

GENE THERAPY 101



VISION HEALTH
ADVOCACY COALITION

Gene therapy is a type of treatment that uses genes to prevent, address or cure a medical condition by fixing the underlying genetic problem. Early gene therapy intervention can help prevent future vision loss and blindness. Gene therapy can also help treat diseases that have few treatment options.

HOW DOES GENE THERAPY WORK?



- Adds new copies of a gene that is broken



- Replaces a defective or missing gene with a healthy version of that gene



- Helps the immune system identify diseased cells

WHAT ARE THE BENEFITS OF GENE THERAPY?



- Helps the body fight disease



- Creates the possibility to target individual genes in the body



- Can stop damage before it occurs



- In some cases, can be curative

WHICH VISION CONDITIONS CAN GENE THERAPY TREAT?

Clinical trials are investigating gene therapy for the treatment of many vision conditions, including retinoschisis, retinitis pigmentosa, usher syndrome, diabetes-related eye conditions, choroideremia, age-related macular degeneration and other genetic vision conditions.

One gene therapy (voretigene neparvovec) has been approved by the FDA for use in the United States to treat inherited retinal diseases.

The success of gene therapy has paved the way for innovative treatments to help vision patients.