THE FUTURE OF THERAPEUTIC GENOME EDITING IN EUROPEAN HEALTHCARE SYSTEMS

The future of genome-editing in European healthcare systems

Commissioned by:
Alliance for Regenerative Medicine
CRISPR Therapeutics
Intellia Therapeutics

Endorsed by:
Ms Maria Teresa Giménez Barbat,
Member of the European Parliament
INTRODUCTION

This report has been developed following the successful conference on “Conversations on Science, Regulation, and Society - the Future of Genome Editing in European healthcare systems”, held in the European Parliament on 25 October 2018 and hosted by Ms María Teresa Giménez Barbat, Member of the European Parliament.

Genome editing is a breakthrough healthcare technology which has the potential to significantly change treatment options and impact current healthcare models. Whilst a topic of global significance, the focus of this report is the discussions and developments taking place in the European Union (EU).

To advance the discourse on genome editing and explore how these technologies can become available for patients, the report brings together the views of representatives from government, industry, patient communities, and civil society. This report does not suggest solutions to the challenges genome editing may face, but rather spotlights opportunities it may bring to encourage continued dialogue. Discussions on pricing and reimbursement are not the focus of this report.

Focusing exclusively on the human health and therapy applications of the technology, the contributions of the authors are grouped around three broad themes:

- Healthcare innovation in the European Union - Fostering an environment that promotes genome editing in a healthcare system
- Genome Editing’s Potential – The patient experience and CRISPR/Cas9
- Making Genome Editing a Reality – The case for cross-sectoral collaboration

Evolution to personalised medicine

Genome editing is not a new concept, it has been explored and discussed for decades. However, new technological breakthroughs have made genome editing more viable as a healthcare intervention. Genome editing is a group of technologies that give scientists and healthcare professionals the ability to add, remove, or adapt genetic material in an organism’s genome. One such technology, which is the focus of this report and has played a key role in the viability of genome editing as a technology, is CRISPR/Cas9.

The growing prevalence of these technologies have played an important role in the evolution toward a more personalised approach to healthcare. With scientific progress, health systems are moving away from the “one size fits all” approach, instead treatments are tailored to defined groups and individuals.

Genome editing in the European context

The 1975 Asilomar Conference on Recombinant DNA, which instigated a moratorium on the genetic modification of humans, led to the first significant regulation of all forms of genetic modification. Over the last 45 years, the recombinant DNA technology has significantly advanced, making the use of genetic modification in disease treatment and cure a potential reality. Despite these advances, the regulatory environment has not kept pace with the rapid scientific developments in the field of genome editing. There is an urgency to develop a consensus between scientific, legal and regulatory communities as to whether this transformative technology should be regulated as technology in itself, or whether the individual techniques and products which are collectively referred to as genome editing should be controlled on a case-by-case basis.

In March 2019, the World Health Organization (WHO) took leadership in the field of genome editing research. By establishing a new advisory committee, which brings together some of the world’s leading experts, the committee will develop global standards for governance and oversight of human genome editing. The WHO is seeking to develop essential tools and guidance for the use of genome editing technology to ensure maximum benefit and minimal risk to human health.

The EU has taken initial steps to clarify the regulatory ambiguity around genome editing technology. In 2014, the European Commission (EC) took stock of the situation around advanced therapy medicinal products (ATMPs), the concept which includes genome editing and other medicines that are based on genes, tissues or cells, and analysed the impact of the EU’s ATMP Regulation. In its report, the EC concluded that the ATMP Regulation had protected patients from
unsound treatments. However, it also recognised shortcomings and identified actions such as reducing discrepancies across the EU regarding the application of genetically modified organisms (GMOs) rules, to help translate scientific progress into medicinal products available to patients.

As part of the EU’s Action Plan on ATMPs, the EC and the European Medicines Agency (EMA) organised an expert group meeting on genome editing technologies on 18 October 2017\(^9\) to explore opportunities and challenges to develop, manufacture, and evaluate these technologies to make them available to patients. On the legislative side, the Court of Justice of the European Union (ECJ) ruled\(^9\) that organisms obtained by new techniques of directed mutagenesis, including genome editing such as CRISPR/Cas9, are considered GMOs, and that they are subject to the obligations laid down by the EU GMO Directive.\(^10\)

The untapped potential of genome editing is extensive. However, in order for patients to reap its rewards, a conversation among the different stakeholders is imperative. This report aims to bring together different voices to drive the conversation and prevent genome editing from slipping down the healthcare agenda.

“Genome editing is an example of new development paradigms that we see progressing with unprecedented speed. It is not a new concept, but recent advancement in science and technologies have generated a wealth of new research that needs to be translated into real treatments for patients,”\(^11\) stated Prof. Giudo Rasi, Executive Director of the European Medicines Agency (EMA) in October 2017.

Europe is one of the leading hubs for healthcare innovation. Over the past five years, numerous projects and initiatives have been launched to accelerate the development of disruptive technologies and translate theory into practice. While efforts are underway to encourage the development of genome editing technologies, some obstacles remain in facilitating their life-saving potential. This chapter gives an overview of the European political ambition and contextualises the European legislative framework to identify barriers and opportunities to making genome editing treatments a reality in Europe.
A TRIUMPH OF EUROPEAN SCIENCE

Genome editing is a game-changing technology and a great triumph of science. Europe has played a leading role in advancing and developing CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) technology. Dr. Francisco J. Martínez Mojica, a microbiologist at the Spanish University of Alicante and one of the early investigators of CRISPR describes the technology as “the greatest genetic revolution in life and health sciences, so far in this century”.

The appropriate medical and therapeutic use of genome editing technologies, these wonderful “molecular scissors”, is precisely what concerns many regulators and policy-makers around the world. European institutions will not remain indifferent to medical and scientific advances that have the potential to vastly improve the lives of patients. This is particularly true of the European Parliament, an institution whose Members are directly elected by European citizens and thus, very close to patients’ needs and concerns.

The expert panel at the event comprised a balanced representation of the main stakeholders involved in this wide-ranging debate, from high-level policy-makers to patient advocates, religious representatives, the research community, and the Biotechnology industry. I would like to thank once again Maria Pilar Aguar Fernández, Janet Lambert, Dr. Thomas Barnes, George Constantinou, Prof. Paolo Gasparini, Nick Meade, Dr. Steve Caffé, and Monsignor Tomasz Trafny for their participation in this important discussion and contributions to this report.

I hope that this publication will help to guide us, and especially the newly-elected Members of the European Parliament, on a new path where trust and hope for scientific research and healthcare improvement must prevail over unformed fears and ideological obstacles. Europe has a clear role to play in the coming years to ensure balanced and intelligent regulation for our health systems, reconciling human and scientific progress. I am convinced that the European Union will continue to be an essential actor to preserve and improve the health and quality of life of patients, and ensure that the Union as a whole remains competitive.

THE GENOME EDITING LANDSCAPE TODAY AND TOMORROW: OPPORTUNITIES AND CHALLENGES

Genome editing is a fascinating, rapidly developing area of regenerative medicine and advanced therapies. These therapies treat the underlying cause of a disease or disorder, rather than focusing on long-term symptom management. They can provide profound and durable responses – perhaps with only a single administration – to patients with a diverse array of serious diseases and disorders, many of whom currently have limited or no treatment options available. Genome editing and other regenerative medicine technologies have the potential to improve the standard of care for hundreds of thousands of patients worldwide and radically transform the healthcare landscape. This technology is not a distant hope, but a fast-approaching reality. Therapies utilizing ZFNs have been in the clinic for several years, with the first results from a clinical trial to treat HIV published in 2014. Newer gene editing technologies, including CRISPR, have begun to enter the clinic as well.

The global genome editing landscape

The genome editing sector is surging. As of August 2019, there are 55 companies worldwide, including 16 clinical-stage companies, active in developing therapies utilizing genome editing technology. Of those, 11 companies, including four clinical-stage companies, are headquartered in Europe.

Genome editing is a maturing field with a number of milestones anticipated in the coming years. There are currently 31 clinical trials ongoing in genome editing, CRISPR Therapeutics and Vertex are conducting the first clinical trials of CRISPR in Europe and the U.S., where they hope to treat patients with beta thalassemia and sickle cell disease.
Europe and beyond: opportunities & challenges

The continued expansion of the sector holds immense promise for patients; such rapid growth and sustained interest represents the confidence of scientists, investors, and other stakeholders in the potential of genome editing. While this will help ensure patients in Europe have access to safe, effective, and EMA-approved genome editing products as quickly as possible, it also presents challenges as existing regulatory and reimbursement systems work to adapt to innovative new therapies.

Lack of regulatory clarity and existing international dissonance in the requirements for the research and development of genome editing products can present a barrier to research and development, and create duplicative reporting and submission requirements when looking to expand a product cross-border. These requirements can be particularly onerous for small startups in this space.

Genome editing technologies also challenge existing healthcare models as single-administration therapies, which have the potential to provide significant, long-term savings to healthcare systems, but often come with high-upfront costs. In Europe, questions of cross-border healthcare and duplicative requirements of national health technology assessments (HTAs) can also delay patient access to novel therapies.

Fortunately, stakeholders in Europe and globally recognize the challenges that outdated, and ill-fitting regulatory systems pose to the uptake of genome editing products and are working together to find solutions.

Moving forward

Over the past decade, genome editing has made considerable strides forward. Genome editing products are already impacting patients in clinical trials worldwide. The science is advancing rapidly, promising considerable increases in the efficacy and precision of genome editing products. Policymakers are focused on finding solutions to the regulatory questions posed by potentially curative treatments for many serious diseases.

Genome editing technologies represent an incredible improvement in the standard of care for thousands of patients across the globe. It is imperative that regulators, payors, and other stakeholders recognize the potential of these therapies, and work with industry and patient advocates to ensure that safe and effective products are made available to patients as quickly as possible.
Thalassaemia is a hereditary genetic blood disorder. Current treatment comprises of two main components: frequent and regular blood transfusions and iron chelation therapy. In addition, it is necessary to monitor iron levels to limit, or if possible avoid, complications to vital organs which may lead to a lower quality of life and disabilities.

Advances in curative methods of the disease, such as gene editing, represent a miracle. Thalassaemia has a high disease burden, accounting both for the time spent for treatment, monitoring the disease status, and the financial weight endured by patients and their families, where effective multidisciplinary treatment is not encompassed in a universal health coverage healthcare system.

The natural history of thalassaemia has radically altered over the last 30 years, with scientific advances transforming thalassaemia from a fatal childhood disease to a chronic illness. With appropriate treatment, the life expectancy and quality of life of patients can be significantly improved. Nonetheless, widespread inequalities in access to quality healthcare in low and medium resource countries, where the large majority of patients live, leads to increased medical complications, disabilities and lower life expectancy. Thus, research into curative approaches provides anticipation and hope globally.

Role of genome-editing

Multiple approaches to cure thalassaemia have recently appeared and others are in the pipeline, including bone marrow transplantation (BMT), gene therapy (GT) and more recently gene editing (GE). BMT is well-established as a curative approach for thalassaemia patients. While eliminating symptoms, the inheritance of thalassaemia to offspring of BMT-cured patients is not altered. BMT–cured patients is not altered. BMT is well established as a curative approach for thalassaemia patients. While eliminating symptoms, the inheritance of thalassaemia to offspring of BMT-cured patients is not altered.

This would allow thalassaemia patients to maintain normal levels of haemoglobin and eliminate the need for blood transfusions, and thereby iron chelation. In contrast to gene therapy, GE precisely targets specific genome regions providing a disease-modifying effect. GE promises to be curative, albeit real patient data has yet to be published.

As in the cases of BMT and GT, there are similar concerns with regards to the effects on fertility. The “off target” effects constitute the major concern of GE approaches that need to be addressed for clinical translation, in addition to the efficiency and toxicity of delivery tools.

GE approaches, aiming to induce the production of BMT, is still in the early stages of ascertaining not having the donor requirements and restrictions (e.g. infertility, immunological complications). GT, liver condition), and side-effects of the treatment factors for success (i.e. clinical status, age and sibling donor; long-term immunosuppression) or fewer than 30% of patients have a fully-matched offspring of BMT–cured patients is not altered. BMT is well established as a curative approach for thalassaemia patients. While eliminating symptoms, the inheritance of thalassaemia to offspring of BMT-cured patients is not altered.

The efficiency of delivery tools.

Thalassaemia in 62 countries, makes significant efforts in this direction. Although the discussion around GE is still in its early days, it is vital for researchers and policy-makers to understand the landscape of diseases, the unmet needs across the world and to provide responses to the concerns of patients. Most notably:

1. Will GE lead to a complete cure?
2. Are there side effects which may lead to a poorer quality of life than before treatment, particularly when compared to existing curative treatments?
3. Are steps being taken to refine the technology to minimize or eradicate off-target effects?
4. How broad and inclusive will the therapy be, considering affordability issues?
5. Will national authorities engage in early dialogue to ensure the appropriate infrastructure is in place for the provision of GE to patients?

Conclusion

Genome editing is a miracle that thalassaemia patients have been awaiting for many years. It is imperative that patients are continuously and reliably informed and involved at all levels to achieve a balance between private interests and public good, and to provide a curative approach that will meet patient expectations and needs.

Advances in curative methods of the disease, such as gene editing, represent a miracle. Thalassaemia has a high disease burden, accounting both for the time spent for treatment, monitoring the disease status, and the financial weight endured by patients and their families, where effective multidisciplinary treatment is not encompassed in a universal health coverage healthcare system. The natural history of thalassaemia has radically altered over the last 30 years, with scientific advances transforming thalassaemia from a fatal childhood disease to a chronic illness. With appropriate treatment, the life expectancy and quality of life of patients can be significantly improved. Nonetheless, widespread inequalities in access to quality healthcare in low and medium resource countries, where the large majority of patients live, leads to increased medical complications, disabilities and lower life expectancy. Thus, research into curative approaches provides anticipation and hope globally.

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Intellia’s goal is to keep developing and advancing genome editing technology to produce curative treatments that can positively transform the lives of people living with severe and life-threatening diseases. Our pipeline is an example of the diversity of diseases that can be addressed. CRISPR/Cas9 and genome editing technologies can play a role in transforming the future of healthcare and play a leading role in improving patient lives.

MAKING GENOME EDITING A REALITY – THE CASE FOR CROSS-SECTORAL COLLABORATION

Genome editing technologies have been described as heralding a “new chapter in medicine.” As a new technology, it may not be well-understood and therefore not broadly accepted. To overcome these hurdles, governments, scientists, industry, patients, and society should all be engaged in a joint dialogue to explore how to use genome editing in a safe and effective manner, driving acceptance within the current healthcare system. This chapter highlights where cross-stakeholder collaboration can play an important role in making genome editing technologies a reality in healthcare.
Advanced Therapeutic Medicinal Products (ATMPs) are complex medicinal products that offer great promise to address some of today’s unmet medical needs. ATMPs include: gene therapy medicinal products (GTMP); somatic cell therapy medicinal products; and tissue engineered products. The development of ATMPs requires levels of high-specialization biomedicine and technical know-how.

The genome editing pipeline is promising, there is a lively research and business space, however, only a limited number of clinical trial developments worldwide. Therefore, it is important that the European Union supports developers by adapting/optimising the application of the regulatory framework to the specific features of these products, issuing scientific guidance and fostering early regulatory dialogue.

In the EU, ATMPs are governed by Regulation 1394/2007 on advanced therapy medicinal products, and receive their marketing authorisation on the basis of this legislation. Although hugely promising, ATMPs can pose risks, including immunogenicity/rejection, they can lead to the development of tumours, and demeritization or loss of cell function. Therefore, ATMPs undergo a thorough review by regulators before they receive authorisation to ensure that products that are available to patients are safe and effective. It is the role of the European Medicines Agency (EMA) to evaluate all ATMPs with the ultimate goal of safeguarding public health by protecting European patients from unsafe and/or ineffective treatments. The evaluation of these products is led by the Committee for Advanced Therapies (CAT) who prepare a draft opinion that is sent to the Committee for Medicinal Products for Human Use (CHMP). The CHMP is ultimately responsible for adopting a final opinion that is shared with the European Commission, which issues a marketing authorisation.

Acknowledging the intrinsic characteristics and variability of ATMPs as distinct treatments, the Regulation governing their authorisation provides for regulatory flexibility. One example is the adoption of a specific Good Manufacturing Practice (GMP) framework. In addition, the EMA relies on the Risk Based Approach (RBA) when providing an opinion on market authorisation for ATMPs. This determines the extent of quality, non-clinical and clinical data that needs to be included in marketing authorisation applications.

As of mid-2018, only 12 products had successfully been authorised as ATMPs in the EU. Of the currently-authorised products, most have been approved in the past five years. As new technologies emerge and more ATMPs undergo clinical trials, it is expected that the number of applications for marketing authorisation will significantly increase. Due to the inherent differences between the types of ATMPs and difference in how the products work, creative thinking is required, and the mentality of the members of the CAT Committee has changed in recent years. The CAT, within certain confines, is willing to work with manufacturers and scientists, to be able to properly evaluate ATMP treatments. Like patients, the EMA is eager to get good products onto the European market.

REGULATION: ARE REGULATORY BODIES READY FOR GENOME EDITING TREATMENTS?

Nick Meade, director of Policy, Genetic Alliance UK

Rare disease patients have a lot in common with each other. Though they may be affected by different rare diseases, their experiences of diagnosis - a slow and difficult process of accessing information; being told not to Google but not much else - are likely to be similar. They will also share the challenge of having a health need that is mostly unmet. Of more than 6,000 rare conditions, only 200 or so have a licensed treatment specifically designed to treat them, and only a tiny handful of these are fully effective at stopping or reversing all symptoms of the condition.

People living with rare diseases look to research and innovation as the source of a solution to the unmet health needs they face. Genome editing has been an exciting prospect for this community for a few years now, as most of rare diseases are caused by genetic disorder. This potential comes in many different forms - genome editing can be used as a research tool to accelerate our examination of the human genome's relationship with rare conditions.

Genome editing can also be used as a treatment: either of somatic cells (cells that are not involved in reproduction) where genome editing can correct glitches (mutations) in the genome that cause a rare condition; or of germline cells (cells that are involved in reproduction) and/or embryos where genome editing could prevent a pregnancy from being affected by a rare condition. With this breadth of potential and opportunity comes a responsibility, for those of us discussing genome editing, to be clear and precise about the scope and use of genome editing. Clarity of scope can help us focus the potential as this, with as much possible applications as this, with as much potential as this, we all have a responsibility to be clear in our communications - researchers, policy-makers, doctors. If we do this, we will be able to bring our two most important stakeholder groups with us: people living with rare conditions and the general public. Only with their understanding and support will we be able to make the most of the potential opportunities of genome editing.

CLEAR COMMUNICATION WITH OUR KEY STAKEHOLDERS

Prof. Paolo Gasparini, Member of the Committee for Advanced Therapies, European Medicines Agency (EMA)

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GENOME EDITING: CHALLENGES AND NECESSITY OF DIALOGUE

Rev. Msgr. Tomasz Trafny, Deputy Commissioner General ad omnia of the Holy See Pavilion

Scientific progress and human health

We are living through a period of profound social and cultural changes. Rapid development of scientific knowledge and technological tools is impacting humanity on different levels. This is particularly true in regard biological and medical sciences as well as biotechnologies like gene editing.

All efforts aimed at helping those who suffer should be much-admired, but more importantly, should be supported on every possible level, especially through investing in research and innovation in biotechnologies and medical sciences. As a matter of fact, for every single discovery with the potential to change patients lives, the longest journey is from labs to patients themselves.

For some people, the voice of the Catholic Church on this particular topic could be considered pointless, if not from the moral or ethical perspective. However, it is worth remembering that although the Catholic Church is deeply interested in ethical dimensions of scientific discoveries, it also cultivates interest for strictly therapeutic purposes of biological, medical and biotechnological findings and their clinical applications, including the issue of gene editing. This is due to the simple fact that the Catholic Church is the largest non-governmental provider of health care services worldwide. Serving those who suffer is one of the important expressions of the scientific community was the very first to raise the issue of gene editing in regards to its potential ethical implications. It shows that the level of discussion among scientists exceeds already more technical issues.

Need of dialogue

In order to positively address questions that will inevitably arise around the issue of gene editing, we should seriously consider the need of a deep and extensive dialogue that goes beyond the closed circles of experts.

Scientific communities are, in general, very sensitive to dialogic processes and increasingly go beyond the usual frontiers of their own investigation or that of the natural sciences, being interested in themes and phenomena traditionally belonging to religion and theological-philosophical reflection, in fact, a correct methodological framework, such a dialogic interaction could originate a positive and fruitful exchange and bring mutual benefits.

However, in regards to some issues like gene editing, there is a need for dialogue that should engage representatives with different themes and backgrounds. Thus, a dialogic interaction could originate a positive and fruitful exchange and bring mutual benefits.

Anthropological interest towards gene editing

Another reason why the topic of gene editing is relevant from the anthropic point of view, is linked to the more general concept of our understanding of nature and more fundamental anthropological questions on identity as a species and consequently on our future.

While applied biology notably accelerates and expands the possibilities of genetic engineering applied also to humans, some interesting ideas emerge that go beyond therapeutic purposes. Some researchers and philosophers claim that genetic engineering can also be used to improve or enhance the human genotype radically. Supporters of this latter idea imagine the human being to be a new edition, updated and strengthened, marking a new frontier in the history of humanity expressed in so-called transhumanism or post-humanism.

From the anthropological and cultural perspectives, many issues are raised by the effort to manipulate DNA opening the path for creating a stronger new genotype. Will strengthened human beings still be part of the species homo sapiens? Also questions of possible future disparities emerge. Will there be new inequalities created between those who belong to the enhanced species and those that remain “normal” or “non-updated” one? What will be the new species’ identity, social status, bond of belonging, and validity of ethical reference?

In this sense, it is worth mentioning that the scientific community was the very first to raise the issue of gene editing in regards to its potential ethical implications. It shows that the level of discussion among scientists exceeds already more technical issues.

EUROPE’S ROLE AS A HEALTHCARE PIONEER

Steve Caffey, MD
Senior Vice-President, CRISPR Therapeutics

Cell and gene therapy products are already reshaping how we view disease; no longer will we be focused on treating symptoms or delaying progression of illness. Instead we can focus on curing the previously-incurable. However, if Europe is unable to provide the stability and predictability needed by innovative companies, they will bypass Europe for the United States and other innovative regulatory systems, including in Asia.

How can the European Union ensure that patients in Europe have early access to and benefit from the life-saving promise of gene-editing?

A regulatory environment has to be created that encourages innovation and, ultimately, facilitates timely patient access to innovative therapies. Patient access to new therapies starts early on, at the clinical development stage, specifically with clinical trial applications. As such, a fast, efficient and streamlined clinical trial approval process is crucial for enabling clinical studies. Until the new EU Clinical Trial Regulation fully comes into effect in 2019, the legislation for clinical trials remains Directive 2001/20/EC. For gene therapies, the Directive allows a 90-day assessment period by regulatory authorities (excluding time for the Sponsor to respond to questions). This can delay the initiation of clinical studies with these treatments compared to other regions. By contrast, clinical trial approval timelines in the US and Canada are 30 days, including the question and answer process between the Sponsor and the Agency. This can make EU Member States less attractive for Sponsors of gene therapy trials and consequently can adversely impact patients’ early access. Clinical trial applications are generally submitted separately in each Member State where the study is intended to take place. This results in a higher administrative burden for multi-site trials. Moreover, Member States may have divergent opinions, in particular when it comes to new technologies, thus potentially complicating the outcome of the regulatory process. Recognising these challenges, the new Clinical Trial Regulation aims to address these challenges and accelerate patient access to safe treatments. A new option to facilitate the GMO application requirements and documentation between the different Member States.

Improving the regulatory environment for clinical trials of genome-edited products is crucial for Europe’s competitiveness, notwithstanding the necessary speed and flexibility in the later stage of review of marketing authorization applications.

What role can Industry play to help Europe become a leader in bringing innovative solutions in healthcare systems?

Industry needs to continue leading in advancing the scientific knowledge in the field of gene-editing therapies. Grounded in the best science with profound knowledge of the biology, superior ethics, and a careful, yet not stifling, approach to balancing potential benefits with well-informed

In implementing clinical trials across EU Member States is that GMO products are subject to very stringent rules. This includes a prior review of the environmental and biosafety aspects of their use and/or release. This additional requirement is a highly-complicating factor. These environmental and biosafety assessments are based on GMO legislation (Directive 2001/18/EC on the Deliberate Release, Directive 2009/41/EC on the Combined Use), which is primarily designed to cover crops and animal genetic modifications rather than medicinal products. These barriers compel biopharmaceutical Sponsors to address information requirements and provide application forms and dosiers which do not allow the necessary flexibility and speed to implement clinical studies of medicinal products containing GMOs. As a consequence, valid and effective treatments may be delayed.

An option to facilitate the GMO application procedures in the EU would be to integrate the environmental and biosafety aspects into the Clinical Trial Application (CTA) process instead of conducting them separately. This would allow for the harmonization of requirements and documentation between the different Member States.

Cell and gene therapy products are already reshaping how we view disease; no longer will we be focused on treating symptoms or delaying progression of illness. Instead we can focus on curing the previously-incurable. However, if Europe is unable to provide the stability and predictability needed by innovative companies, they will bypass Europe for the United States and other innovative regulatory systems, including in Asia.
risk mitigation strategies will continue to produce breakthrough therapies to address many intractable illnesses.

The means to invest in truly innovative products with high manufacturing and development costs is critical in this emerging area with unprecedented development and commercialization paths. Partnering between multiple stakeholders will also be essential to devise new pricing and reimbursement strategies to maximize patient access.

The enormous potential of genome editing and its application for the development of medicinal products to cure very serious diseases can be realized within the right legislative and regulatory environment with the collaboration of all parties and guarantee that patients can benefit from these life-saving technologies.
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14. Approximately 5,000-8,000 distinct rare diseases affect 6-8% of the EU population i.e. between 27 and 36 million people. 80% of rare diseases are of genetic origin, and are often chronic and life-threatening

CONTACTS