KUNGL. VETENSKAPS-AKADEMIEN
THE ROYAL SWEDISH ACADEMY OF SCIENCES

The Royal Swedish Academy of Sciences has decided to award the Nobel Prize in Chemistry 2020 to **Emmanuelle Charpentier** and **Jennifer A. Doudna** "for the development of a method for genome editing".

The Nobel Prize 2020 **in Chemistry**

Genetic scissors: a tool for rewriting the code of life

Emmanuelle Charpentier and Jennifer A. Doudna have been awarded the Nobel Prize in Chemistry 2020 for discovering one of gene technology's sharpest tools: the CRISPR/Cas9 genetic scissors. Researchers can use these to change the DNA of animals, plants and microorganisms with extremely high precision. This technology has revolutionised the molecular life sciences, brought new opportunities for plant breeding and may make the dream of curing inherited diseases come true.

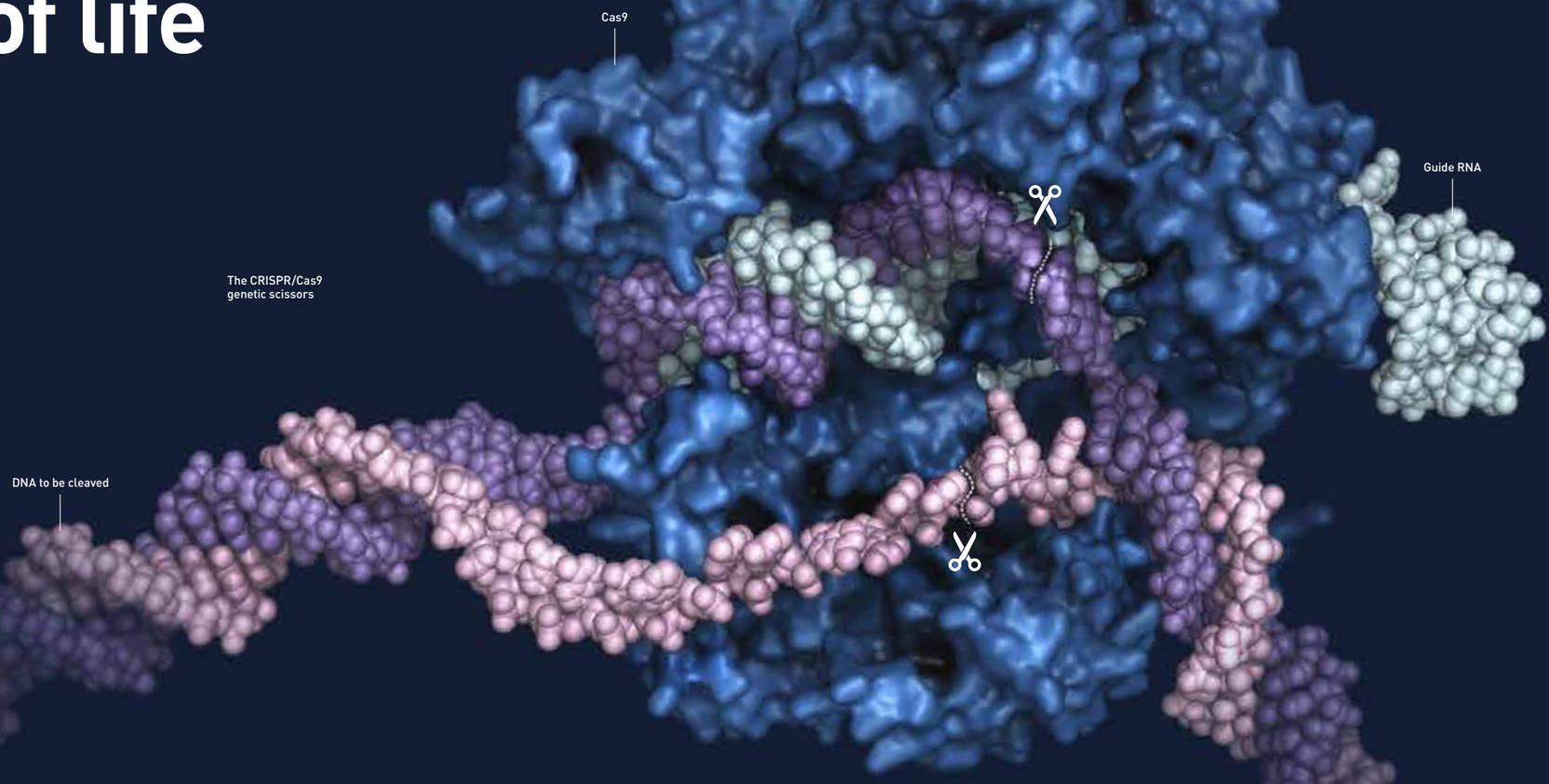
Researchers need to modify genes in cells if they are to find out about life's inner workings, which used to be time-consuming and sometimes impossible work. A cell's genome is like an enormous encyclopaedia with thousands of volumes, so localising a specific gene and rewriting its code was more difficult than finding a needle in a haystack. However, thanks to the genetic scissors, CRISPR/Cas9, it is now possible to change the code of a gene over just a few weeks.

As so often in science, the discovery of these genetic scissors was unexpected. **Emmanuelle Charpentier** was studying a pathogenic bacterium, *Streptococcus pyogenes*, when she discovered a previously unknown molecule, tracrRNA, which turned out to be an important part of the bacterium's ancient immune system, CRISPR/Cas.

CRISPR/Cas protects bacteria from viruses. The system forms a type of molecular scissors, which recognises and cleaves the virus DNA. Emmanuelle Charpentier started collaborating with **Jennifer Doudna**, and they succeeded in recreating the bacteria's genetic scissors in a test tube.

In an epoch-making experiment, they then reprogrammed the genetic scissors. They proved that the scissors could be controlled so any DNA molecule can be cut at a predetermined site. Where the DNA is cut, it is easy to rewrite the code of life.

Since Charpentier and Doudna discovered the CRISPR/Cas9 genetic scissors in 2012, the use of this tool has exploded. This method is hugely powerful and affects us all.

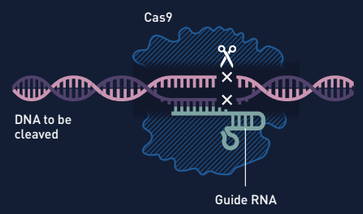


Emmanuelle Charpentier
Born 1968 in France. Director of the Max Planck Unit for the Science of Pathogens, Berlin, Germany.

Jennifer A. Doudna
Born 1964 in the USA. Professor at the University of California, Berkeley, USA.

Genetic scissors that cleave DNA

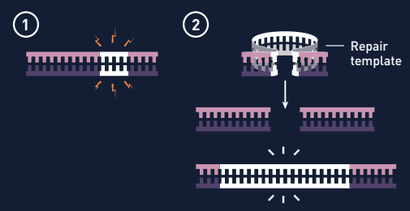
When researchers are going to edit a genome, they artificially construct a *guide RNA*, which matches the DNA code where the cut is to be made. The scissor protein, *Cas9*, forms a complex with the guide RNA that takes the scissors to the correct place in the genome.



Life's code is rewritten when DNA is repaired

Researchers can allow the cell itself to repair the cut in the DNA (1). In most cases, this leads to the gene's function being turned off.

If the researchers want to insert or edit a gene, they can specially design a small DNA template for this (2). The cell will use the template when it repairs the cut in the gene, resulting in a change in the genetic code.



Important tool for basic research

Researchers around the world now use these genetic scissors to understand how genomes govern the functioning of cells and organisms. By deactivating or editing a gene, they can find out what function it has, such as during the course of a disease.

Revolutionising plant breeding

Plant researchers have been able to precisely develop crops that better tolerate drought in hot climates, as well as ones which resist mould and pests that would otherwise require the use of pesticides. For example, they have modified rice so that it does not absorb toxic heavy metals, such as arsenic, from the soil.

Hope of curing inherited diseases

In medicine, the genetic scissors are contributing to new immunotherapies for cancer and trials are underway to make a dream come true – curing inherited diseases. Researchers are already performing clinical trials to investigate whether they can use CRISPR/Cas9 to treat blood diseases such as sickle cell anaemia and beta thalassemia, as well as inherited eye diseases.

Genome editing is only used to treat specific tissues, since editing sex cells or embryos in a manner that makes these changes inheritable is currently prohibited. Humanity will encounter various new ethical issues due to the potential offered by these genetic scissors, although this tool will also contribute to solving many of the challenges now facing the world.

LEARN MORE ABOUT THE NOBEL PRIZES AT WWW.KVA.SE More information about the Nobel Prize in Chemistry 2020 is available at *www.kva.se/nobelchemistry2020* and *www.nobelprize.org*, with detailed information about the Prize and the Laureates, suggested reading and videos.

Editors: Claes Gustafsson, Gunnar von Heijne, and Pernilla Wittung Stafshede, The Nobel Committee for Chemistry, The Royal Swedish Academy of Sciences; Ann Fernholm, Science Writer; Clare Barnes, Translator; Sara Gustafsson, Editor; and Lovisa Nycander, Nobel Assistant, The Royal Swedish Academy of Sciences. **Graphic design:** IVY Agency AB **Illustrations:** Johan Jarnestad/Infographics.se **Print:** Åtta45

Printing and distribution made possible by VOLVO

Box 50005, SE-104 05 Stockholm, Sweden
+46 8 673 95 00, kva@kva.se, www.kva.se
Posters may be ordered free of charge at www.kva.se/nobelposters