

Human Genome Editing

Issue Background

A genome is comprised of DNA and functions as the “instruction book” of a cell. Genes are specific strands of DNA which provide the cell with instructions for making the different proteins it needs to properly function. Humans have approximately 20,000 genes, and today there are known to be more than 6,000 genetically based diseases.

Human genome editing is a process by which a DNA sequence is modified to elicit a desired outcome within a living cell. Though DNA modification techniques have existed for decades, recent advances in genome editing technology have provided scientists and researchers with far more precise and efficient genome editing tools. The technology is emerging as an important advancement in the way we treat and cure disease.

Scientists are exploring myriad potential uses for genome editing tools, including clinical, agricultural, and environmental applications. In basic research, genome editing is being used to determine the roles different genes play in disease. Medical researchers are exploring ways to use genome editing to treat or prevent genetically-defined human diseases.

Policy Position

Over the past 40 years, the United States has continuously added to a biomedical R&D framework of laws, regulations, and guidelines to keep pace with advances in genomics. Today, research in genomic medicines is principally governed by the U.S. Department of Health and Human Services (**HHS**) and two of its constituent agencies: The Food and Drug Administration (**FDA**) and the National Institutes of Health (**NIH**).

Current regulatory language in the United States covers human genome editing through its references to “genetic therapies” or “genetic manipulations.” The intention of gene therapy and genome editing clinical applications in somatic cells (i.e. mature non-heritable tissue cells such as liver, lung, or blood cells) is so analogous that the policies in place effectively govern both applications. BIO shares the view reached by other leaders in the scientific and regulatory community, including the National Academies of Sciences and Medicine (NAS/NAM), that clinical applications of genome editing in **somatic cells** can be appropriately evaluated within existing, well-established regulatory frameworks. However, BIO views the science of **germline** genome editing as having not advanced sufficiently for clinical applications to be appropriate at this time.

As scientific developments progress, BIO urges continued discussion and engagement on this topic with important stakeholders, including members of the patient, caregiver, regulatory, legal, academic, ethical, and faith communities, to determine if and under which conditions this status quo should be changed.

Key Points

- ✓ In 2017, the **first genome editing clinical trials in humans initiated in the United States** is targeting a metabolic disorder known as Hunter Syndrome.
- ✓ Genome editing has the potential to someday **mitigate, prevent, or cure** many genetically defined diseases.
- ✓ Research is currently underway on clinical applications of genome editing technologies to treat genetic disorders like **sickle cell disease, cystic fibrosis, congenital blindness, hemophilia, amyloidosis, and lysosomal storage** disorders.
- ✓ In the pipeline, **202 projects using gene therapy** are underway.