

# The Promise of Human Genome Editing to Confront Devastating Diseases

## The Example of Huntington's Disease

Huntington's Disease (HD) is a fatal, incurable genetic disease that runs in families. HD causes nerve cells in the brain to break down, resulting in progressive deterioration of physical and mental disabilities in the prime years of life.

About **30,000** Americans have HD. **200,000** more are at risk.

### How could genome editing help?

#### BASIC RESEARCH

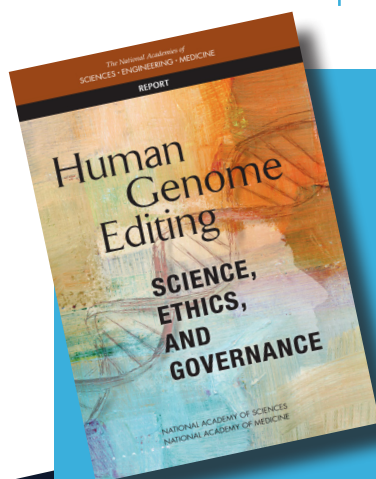
Scientists are already researching how to “delete” the genetic abnormality that causes HD.

#### SOMATIC THERAPIES

“Somatic cells” make up the tissues of the body. One day, doctors might be able to use genome editing techniques in somatic cells to treat someone with HD.

#### GERM CELL THERAPIES

“Germ cells” are reproductive cells that give rise to sperm or eggs. Therefore, characteristics of germ cells get passed to the next generation. One day, doctors might be able to use genome editing techniques in germ cells to ensure that parents with HD don't pass the disease to their children.



Although the science is still developing, human genome editing has great potential to treat and prevent serious, life-threatening diseases like Huntington's, cystic fibrosis, sickle-cell disease, and more. A new report from the National Academy of Sciences and National Academy of Medicine lays out the complex scientific, regulatory, and ethical issues involved in this exciting new frontier.

Learn more: [nationalacademies.org/gene-editing](https://nationalacademies.org/gene-editing)