

Project Breakthrough

Disruptive Technology Executive Briefs

GENE EDITING

Unlocking the power of biology

Gene editing changes the genetic material (DNA or RNA) of a living cell. It uses a range of different methods and techniques to add, subtract, or replace individual genetic bases and sequences. In medicine, the process of gene editing has enabled the in-depth study of diseases, allowing clinicians and researchers to understand their root causes. This focus on causes as well as on treatment is the most important aspect of gene editing.

While gene editing has primarily been seen as a medical technology, it also has exciting uses in many other fields including agriculture and biofuels where it can create strains of crops or algae that are more resistant to diseases.

Gene editing is a controversial technology as it impacts the building blocks of life, often raising public concerns. However, its increasing use cannot be ignored and awareness of its applications is essential if possible negative impacts are to be managed.

The technology

The leading technology, CRISPR (clustered regular interspaced short palindromic repeats) is a highly cost effective gene editing technology (estimated, by Professor James Haber, Brandeis University, to be 150 times cheaper than other leading technologies). It is also easy to use requiring less training and

education than other technologies. The use of CRISPR makes it possible to locate and edit specific spots on any DNA by disabling or knocking out genes, patching in a new gene, or replacing an existing gene.

The potential

In the short term, gene editing technology could be used to edit T-cells (white blood cells critical to the immune system) to better recognise cancer, or to edit immune cells to mimic a rare, naturally occurring immunity to HIV. This could turn these often terminal conditions to chronic or curable ones.

In the longer term, this work could identify and cure debilitating genetic disorders by editing these defects out of the patient's DNA entirely. This would mean they would never become ill from a known genetic cause.

The barriers

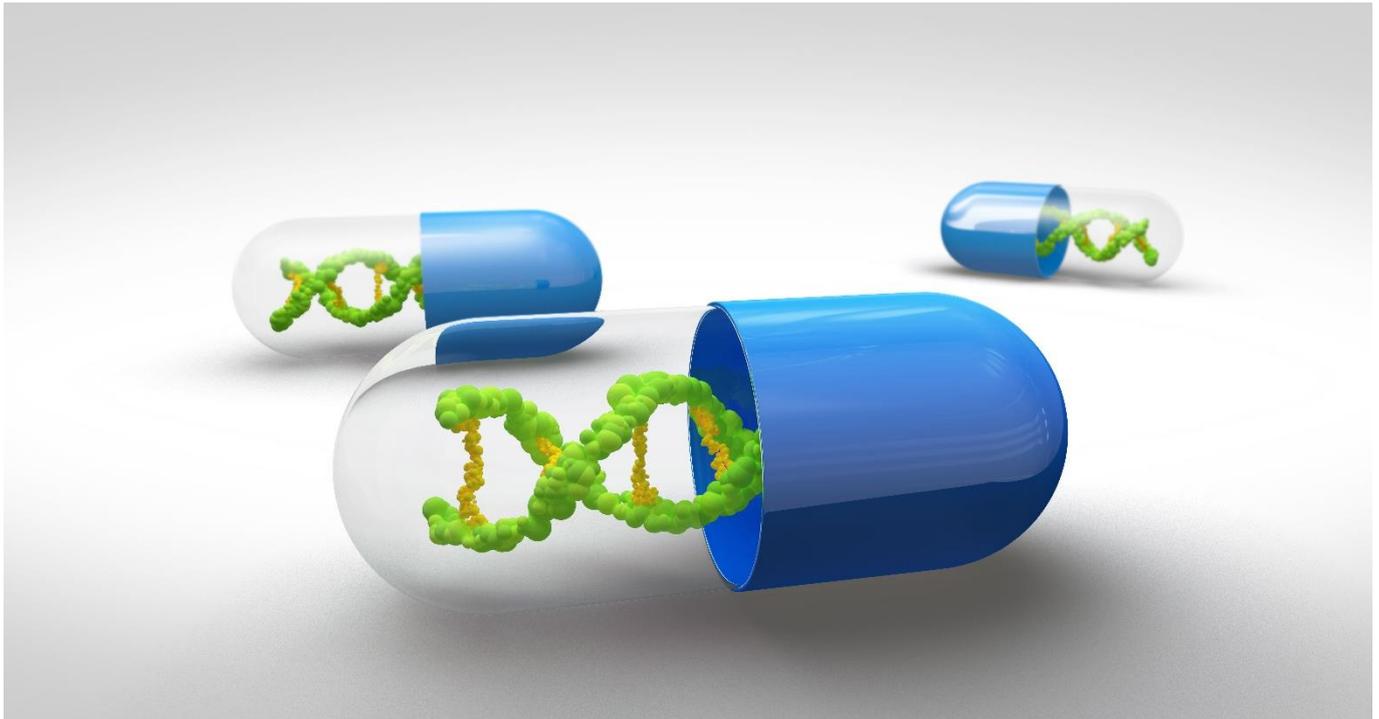
By directly editing DNA this technology has the ability to pass traits on to future generations, and the potential to cause irreversible harm to human health and the environment, whether as the result of intentional misuse or a lack of understanding. For this reason, risks need to be understood and mitigated in order to enable safe adoption and positive benefits.



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Some Example Applications...



New cures for diseases

One interesting example of the potential applications of gene editing comes from a series of experiments performed by the Salk Institute in California. In this study, scientists were able to partially restore the vision of blind rats suffering from the genetic condition retinitis pigmentosa. Genetic diseases in humans, such as cystic fibrosis or muscular dystrophy, could potentially be treated in much the same way.

Another factor which has excited scientists is the precision of gene editing. Unlike pharmaceutical or surgical treatments it focuses only on the affected genes, it does not affect healthy cells or tissue.

Agriculture

CRISPR has the potential to significantly impact agricultural productivity, to benefit of the developing world. In 2016 the first vegetables altered with CRISPR technology were eaten at Umea University, Sweden. The fact that no genetic material is added (unlike some traditional genetic modification techniques), and it is a far more targeted and accurate approach, means CRISPR offers a potential way to address many concerns in this area.

Biofuels and polymers

The University of California have adapted CRISPR technology to develop a yeast strain that enables the production of new precursors of biofuels and polymers. This could provide a more affordable, renewable source of fuel and specialty polymers.

Key Numbers

\$9.5 bn

Estimated market size in 2025

Source: Markets and Markets

\$24 bn

Size of advanced global biofuels market by 2024

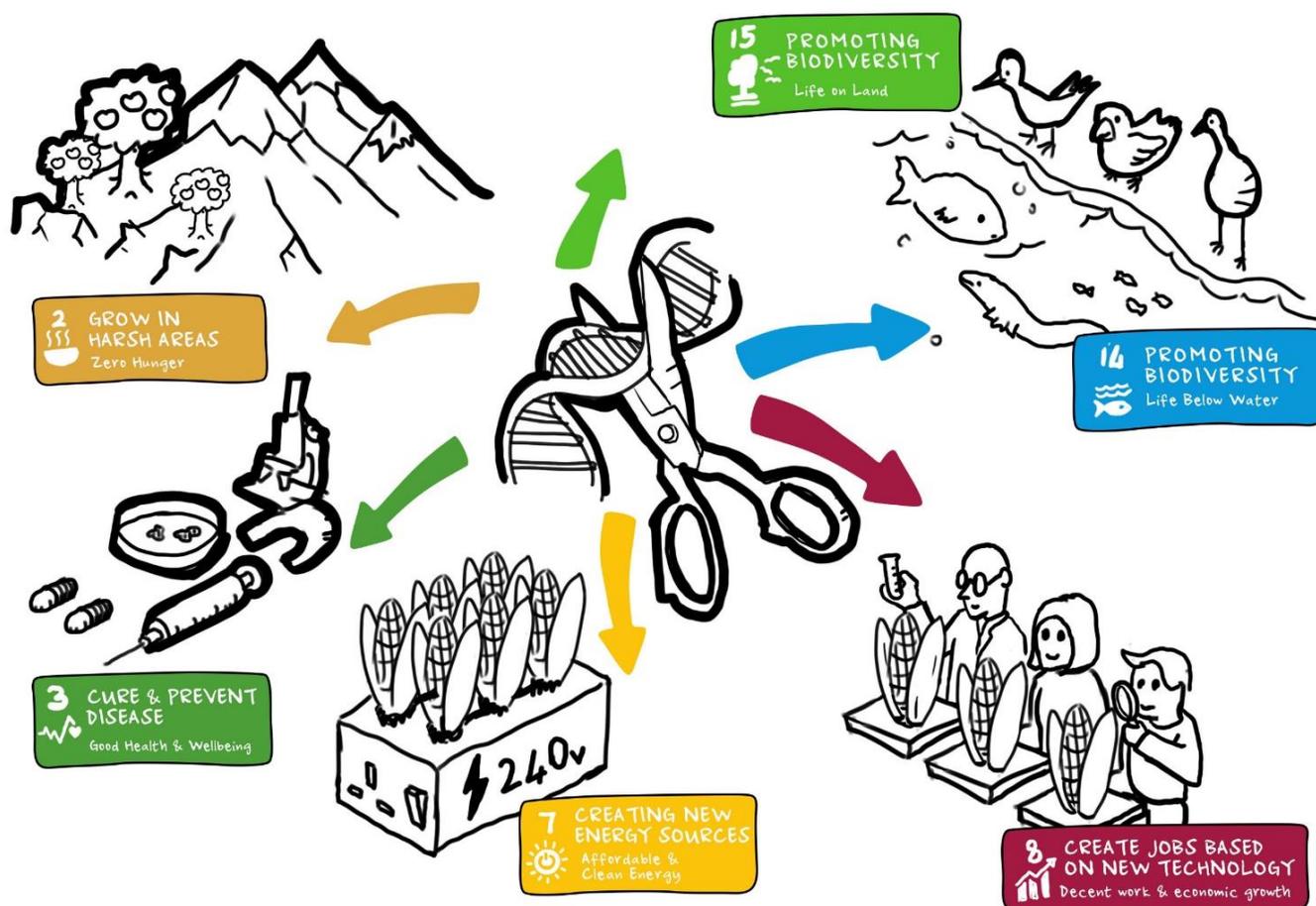
Source: Allied Market Research

\$1.3 bn+

Estimated start up financing in 2016

Source: CB Insights

Advancing the Sustainable Development Goals (SDGs)



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Gene editing has the potential to advance many of the SDGs. Below are some examples of areas of application across a wide variety of sectors.

π SDG 2 Zero hunger

- Improve the ability of crops to grow in resource-limited areas.
- Manage the stock and productivity of livestock in a humane and ethical manner.

π SDG 3 Good health and wellbeing

- Cure or prevent diseases rather than treat symptoms, which is the current focus of most modern medicine. This has the potential to fundamentally transform how care is delivered.
- Personalise care to patients. Within the wider movement towards providing genomic medicine, analysing an individuals' genetic make-up can identify if a patient will respond well to a drug treatment, and allow focused interventions which minimise unpleasant or harmful side-effects.
- Use of gene editing to fight diseases associated with aging.

- Bring in a new class of treatments for debilitating diseases.
- Enable new advances in drug development by modifying more complex organisms to produce new proteins that can be used as medicine (for example insulin).

π SDG 7 Affordable and clean energy

- Create new energy sources by enabling organisms to produce a biofuels such as bioethanol more efficiently. This will help reduce the reliance on non-sustainable or environmentally damaging energy sources such as fossil fuels.

π SDG 8 Decent work and economic growth

- Create new jobs through a range of new start ups and products/service businesses based on this technology. As with any new industry, most economies are starting from a similar place, so the developing world has a great opportunity to enter this industry at a similar point to many developed countries.

π π SDG 14/15 Life on land/below water

- Support biodiversity in both areas.

Technical Considerations

Since the technology is in its early stages, there are still a number of technical challenges to be overcome to make it safe and viable. Some of these are outlined below.

Accuracy

In order to ensure that a gene editing treatment is successful, it requires the correct editing of a very specific gene or set of genes. If the mutation misses the intended target, it could lead to cancer or other serious health problems. Although CRISPR has been developed in order to minimise these effects, even low levels of unintended change can have significant detrimental effects.

Delivery mechanism / immune response

The gene editing enzymes need to be delivered to the correct cellular location in the body whilst also avoiding immune responses and disruption to DNA. A number of techniques are being developed to do this but the human immune system makes it difficult for doctors and researchers to successfully deliver the gene editing enzyme to the proper location without being destroyed.

Scale up

Given the specificity of these potential treatments, off-the-shelf solutions may be less efficient. This means more effort will be needed to test each individual treatment option before it can be used. This creates significant hurdles for large scale production before widespread adoption is possible.

Enabling New Business Models

Gene editing can vastly increase efficiency as well as create new markets and opportunities.

Gene editing will have an impact across the healthcare value chain, from innovative approaches to the development of drugs and novel treatments, to re-evaluation of clinical trials, and disruption of the traditional prescription infrastructure to deliver personalised treatment options. It will also raise questions about where treatment is delivered - hospitals or outpatient facilities - and by whom - doctors or highly trained gene editors.

The ability to cure diseases rather than just treat symptoms will disrupt the business models of the healthcare and the pharmaceutical industry. A reduced need for long term treatment of diseases will lower demand for traditional drugs. The need to recoup the R&D costs of developing fast-acting cures will then have a lasting impact on commercialisation approaches, pricing strategies and how insurance companies reimburse treatment.

This potentially challenges the traditional structure of pharmaceutical companies. The ease-of-use and low-costs associated with treatment techniques as a result of gene editing could lower barriers to entry, and lead

to the rise of 'garage biotech' where amateur scientists develop innovative, complex potential therapies. There will also be new business opportunities for providers able to meet the need for cells with specific mutations and other adjacent services required for complex treatments using gene editing.

Gene editing will enable a number of the disruptive business model levers identified on the Project Breakthrough website, specifically:

A more personalised product or service

Gene editing will allow treatment to be personalised to individuals, based on their genetic make-up. This potential will have implications for how treatments are developed, approved and distributed. For example, since off-the-shelf solutions will not work, pharmacies as we know them will no longer be necessary unless they adapt to meet the need to deliver personalised treatment options.

Usage based pricing

The ability to cure or prevent rather than manage symptoms may make outcome based pricing a more attractive and effective mechanism for both providers and purchasers, rather than the current volume based models.

More Examples...

Using CRISPR Technology to modify crops

<https://www.pioneer.com/home/site/about/news-media/news-releases/template.CONTENT/guid.95ED9999-5719-1E79-8DA3-CE9A62C58CA2>

Gene Editing successfully used on girl with leukemia

<https://www.newscientist.com/article/dn28454-gene-editing-saves-life-of-girl-dying-from-leukaemia-in-world-first/>

CRISPR used to create oil producing yeast

<https://www.sciencedaily.com/releases/2016/01/160126162324.htm>



United Nations
Global Compact

The United Nations Global Compact is a call to companies everywhere to align their operations and strategies with ten universally accepted principles in the areas of human rights, labour, environment and anti-corruption, and to take action in support of UN goals and issues embodied in the Sustainable Development Goals. The UN Global Compact is a

leadership platform for the development, implementation and disclosure of responsible corporate practices. It is the largest corporate sustainability initiative in the world, with more than 9,000 companies and 3,000 non-business signatories globally.



Project Breakthrough

Project Breakthrough – a collaboration between UN Global Compact, Volans and partners – spotlights the best thinking in sustainable innovation. It showcases innovators across mainstream companies and next generation entrepreneurs who are developing solutions with the potential to achieve exponential impact. It features analysis and resources designed to help leaders understand the new business models and technologies that will be crucial in achieving the SDGs, catalysing action amongst today's businesses to meet the needs of tomorrow's world.



The Disruptive Technology Executive Briefs are produced in collaboration with PA Consulting Group, combining cross sector technology, innovation and business design expertise. The briefs are intended as an easy to digest introduction to disruptive technologies, to help organisations understand how they could advance the Sustainable Development Goals and business performance. These overviews explore key features, examples of applications, potential positive and negative impacts, and how they may enable the new business models.

Visit www.projectbreakthrough.io for more information, or contact projectbreakthrough@unglobalcompact.org