ACHIEVING THE POTENTIAL OF GENOME EDITING

The perspective of the European Biotech Industry

EuropaBio®

The European Association for Bioindustries

JUNE 2019

The European Union has a unique opportunity to create headroom for innovation and continued investment for the future by fostering and guiding forward a major scientific breakthrough - genome editing.

Genome editing is an umbrella term for a range of tools[1] that enable changes at targeted sites in the genome of an organism. Applied worldwide by researchers in academia, governmental research institutions and industry, these innovations represent a promising next step in research towards beneficial uses in medicine, agriculture and the bio-economy aimed at addressing some of society's grand challenges.

While researchers, academia, industry and regulatory authorities have in-depth understanding of genome editing in terms of the underlying science and resulting benefits and challenges, this knowledge has not yet been fully passed on to the general public.

To help respond to this need, EuropaBio, the voice of the biotech industry, has developed this paper to bring the latest views from the developers and users of genome editing tools and applications.

Public engagement and proportionate regulatory policies would help ensure that Europe, together with the rest of the world, can reap the great societal and economic benefits that genome editing can bring.

[1] Genome editing methods allow for precise changes at targeted sites and include inter alia ODM (oligonucleo tide directed mutagenesis), and a range of site directed nucleases (SDNs) such as CRISPR-Cas (clustered regularly interspaced short palindromic repeats) and TALEN (transcription activator-like effector nucleases) and ZFN (Zinc Finger Nucleases).

HOW TO ACHIEVE GENOME EDITING'S POTENTIAL

EuropaBio calls upon European decision-makers to create platforms to ensure consumer confidence and to provide regulatory clarity to enable innovation for developing products by genome editing methods.

To this end, EuropaBio calls for:

- an inclusive, fact-based platform for dialogue, information sharing and trust building to be set up by the European Commission that brings together the EU Member States, industry, public researchers, academia and civil society;
- a risk-proportionate, predictable, science-based and non-discriminatory application of existing policy approaches allowing innovation to benefit European society and the environment.

We stand ready to contribute to these efforts with our broad expertise in genome editing as it applies to healthcare, bio-economy and agriculture.

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CURRENT AND FUTURE DEVELOPMENTS ENABLED BY GENOME EDITING

Researchers in public and private institutions across Europe and the globe have embraced genome editing because it is more precise, efficient, versatile and provides ample opportunities to develop new and improve existing processes and products in the areas of:

- Healthcare;
- The bio-economy;
- Food & feed,

all to the benefit of society and the environment. In certain cases, genome editing makes it possible to obtain products comparable or indistinguishable from those produced by natural processes and conventional techniques, such as mutagenesis.

A host of potential fields of application exist for genome editing, such as:

- The development and production of medicines, therapies, diagnostic agents, and vaccines for treatment of human diseases;
- Bio-based chemicals and bioenergy for better environmental stewardship;
- Nutrient-enriched and stress-resilient crops for fighting food insufficiency, and beyond.

For example, existing developments include:

- Therapeutic tools for recognising specific viral DNA;
- Production of bio-based chemicals, like lactic acid and succinic acid, for replacing petrochemical building blocks;
- Non-bruising potatoes for reducing food waste

2

AN EFFECTIVE AND PROPORTIONATE REGULATORY FRAMEWORK IS ESSENTIAL TO SECURE TRUST, INNOVATION, JOBS, INVESTMENT AND TRADE

Over the past decade, the European Commission[2] and EU Member States' regulatory[3] and scientific[4] bodies have been following and closely analysing the development of genome editing tools and applications. In particular, whether the EU framework regulating the use of existing methods for genetic modification[5] applies to products produced by means of genome editing, has been a subject of continuous discussion.

An EU Court of Justice ruling of 25 July 2018 interpreted the EU Directive 2001/18 in such a way that organisms resulting from the modern genome editing tools of directed mutagenesis are uniformly to be treated like genetically modified organisms. The implementation of this ruling can have very negative socio-economic consequences for European citizens and the entire biotech sector.

The Group of Chief Scientific Advisors[6] (November 2018) stated that: "meeting the obligations of the GMO Directive implies cost and long duration of the approval process, which are difficult and onerous to bear, particularly by small and medium enterprises". Therefore, they recommend "revising the existing GMO Directive to reflect current knowledge and scientific evidence, in particular on gene editing" and state that the GMO Directive is "no longer fit for purpose".

[2] New techniques in Agricultural Biotechnology, High Level Group of Scientific Advisors. Explanatory Note 02/2017, European Commission Scientific Advice Mechanism (SAM)

[3] New Techniques Working Group: Final Report, 2012

[4] EFSA Scientific Opinion addressing the safety assessment of plants developed using Zinc Finger Nuclease 3 and other Site-Directed Nucleases with similar function (2012) EFSA Journal 10(10):2943

[5] Directive 2001/18/EC of the European Parliament and of the Council of 12 March 2001 on the deliberate release into the environment of genetically modified organisms and repealing Council Directive 90/220/EEC and Directive 2009/41/EC of the European Parliament and of the Council of 6 May 2009 on the contained use of genetically modified micro-organisms.

[6] A Scientific Perspective on the Regulatory Status of Products Derived from the Gene Editing and the Implications for the GMO Directive, High Level Group of Scientific Advisors, Statement 11/2018, European Commission Scientific Advice Mechanism (SAM)

3

CONSIDERATIONS FOR DEFINING THE BEST PATH FORWARD FOR EU SCIENTISTS, BUSINESSES, PATIENTS, AND CONSUMERS

To prevent further attrition of biotech's potential to other regions and to boost the EU's competitiveness and innovation, and reach environmental and climate commitments, we call for a change in the EU regulatory framework. Our goals is to obtain science-based rules that accommodate scientific progress and ensure that organisms developed through genome editing are not subject to disproportionate regulatory requirements, in particular when the same products could also be obtained through earlier breeding methods, classical mutagenesis or could simply result from spontaneous processes in nature.

Guided by the needs of society and supported by the science, EU decision-makers should engage with public and private researchers in communicating to their citizens on the uses of these tools hence raising societal awareness of the benefits. It should be recognised that with needed medical breakthroughs, improved food and feedstuffs and industrial production methods, and products that are environmentally beneficial, genome editing is a vital part of securing Europe's place as a global leader in science, innovation and competitiveness.

Only a proportionate, predictable, fit-for-purpose and science-based policy approach, providing equal regulatory treatment to equivalent products independent of their production method, will enable to leverage the full potential of genome editing to benefit citizens, the economy and the environment.

Finally, both public and private ventures applying genome editing in various areas should continue working towards enhancing the precision and efficiency of these tools while strictly abiding by ethical codes for conducting responsible research.

EuropaBio calls upon European decision-makers to take policy action. The European Commission should take an active role in bringing stakeholders together and should provide proportionate regulatory policies to enable innovation for developing products by genome editing methods.

To this end, EuropaBio calls for:

- a fact-based platform for dialogue, information sharing and trust building to be set up by the European Commission that brings together policy makers, public researchers, civil society and industry;
- science-based, predictable and proportionate rules that reflect technical progress and that seek to ensure that organisms developed with more sustainable, precise, modern mutagenesis techniques are not subject to disproportionate regulatory requirements.

We stand ready to contribute to these efforts with our broad expertise in genome editing as it applies to healthcare, bio-economy and agriculture.

About EuropaBio

EuropaBio, the European Association for Bioindustries, is the recognised voice of the European biotech community championing world-class solutions for society's challenges. EuropaBio and its members are committed to the socially responsible use of biotechnology[7] to improve the quality of life, to prevent, diagnose, treat and cure diseases, to improve the quality and quantity of food and feedstuffs and to move towards a bio-based and zero-waste economy. EuropaBio represents 80 corporate and associate members and bio-regions, and 15 national biotechnology associations in turn representing 1800 biotech SMEs.

[7] EuropaBio's Core Ethical Values, June 2016

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