Building a healthier future

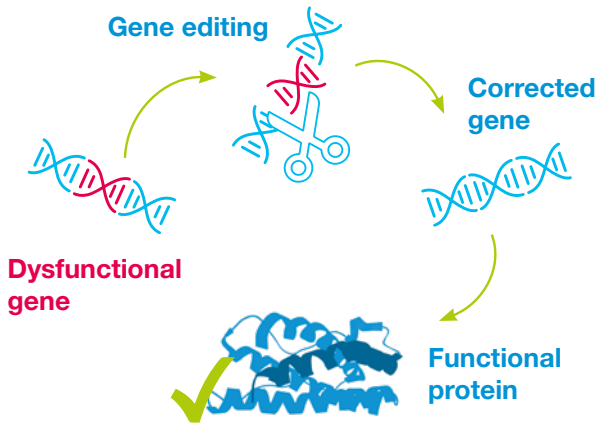
Cell Therapy & Gene Therapy

Gene Editing

How genetic diseases occur and how we want to address them

DNA carries our genetic information and works like an instruction manual for creating life. Although it usually replicates with fairly high fidelity, mistakes do happen and changes in the DNA sequence can occur. These genetic alterations can be inherited or newly acquired and unexpected. While these changes can be harmless, sometimes they alter the functionality of a cell and can be the root cause of one of the thousands of rare and sometimes life-threatening genetic diseases.

Diseases resulting from mutated genes are obvious targets for newly discovered gene editing technologies. This technique enables scientists to, for example, correct faulty genes that cause a specific disease in order to reverse certain symptoms or prevent the disease from occurring in the first place.



Gene editing can correct faulty genes

Gene editing: A biomedical “Swiss army knife”

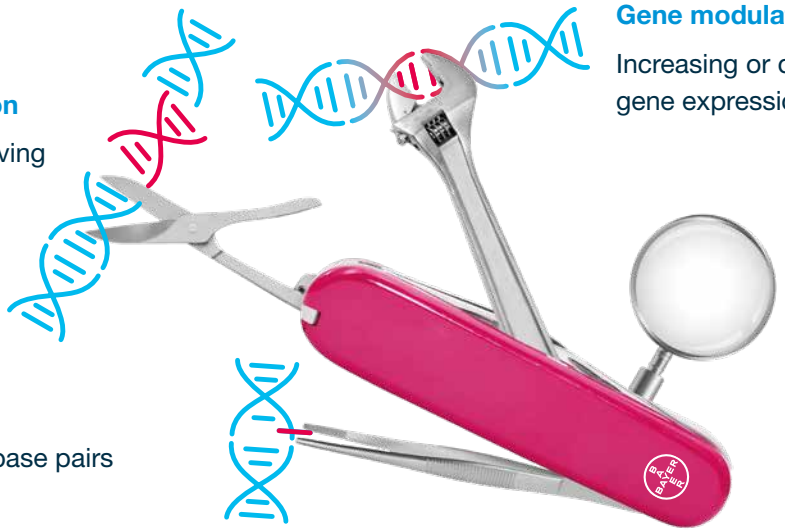
Gene editing is the targeted manipulation of genetic material. Think of it like a Swiss army knife: It offers diverse ways of editing DNA, such as inserting functional genes into cells or correcting mutations in the genome enabling a wide range of therapeutic applications.

Deletion/Insertion

Installing or removing DNA sequences

Point mutation

Changing single base pairs



Gene modulation

Increasing or decreasing gene expression

Outlook

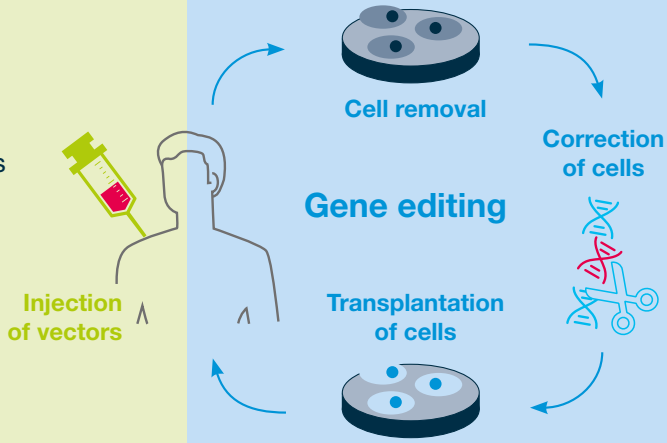
Gene editing is an evolving field that will advance cell and gene therapy

What forms can gene editing take?

Gene editing can take several forms including ex vivo and in vivo gene therapy:

In vivo gene therapy

works by injecting a gene therapy, specifically prepared with the instructions for the job, which can remove unwanted gene sequences or introduce new therapeutic genes directly inside the bodies of patients.



Ex vivo gene therapy

works by isolating cells with a genetic defect from a patient, growing these cells in a culture, correcting the defect, and then transferring these back into the patient's body.

Rewriting the code of life

By understanding the underlying genetics that cause disease, the right editing technique can potentially be coupled with the right disease. Research is ongoing to create gene editing therapies that might be able to combat cancer, blood diseases, infectious diseases, and rare inherited genetic diseases. One example of this is CRISPR, which is being explored in clinical trials as a therapy for diabetes, sickle cell disease, and many other conditions. Gene editing can also serve as enabling technology for cell therapies.

In 2020 Emmanuelle Charpentier and Jennifer A. Doudna received the Nobel Prize in Chemistry "for the development of a method for genome editing". They discovered a way to use a technology known as CRISPR/Cas9 to change the DNA of animals, plants, and microorganisms with extremely high precision. CRISPR/Cas9 is often referred to as genetic scissors.[1]

Bayer's commitment to advancing gene editing

We are supplementing our internal capabilities in gene editing and therapeutic research with Mammoth Biosciences' ground-breaking gene editing platform and Acuitas's lipid nanoparticle technology. Our goal is to develop technologies that will help us deliver better therapeutic options for patients.

While research is still in its early stages, the gene editing space is advancing at a rapid pace. We are investing in innovation now to push beyond what is considered possible, to surpass industry conventions and explore radically new approaches.

2022

We entered into a collaboration with Mammoth Biosciences, accelerating our gene editing capabilities to enable next-generation cell therapies, gene therapies and drive standalone therapeutic applications.

Mammoth Biosciences

2023

We entered into a collaboration with Acuitas Therapeutics in the field of lipid nanoparticles, an important delivery system for gene therapies.

ACUITAS THERAPEUTICS

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1. Press release: The Nobel Prize in Chemistry 2020. NobelPrize.org. Nobel Prize Outreach AB 2022. Tue. 13 Sep 2022. https://www.nobelprize.org/prizes/chemistry/2020/press-release/