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 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 encyclopedia with thousands of volumes, so locating 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, called Cas9, and a guide RNA. Emmanuelle Charpentier started collaborating with Jennifer Doudna, and they succeeded in recreating the bacterial 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.

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. The scissors make it possible to investigate whether they can use CRISPR/Cas9 to treat blood diseases such as sickle cell anaemia and beta thalassaemia, as well as inherited eye diseases.

Hope of curing inherited diseases

In medicine, the genetic scissors are contributing to new treatments for cancer and many other 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 thalassaemia, as well as inherited eye diseases.

Genome editing is only used to treat specific diseases, since adding new cells or embryos is a manner that makes these changes inheritable and 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.