Gene Therapy for Inherited Retinal Disorders

Identify the Gene Mutation

Genetic testing identifies the specific gene mutation behind a person’s vision loss or impairment. Since there are hundreds of retinal disease genes, it is important to know the gene mutation to determine treatment eligibility.

The Eye is Easy to Access

Compared to other organs of the body, the eye is small and easy to access for treatment administration. This makes inherited retinal disorders (IRDs) strong candidates for gene therapy.

Vector Delivery

Gene therapy uses a vector—a virus without the disease causing parts—to deliver a working gene into the cells. The vectors are delivered via injection to the eye, which enables the cells with the new functioning genes to start doing the work the faulty genes could not.

Control Progression

By targeting the cause of disease—a faulty gene—gene therapy aims to stop disease progression and improve vision with a one time administration.