



Genome editing FAQ

What is genome editing?

Genome editing is an advance in biological tools with the potential for a wide range of applications. Genome editing works much like editing text on a computer. First, a specific search is conducted within the organism's genome ("text") to locate the place where a specific change in the DNA sequence ("letters") is desired. Then, the genome editing tool acts as a pair of biological 'scissors' to 'mark' that place by cutting DNA between the "letters". Finally, the desired DNA sequence ("letter") changes are accomplished with the help of a natural process which relies on the cell's own repair mechanism that can add or delete "letters" or "edit" them (substitute one letters for another). These changes to an organism's own "letters" (DNA) result in a specific characteristic. This could be e.g. the correction of a malfunctional gene. The terms 'New Genomic Techniques (NGTs)' and formerly 'New (Plant) Breeding Techniques (NBTs/NPBTs)' are used by the EU institutions to describe a set of techniques which includes but is not limited to genome editing. When applied to plants, Genome Editing is an important part of Plant Breeding Innovation (PBI).

What is CRISPR?

CRISPR (**C**lustered **R**egularly **I**nterspaced **S**hort **P**alindromic **R**epeats), pronounced "crisper" or also referred to as CRISPR-Cas) is a recently developed, efficient and versatile genome editing tool. The CRISPR-Cas system was originally discovered as a bacterial "immune system" that confers resistance to viruses. Within the natural system, specific sequences within viruses (CRISPR sequences) are recognized by the pair of bacterial biological 'scissors' (Cas enzyme) in order to protect bacteria from viral infection.

Other genome editing tools include zinc finger nucleases (ZFNs) and transcription activator-like effector nucleases (TALENs) but they all function using the same principles of biological 'scissors'.

Why do scientists use Genome Editing?

Because of its precision and efficiency.

State-of-the-art genome editing approaches are intrinsically more precise and accurate to achieve a desired improvement as compared to the more traditional approaches. The use of the latest genome editing techniques with their ability to produce very precise and efficient changes at targeted sites of the genome enables

better control of the product's characteristics, as stated inter alia by the [European Commission's Scientific Advisory Mechanism \(SAM\) in 2017](#), the [2018 statement by the Group of Chief Scientific Advisors](#), and by the [European Academies' Science Advisory Council](#) in 2020.

Another advantage of using genome editing is that it can make the breeding process or microbial strain development process much more efficient. However, this advantage in R&D would be lost if the products then would have to face years of regulatory delay before being put onto the market. In addition, the potential markets for many products are small compared to the regulatory investment which would be required to place these products on the market, i. e. it is entirely impossible to recoup investments in these cases.

How is Genome Editing different from other techniques?

In conventional breeding, breeders do not know exactly where in the genome the changes occur. In contrast, genome editing induces targeted modifications in genes. What form the modification takes at the defined location depends on how the tools are used as part of the genome editing process. Many applications of genome editing produce organisms that could otherwise be found in nature or developed with traditional tools. Such organisms are “non-transgenic” – they do not contain “foreign” DNA – DNA from a different species. The final result (DNA sequence) offers no clues as to whether a mutation has taken place naturally or as a result of a new scientific technique.

What can be achieved using genome editing?

The private and public sectors are using the tools of genome editing to develop a range of new products especially for healthcare, industrial biotechnology and agriculture (plants and animals). Check out our non-exhaustive [list of over 200 genome-editing or viral vector related products and research projects](#), drawn from various resources and covering healthcare, industrial and agricultural biotech, as well as our [“What if” Factsheets](#) with several examples per sector, and the next FAQs.

What can genome editing bring for healthcare?

In medicine, Advanced Therapy Medicinal Products (ATMPs), the majority of which are NGT-products, have an incredible potential to treat currently incurable genetic diseases, rare conditions and offer durable and life-changing solutions for patients. Some therapies address the root cause of the disease offering patients the prospect of a cure with potentially one intervention only. Cell and gene therapies are sometimes manufactured specifically for one individual patient creating tailored medicine. Currently, ATMPs are developed and/or applied for cancers, such as rare blood & skin cancers, inherited blindness and blindness caused by injury, rare genetic diseases, such as Crohn's disease, epilepsy, Parkinson's disease, Alzheimer's, spinal muscular atrophy, rheumatoid arthritis, diabetes, etc. The healthcare examples EuropaBio has featured in our [“What If” factsheets](#) concern targeted cancer treatments, Childhood blindness, sick blood cells and AIDS. Check out also our [non-exhaustive list of over 200 genome-editing or viral vector related products and research projects](#).

What is the status of genome editing in healthcare?

Europe has been a pioneer in the field of Advanced Therapy Medicinal Products (ATMPs) in terms of their development, authorisation, and regulation, thereby supporting patient access to these life-changing therapies. Between January 2014 - June 2019, 323 investigational clinical trials were initiated in Europe. However, this is less than half of what was observed in North America and Asia, with the number of new clinical trials increasing by <2% in Europe versus 36% and 28% in North America and Asia, respectively.

2019 was a significant year of growth for the advanced therapies sector in Europe. There were 260 ongoing advanced therapy medicinal product (ATMP) clinical trials involving the vast majority of EuropaBio biopharmaceutical member companies. At EuropaBio, more than 50% of member companies are active in advanced therapies.

What can genome editing bring for the bioeconomy and industrial biotech?

The examples EuropaBio has selected for our ["What If" factsheets](#) concern algae to make biofuel, enzymes to produce hydrogen peroxide without petroleum, and wood that is transformed into food preservatives. A few examples of the benefits of gene technology when applied to microorganisms:

- Microorganisms for agriculture: Agricultural crops depend on the soil microbiome (bacterial and fungal community). Introducing genetically engineered microbes into soil microbiomes could for instance improve nutrient or water uptake, with positive effects on stress tolerance. The benefits would be a more consistent yield of crops under challenging climate conditions without the need for additional use of mineral fertilizers or irrigation. Microorganisms can also be used as crop protection against pests to reduce the need for additional plant protection products.
- Microorganisms for animal health and nutrition: improving the utilization of feed, leading to less waste production, impacting the gut microbiome in a positive way (probiotics) and improving the survival rate of the animals. The genetic basis for e.g. antibiotic resistance can be eliminated accurately and easily.
- Microorganisms for food production: e.g. contributing to more efficient utilization of raw materials and reducing food waste.
- Microorganisms for fuel and chemical production based on renewables.
- Performance enzymes, which cannot be industrially produced using wild-type microorganisms, are readily produced by genetically improved microorganisms and used in a wide range of applications such as food production, animal nutrition, cleaning products, biofuels, textile manufacturing, bioplastic, etc.

These benefits are not specific to NGTs; however, the benefits can be realized with higher precision, higher accuracy, and often through more subtle changes needed to realize a particular, desired effect. In addition, they can be reached with higher

technical ease. Check out also our [non-exhaustive list of over 200 genome-editing or viral vector related products and research projects](#).

What is the status of genome editing in industrial biotech?

Genome editing is part of the technology toolbox used in industrial biotechnology for microorganisms used either as production strains in contained use, or in a live form. Due to the precision and ease of genome editing, all genetic engineering work is transitioning rapidly from the older “cloning” techniques to state-of-the art genome editing techniques, be it for single base substitutions or the introduction of entire heterologous genes. Genetically Modified Microorganisms (GMMs) are widely used in the manufacturing of everyday products and pharmaceuticals; for instance, enzymes are used in a wide range of applications in the food, feed, biofuel and detergent industries. Gene technology is for example being used to develop new enzymes. It is a matter of fact that for some, genome editing is already now the norm rather than the exception.

What can genome editing bring for food and agriculture?

There are well over 150 examples of genome edited plants, as well as some genome edited animals in our [non-exhaustive list of over 200 genome-editing or viral vector related products and research projects](#).

Genome editing can improve plant varieties in ways that are beneficial to the farmer (disease resistance, better stress tolerance, etc.) or ways that are beneficial to the consumer (improved nutrition, longer shelf life, etc.). For a recent overview of ongoing research on ways to tackle flooding, salinity, extreme temperatures, to reduce fertilizer use etc., please see [Bailey-Serres et al, “Genetic strategies for improving crop yields” \(Nature, 2019\)](#). Some of these improvements can be achieved with traditional breeding, but that takes much longer than with state of the art NGTs. Efficiency is essential, because the challenges are increasing with the need to double food production by 2050 (according to the FAO) on very limited agricultural land, and with a view to climate change (pests move to new areas, weather extremes, etc). The plants EuropaBio has selected for our [“What If” factsheets](#) are: gluten free wheat, low acrylamide potatoes and healthier oil soya, all of which provide important consumer and health benefits.

What is the status of genome editing for plants?

When it comes to genome edited plants, multinational agri-tech companies are all active in R&D using genome editing, yet they account for only a small proportion of the developers of NGT-derived plants. The majority are public institutions (including universities) and medium sized companies. For details, see our [list of genome edited products and projects](#).

In the USA, a first genome edited crop is now being grown: a type of soya bean which yields heart-healthy oil. The Julius-Kühn Institut (JKI, a German government institute) [listed 140 plants](#) submitted prior to March 2020 which, according to JKI, can be characterised as covering ‘market oriented’ or ‘ready for market’.

The EU's GMO authorisation process, which currently applies also to genome edited organisms, makes very many potential innovations economically unfeasible for the EU

market. The multi-national agri-tech companies have focused their R&D efforts on other parts of the world. This is in line with their decision adopted for transgenic crops several years ago to focus on other continents, particularly North and South America, due to the dysfunctionality of the EU's authorisation process for cultivation of GM crops. Without a favourable reform of the EU's partly dysfunctional regulatory environment, it is likely that very few NGT-derived plant products will be imported into the EU, and that none will be developed and/ or cultivated in the EU.

What is the regulatory status of genome edited products in the EU?

Since a European Court of Justice ruling on 25 July 2018, plants, animals, and micro-organisms resulting from genome editing are to be treated like genetically modified organisms (GMOs)¹ and are governed by the GMO Directive (Directive 2001/18/EC). This approach is in contrast with the regulation of genome edited organisms in many other parts of the world, including North and South America, Australia, and Japan.

When and where will genome edited products be available?

Wide commercialization of genome edited products is yet to come. Genome edited soybeans which yield heart-healthy oil are already on the U.S market, and many other genome edited products are considered as 'ready for market'. (For details, please see our [list of genome edited products and projects](#))

It is unlikely that we will be benefiting from genome edited plants in the EU soon. In agriculture, it has proven virtually impossible to obtain EU authorisation to grow regulated GMO crops in Europe. Even an EU authorisation for GMO crop import, which are all harvested outside the EU, is very costly and lengthy. For industrial biotech and, to some extent, for healthcare, Europe is likely to also lag behind other geographies in the uptake of genome editing. Check out the [Genetic Literacy Project's Global Gene Editing Regulation Tracker](#) for a global regulatory overview and existing products and projects per region.

¹ Court of Justice of the [EU Ruling on Case C-528/16](#) and associated [Press Release](#). [EuropaBio Press Release](#).