ABOUT THE SCIENCE

Engaging on gene editing means connecting technical and scientific conversations with societal values, priorities and perspectives. Below are some basic resources about gene editing and CRISPR that can be helpful in providing background explanations to non-technical audiences in order to help enable full participation.

WHAT IS GENE EDITING?

Techniques to alter the DNA of an organism (genetic engineering) have existed for decades, and scientists have used these techniques to understand the living world and make advances aimed at improving our quality of life. While past methods for genetic engineering have relied on the ability to introduce or add genes into a genome, gene editing can be used to make additions, deletions, or substitutions of almost any DNA sequence within a cell or organism, and to do so with more precision and reliability than ever before.

Although many gene editing tools exist, such as TALENs, ZFNs, and meganucleases, CRISPR is the most commonly used and most versatile among them. Gene editing with any tool, including CRISPR, typically works by precisely targeting and cutting a selected DNA sequence. This cut is then repaired by a cell's natural repair mechanisms, enabling the introduction of new DNA content into the cut site or the disruption of the DNA sequence at the target site.

Gene editing represents a new biotechnological paradigm that is distinct from genetic modification or genetically-modified organisms (GMOs). Briefly, GMOs are organisms that contain introduced genetic material, sometimes from another species, that confer a desired trait or functionality. Gene editing, by contrast, expands the possibilities beyond gene addition, to enable changes that are otherwise indistinguishable from those occurring naturally or through traditional breeding.

WHAT IS CRISPR?

From the IGI's "What is CRISPR?"

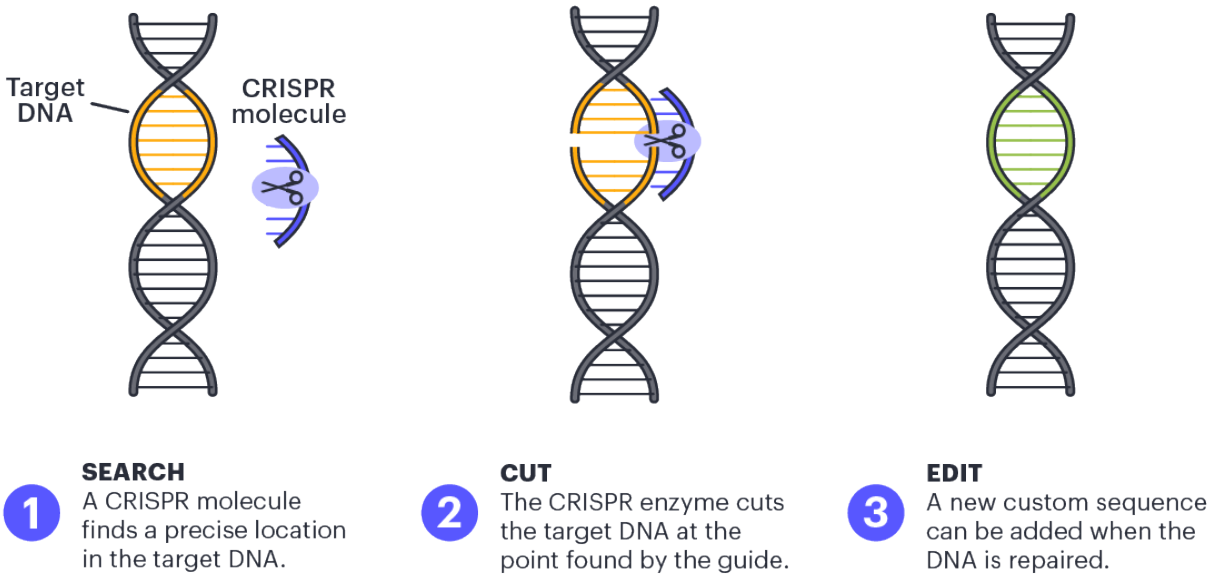
DNA is the instruction manual for life on Earth. It encodes the fundamental properties of an organism — how it lives, grows, and reproduces. Changing a DNA sequence in a living cell is known as genome editing or gene editing. For a long time, this was either impossible or extremely challenging.

The discovery of CRISPR genome editing has made this process much easier. In 2012, research by IGI founder Jennifer Doudna, Emmanuelle Charpentier, and their teams developed a method of repurposing a bacterial immune system called CRISPR — an acronym that stands for Clustered Regularly Interspaced Short Palindromic Repeats — to make breaks in DNA at precise locations, using a CRISPR-associated enzyme (the Cas9 protein) like molecular scissors to cut DNA.

Scientists can now edit the genome of living organisms by adding new fragments of DNA for the cell to use as

a template when it repairs the break in the DNA. In this way, scientists can replace a disease-causing mutation with a healthy sequence or make other modifications to the genome.

Alternatively, scientists can use this method to "knock out" a gene entirely — a technique that is frequently used to study the functions of genes — or to modify portions of the genome that affect how genes are expressed, known as "epigenetic editing." Together, these methods give scientists powerful new tools to treat disease, improve agriculture, and study fundamental questions of biology.



Source: Innovative Genomics Institute

Connecting science and societal benefits

Gene editing is a powerful technology that has the potential to do immense good, but only if implemented safely and in the right contexts. Applying gene editing technologies requires much more than just technological advances – it requires the appropriate sociopolitical landscape, regulatory and safety standards, resources, and public trust. To that end, **societal engagement** around gene editing is critical.