

Genome Editing - Wellcome Trust's perspective

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March 2016

Why does Wellcome care?

Our philosophy: Good health makes life better. We want to improve health for everyone by helping great ideas to thrive

As part of this philosophy we must be more than a science funder - we support full and inclusive exploration of scientific innovation, including the social context and public engagement.

Genome editing tools potentially contribute to that mission through research and therapeutic applications.

Wellcome's Position – the Statement



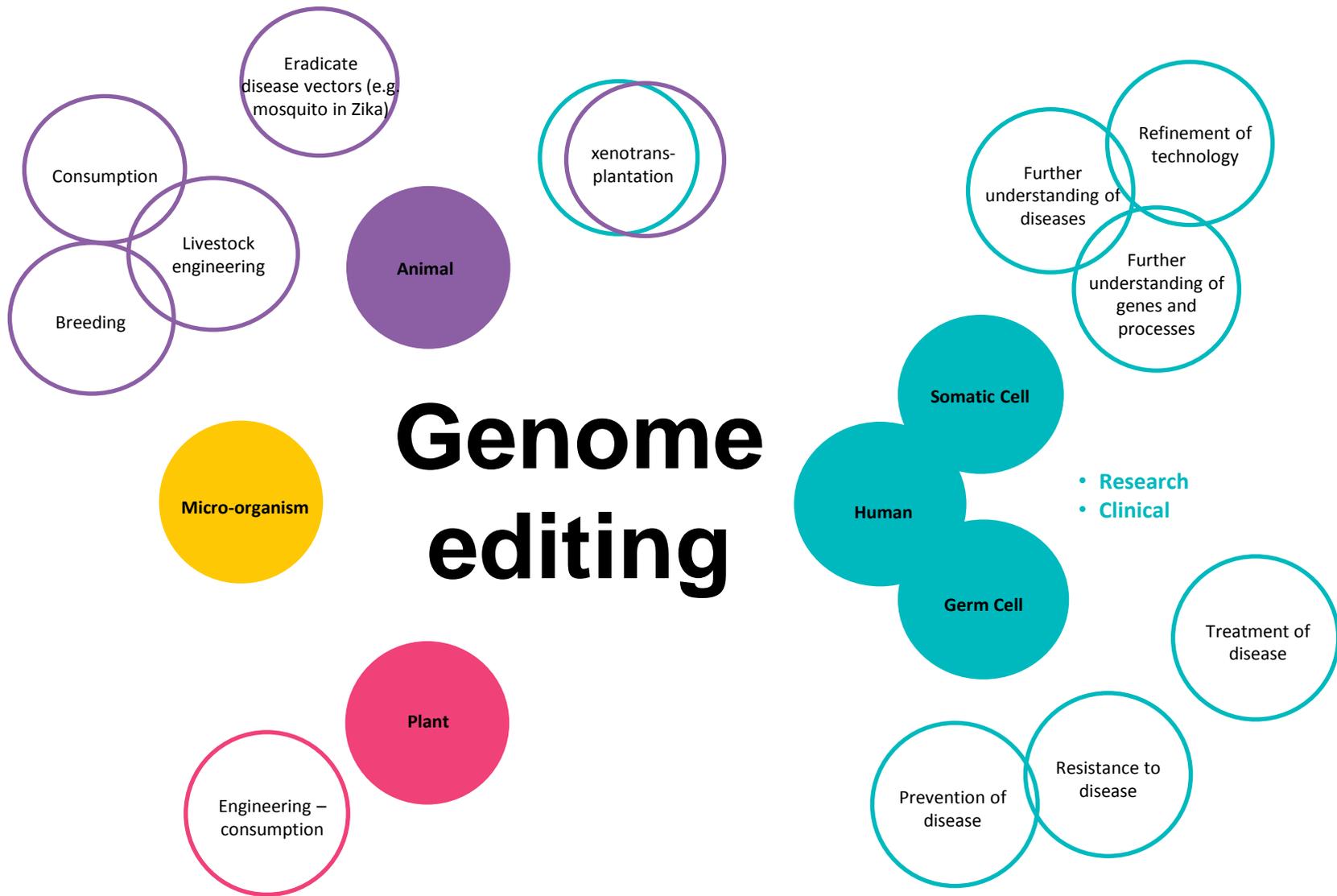
Genome editing in human cells – initial joint statement

Genome editing is a powerful technology that has the potential to improve health. It allows sections of DNA from a genome to be precisely replaced or removed using “molecular scissors”. The application of these tools is already having a game-changing effect on research intended to further our understanding of the roles of specific genes and processes in health and disease. In the future, these tools also hold the potential to be applied clinically to prevent or treat lethal and/or seriously debilitating genetic diseases.

The concept of genome editing is not new: for many years, scientists have applied a range of tools to manipulate genetic sequences. However, rapid technological developments in this area – namely the emergence of the CRISPR-Cas9 system – have meant that targeted, highly efficient editing of a genome sequence may become relatively simple. This has cast a spotlight on these technologies, and, in particular, the possibility that they could be applied to enable widespread editing of human cells for therapeutic benefit.

Sept 2015

- Genome editing has huge potential to improve health and we welcome this advance.
- Nothing should be automatically ruled in or out before it has been fully explored.
- Broader than science – need for an exploration of the ethical acceptability, social context, public perspective and oversight.



Gene drive

GM / GMOs

Dual use – bioweapon

Stem cells

Gene therapy

Mitochondrial donation

Why share an 'initial' position?

September 2015

The screenshot shows the Wellcome Trust website's navigation structure. At the top, there are tabs for 'Our philosophy', 'Funding', 'Managing a grant', 'Education resources', 'News', and 'Investments'. Below these are 'Publications', 'Working here', 'Policy', 'Strategy', 'Organisation', 'History', 'Timeline', 'Logo usage', and 'Contact us'. A sidebar on the left contains a 'Policy and position statements' section with sub-links for 'UK regulatory and legislative provisions', 'Germ cells and human embryos', 'Data sharing', 'Human Fertilisation and Embryology Act', 'Influence', and 'Open access'.

This screenshot shows a joint statement from several organizations: The Academy of Medical Sciences, amrc, CANCER RESEARCH UK, BBSRC, MRC, Progress Educational Trust, wellcome trust, and wellcome trust sanger institute. The title is 'Genome editing in human cells – initial joint statement'. The text explains that genome editing is a powerful technology that can improve health by precisely replacing or removing DNA segments. It notes that while the application of these tools is already having a game-changing effect on research, their clinical use to prevent or treat lethal and/or seriously debilitating genetic diseases is still a future possibility. The statement also mentions that the concept of genome editing is not new, but recent technological developments, particularly the CRISPR-Cas9 system, have made targeted editing of a genome sequence relatively simple.

This screenshot shows a blog post from the Wellcome Trust website. The title is 'What is genome editing and how does it work?'. The post is dated 10 SEP, 2015 and is written by Kate Ashleson Gray. The main image is a 3D rendering of a DNA double helix. The right sidebar features a search bar and a 'RECENT POSTS' section with links to articles such as 'How do people feel about companies accessing health data?', 'Digital Phenotypes – Health research in the digital age', 'Wellcome Trust Research Round-Up: 07-04-16', 'Image of the Week: Zika', 'Cim Brain Prize awarded to three British neuroscientists', and 'Public Engagement Events Listing - March 2016'.

- Proactively recognise the complexity of these technologies and start discussions
- Provide background and information to ensure that these discussions are constructive and everyone can contribute.
- We don't just want to hear extreme positions – we want to hear interim positions and areas of uncertainty.
- Manage expectations and ensure realism is brought to the discussion.

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Why share an 'initial' position II?

September 2015

The screenshot shows the Wellcome Trust website's navigation and sidebar. The main navigation includes: Our philosophy, Funding, Managing a grant, Education resources, News, Investments, Publications, Working here, Policy, Strategy, Organisation, History, Timeline, Logo usage, Contact us, and Get involved. The sidebar menu includes: Policy and position statements, Consultation responses, and Spotlight issues (Data sharing, Human Fertilisation and Embryology Act, Influence, Open access).

The screenshot shows the title page of a joint statement. Logos at the top include The Academy of Medical Sciences, amrc, CANCER RESEARCH UK, BBSRC (Innovation for the future), MRC (Medical Research Council), Progress Educational Trust, wellcome trust, and sanger institute. The title is "Genome editing in human cells – initial joint statement". The text states: "Genome editing is a powerful technology that has the potential to improve health. It allows sections of DNA from a genome to be precisely replaced or removed using 'molecular scissors'. The application of these tools is already having a game-changing effect on research intended to further our understanding of the roles of specific genes and processes in health and disease. In the future, these tools also hold the potential to be applied clinically to prevent or treat lethal and/or seriously debilitating genetic diseases." It also mentions that the concept is not new and that CRISPR-Cas9 systems have made it simpler.

The screenshot shows a blog post on the Wellcome Trust website. The title is "What is genome editing and how does it work?". The author is Kahn Allison Gray. The post date is 10 SEP, 2015. The topic tags are CRISPR, gene editing, Genome, Genome editing. The image shows a 3D model of a DNA double helix. The sidebar includes a search bar and a "RECENT POSTS" section with links to articles like "How do people feel about companies accessing health data?", "Digital Phenotypes – Health research in the digital age", "Wellcome Trust Research Round-Up: 07.04.16", "Image of the Week: Zika", "Cain Brain Prize awarded to three British neuroscientists", and "Public Engagement Events Listing - March 2016".

- Support open discussion and support make sure that the debate doesn't vilify scientists - concerned about the impact of an increasingly wide-ranging and vague moratorium.
 - Starting with a ban frames the discussion in unhelpful assumptions and could stifle discussion.
 - We welcome measures encouraging openness and transparency and initiatives that encourage a progressive environment.

What is Wellcome's position?

Germ Cell

Support the use of genome editing in **research**. This includes using **somatic cells** and using **germ cells / embryos** up to 14 days

Somatic Cell

Support the **therapeutic potential** to use genome editing to edit **germ cells** or **embryos**

Support genome editing **clinically** when editing **somatic cells**

This reflects the UK legislative and regulatory framework –

Framework supported and facilitated by robust governance.

Research

Clinical

Where now?

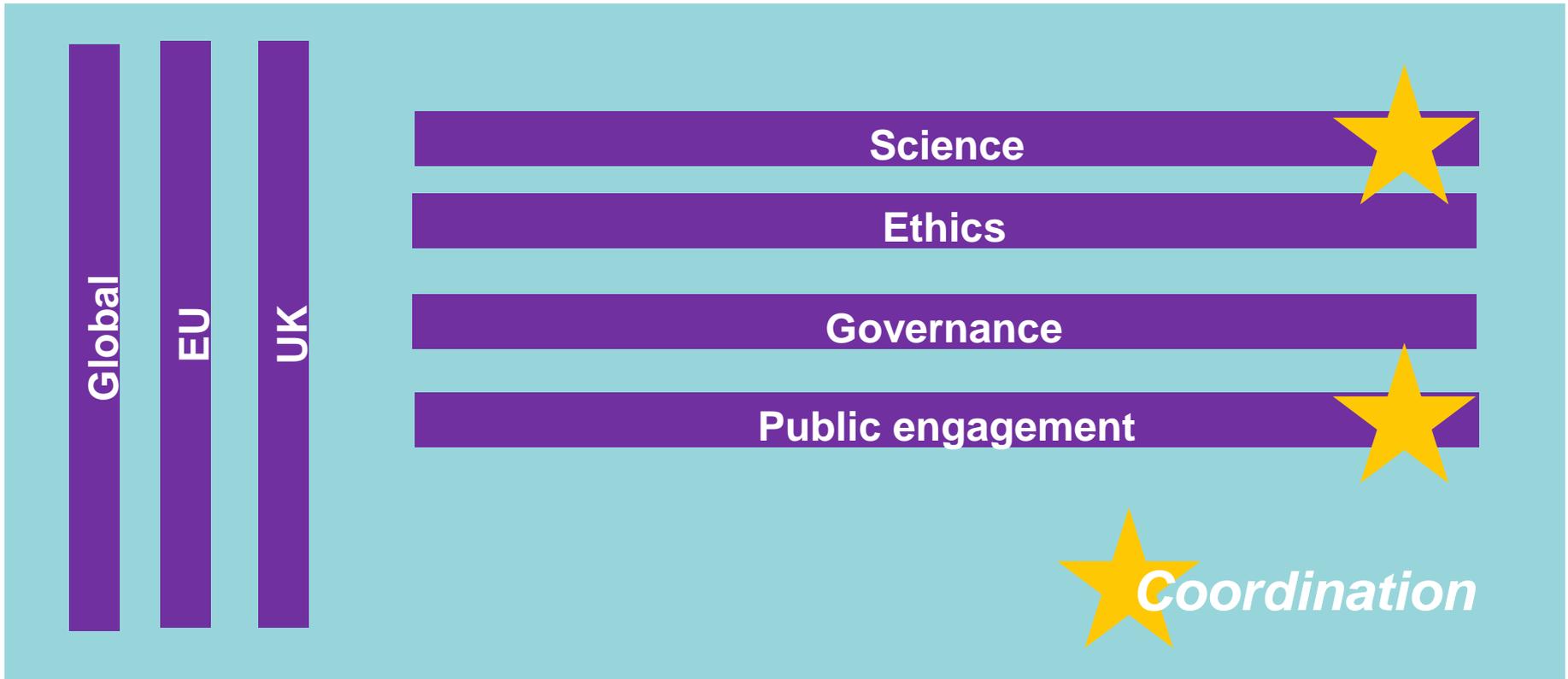
Advance ideas by funding applications to further the science; explore the ethics and engage with society are all being considered through our schemes.

Seize opportunities to bring together different communities to explore genome editing. We have funded a number of initiatives. For example:

- Hinxton Group – Statement on Gene Editing (Sept 2105)
- US, UK and Chinese National Academies (International Summit Dec 2105 & working group (ongoing))

Meet and discuss with colleagues in research and industry, in the UK, Europe and internationally and act on opportunities to accelerate change.

Where next? (not just Wellcome!)



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Thank you