

Building a
healthier future

Cell & Gene Therapy

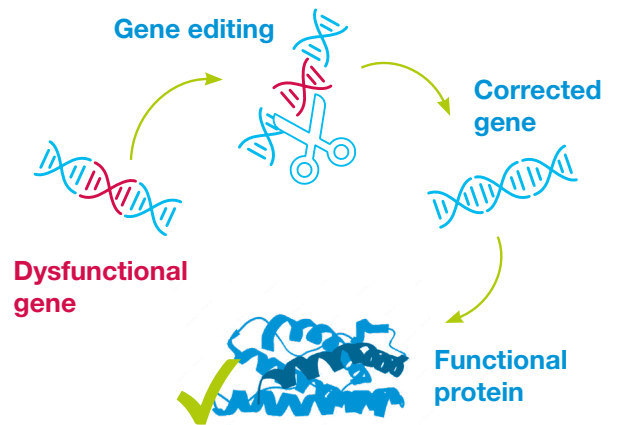


Gene Editing

How genetic diseases occur and how we want to address them

DNA constitutes our genes 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 a diversity of ways to edit DNA, such as inserting functional genes into cells or correcting mutation 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.

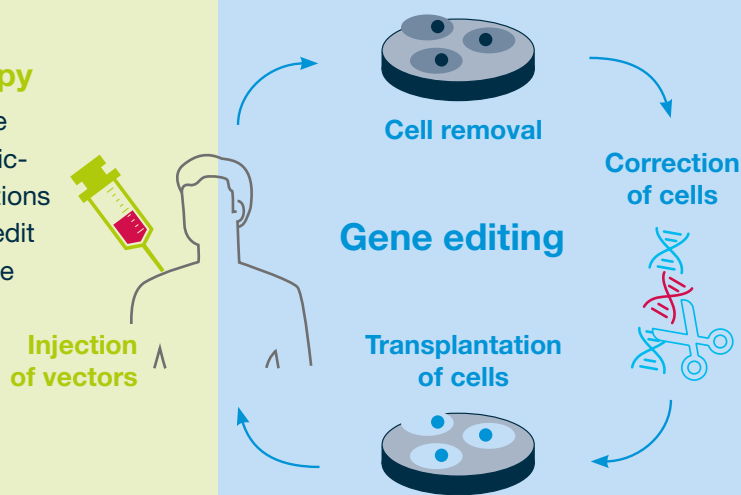


What forms can gene editing take?

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

In vivo gene therapy

works by injecting gene therapy vectors, specifically coded with instructions for the job, which can edit 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, introducing the therapeutic gene into them, and then transferring these back into the body to help fight a disease.

Rewriting the code of life

By understanding the underlying genetics that cause disease, the right editing technique can be coupled to the right disease. Research is ongoing to create gene editing therapies that can 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, and many other diseases. 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

Supplementing our internal capabilities in gene editing and therapeutic research with Mammoth Biosciences' ground-breaking gene editing technology, we are working 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.



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In 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.



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/>