno-associated virus (AAV) vector gene therapy approved in the United States.

Treatment is delivered surgically via subretinal injection.

The agent should only be administered to patients with mutations on both copies of the RPE65 gene who have sufficient viable retinal cells, as determined by their treating physicians.