What is gene therapy good for?

ARM Foundation *for* Cell & Gene Medicine

TYPES OF GENETIC DISORDERS

There are 3 basic types of genetic disorders:

1. **Single-gene disorders,** where a variation in one gene creates disease
2. **Chromosomal disorders,** where chromosomes are missing, duplicated, or changed
3. **Complex disorders,** where two or more gene variations and environment are factors in the disease

Currently, gene therapy aims to treat disorders caused by single-gene variations.

Single-Gene Disorders (examples)	Chromosomal Disorders (examples)	Complex Disorders (examples)
Spinal Muscular Atrophy	Down Syndrome (Trisomy 21)	Heart Disease
Blindness caused by Genetic Retinal Dystrophy	Patau Syndrome (Trisomy 13)	Diabetes
Sickle Cell Disease	Edwards Syndrome (Trisomy 18)	Alzheimer's & Parkinson's Disease
Huntington's Disease	Klinefelter Syndrome	Asthma
Hemophilia A & B	Turner Syndrome	Autism

HOW GENE THERAPY WORKS

In human gene therapy, doctors modify a person's genes to treat or alter living cells to restore function, reduce further damage and pain, or potentially cure the patient.

Gene therapies can work by

- Replacing a disease-causing gene with a healthy version of the gene
- Introducing a new or modified gene into the body to help treat a disease
- Inactivating or "silencing" a gene that doesn't function properly

If a disease-causing gene is making an important cell protein function poorly or not at all, gene therapy seeks to restore the gene's ability to make a protein correctly and therefore restore certain functions.

Researchers select the right approach based on the best current understanding of the genetic cause of the disease.