Genome Editing - Wellcome Trust's perspective

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wellcome trust

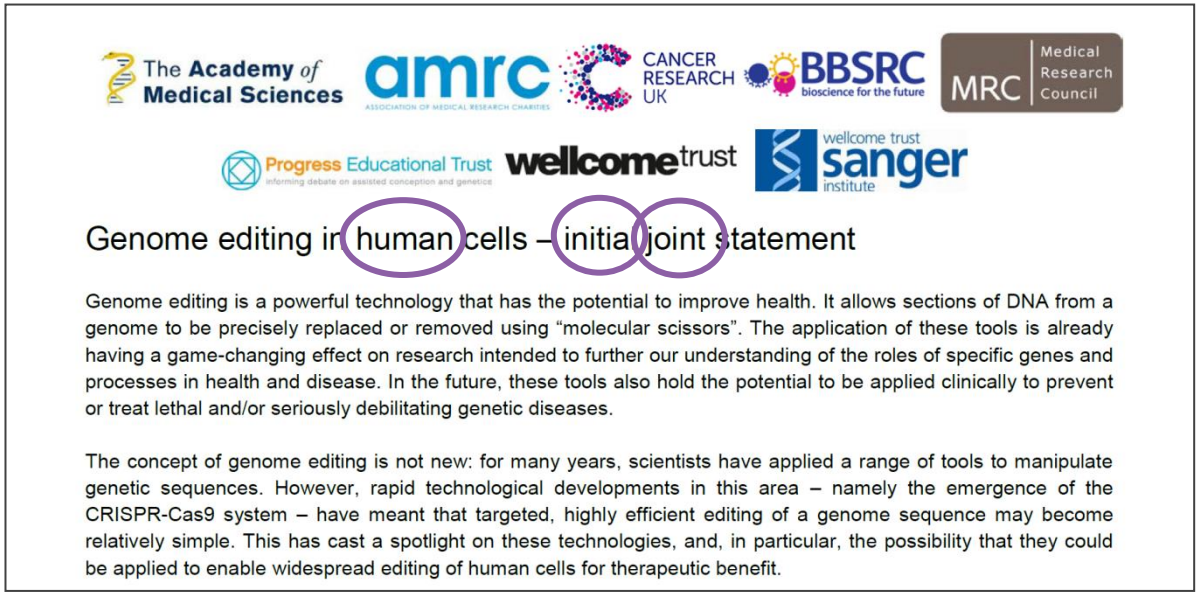
Why does Wellcome care?

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



The Academy of Medical Sciences · amrc · Cancer Research UK · BBSRC bioscience for the future · MRC Medical Research Council · Progress Educational Trust · wellcome trust · wellcome trust sanger institute

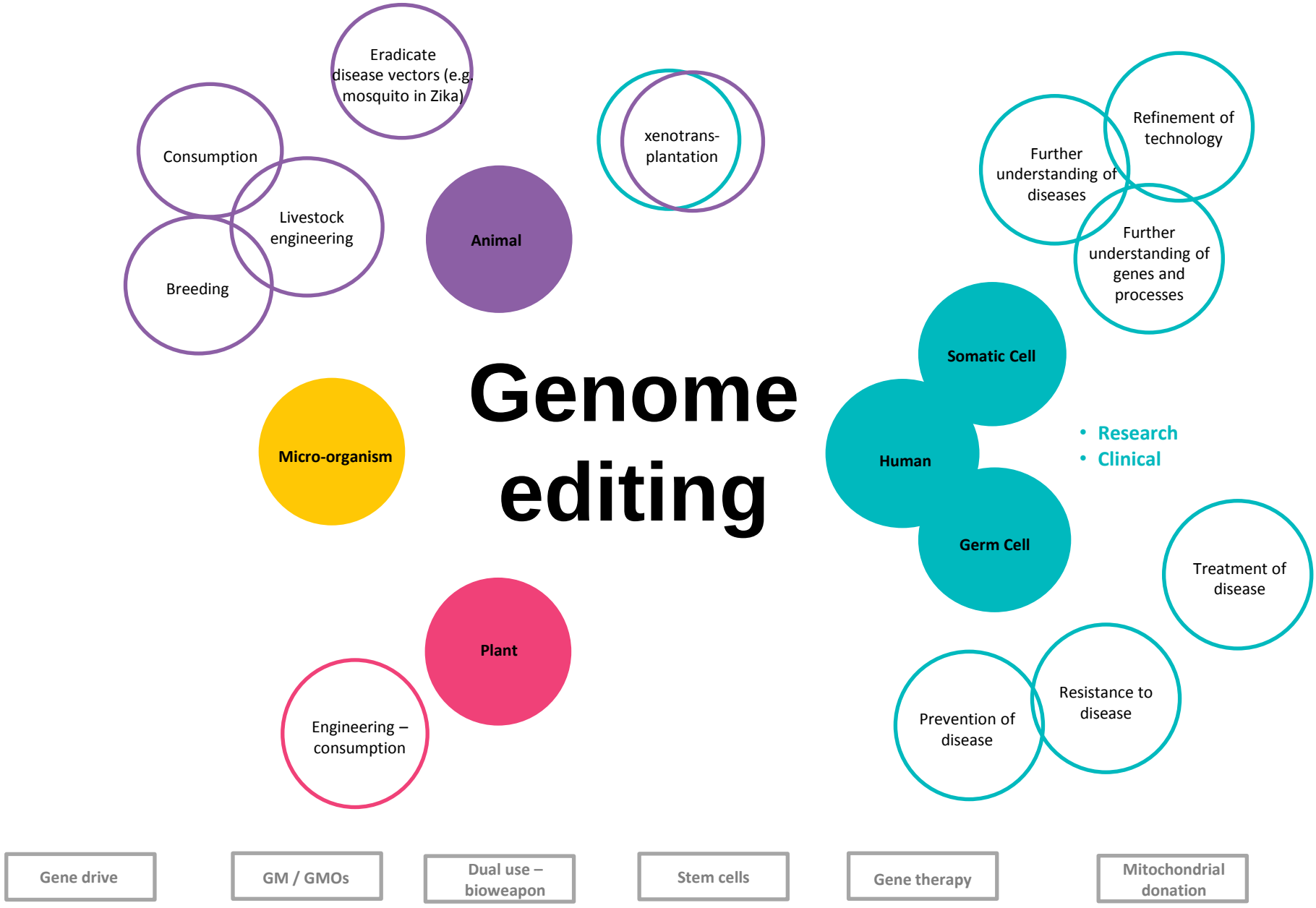
Genome editing in human cells – initial joint statement

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.

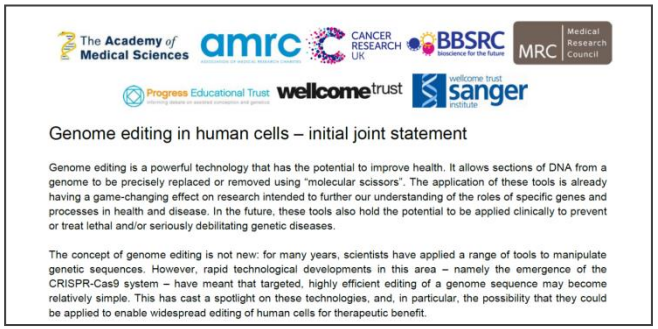
Sept 2015

- 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.


Eradicate disease vectors (e.g. mosquito in Zika)
Consumption
Livestock engineering
Breeding
Animal
xenotrans-plantation
Refinement of technology
Further understanding of diseases
Further understanding of genes and processes
Somatic Cell
Human
Germ Cell
• Research
• Clinical
Treatment of disease
Resistance to disease
Prevention of disease
Micro-organism
Plant
Engineering – consumption
Genome editing
Gene drive
GM / GMOs
Dual use – bioweapon
Stem cells
Gene therapy
Mitochondrial donation

Why share an ‘initial’ position?

September 2015



- 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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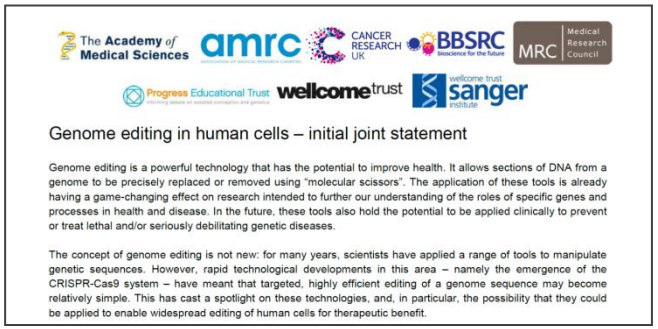
Why share an 'initial' position II?

September 2015



- 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

Somatic Cell

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

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?

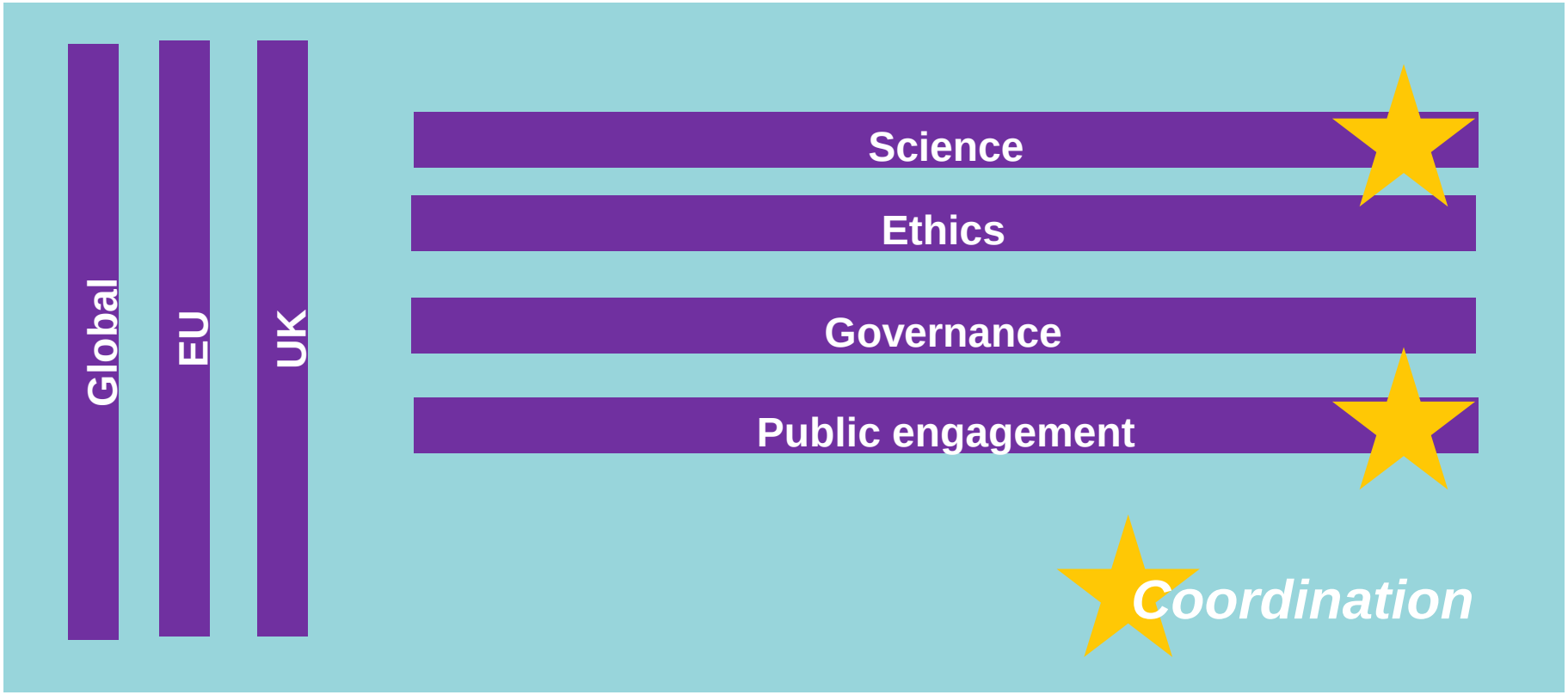
Advance ideas by funding applications to further the science; explore the ethics and engage with society are all being considered through our schemes.

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