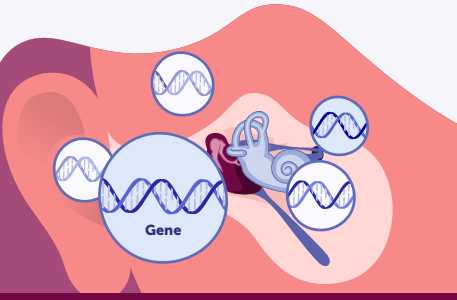
Gene Therapy for Genetic Hearing Loss

A Look Inside the Ear

The human ear is a complex organ with many parts that work together to help us hear. More than half of hearing loss cases present from birth have a genetic cause. There are hundreds of types of genetic hearing loss affecting different genes and different cells in the ear.



Genetic Testing for Hearing Loss

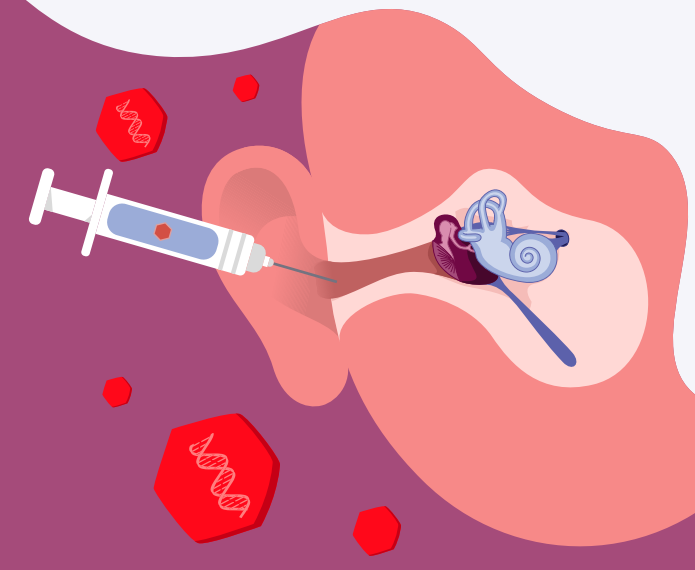
The only way to confirm the cause of hearing loss is through genetic testing. These tests can help inform personalized care with the choice of hearing aids, cochlear implants, use of sign language, other forms of visual communication—or a gene therapy treatment.

It is best to discuss treatment options with a trusted healthcare provider.

How Gene Therapy Works

Gene therapy is being researched in clinical trials to target different underlying genetic causes of hearing loss. Clinical trials are a required part of the research process to investigate if a treatment is safe and effective.

One approach seeks to correct a non-working otoferlin gene (OTOF). This gene produces a protein called otoferlin, which is key for sounds to be transmitted from the ear to the brain. Viral vectors are used to deliver a working copy of the OTOF gene into the cells of the inner ear.



Delivering the Vector

Scientists have learned how to remove the harmful parts of the virus so only the working genes are delivered.

This is administered through an injection to the inner ear. Once delivered, the working OTOF gene can help restore proper protein production and aims to enable hearing capabilities.

American Society of Gene + Cell Therapy

Visit patienteducation.asgct.org for more information