

Nuffield Council on Bioethics: Genome Editing

Response by the Wellcome Trust

February 2016

Key points

- Genome editing is currently a valuable research technique which is used to further our understanding of genes and biological processes underpinning health and disease. In the future it is possible that this technique could be used in the treatment and prevention of disease.
- There is a need to explore the scientific and clinical potential of genome editing in a timely and inclusive manner. This must be genuinely open-minded without presuming that anything should be ruled in or out.
- The ethical questions raised by genome editing should be considered on a case-by-case basis, depending on the genes being modified and the context in which this takes place.
- Like many scientific innovations and emerging technologies, there is a need to engage with society about genome editing, and that requires openness about how and why the techniques are, and could be, used.
- Robust governance and oversight mechanisms of emerging technologies are essential. These must be proportionate and flexible to keep pace with fast-moving scientific developments. In the UK, this is assured through established legislation and independent regulation, which is grounded in social, ethical and public opinion.

Introduction

1. As an organisation dedicated to improving human health, we believe that innovations that have the potential to overcome disease are to be welcomed and warrant detailed consideration. We therefore welcome the Nuffield Council of Bioethics' timely focus on genome editing. Given our core interests, our response focuses on the editing of human cells, but we recognise that many of the issues will be cross-cutting and the broad holistic view of the Nuffield Council of Bioethics' work in this space is welcome.
2. Our response outlines the Wellcome Trust's current initial position, which will continue to develop, and some of the key considerations and discussions necessary to inform this evolution. We are a signatory to the 'Joint Statement on Genome Editing in Human Cells'¹ and our perspective outlined below reflects and elaborates on this.

Current Wellcome Trust position

3. Genome editing techniques allow precise alteration of the genetic sequence in single genes or groups of genes. These techniques are becoming quicker, cheaper and easier to use than the alternative approaches, allowing a wider range of scientists to employ them in their work. Despite these remarkable advances, our understanding of genome editing technologies and their potential is still at a relatively early stage.
4. It is helpful to draw distinctions between research and clinical applications; however we recognise that developments in research could inform clinical use in future, for example, research to refine

¹ <http://www.wellcome.ac.uk/About-us/Policy/Spotlight-issues/Genome-editing/WTP059704.htm>

and improve the genome editing technique. However, research need not necessarily lead to clinical applications, and regulators and society will need to consider the two issues independently.

Research

5. The relative ease and efficiency of new genome editing technologies is making this technology far more accessible and increasingly a standard technique in research worldwide. Within the UK, this research may involve the use of somatic (non-reproductive) or germ cells, including human embryos up to 14 days old - within the confines of the HFE Act 2008 - where appropriately justified and supported by rigorous scientific and ethical review. Genome editing technologies are already having a game-changing effect, furthering our understanding of genes and processes underpinning health and disease, and giving new insight into fundamental questions of human biology.

Applications

6. Genome editing technologies hold significant potential for clinical application in the future. We would be open to supporting the development of new therapeutic approaches should the evidence from research advance sufficiently to justify their use, and it is ethically acceptable and on balance supported by society. It is important to emphasise that the science is still at a relatively early stage and potential therapeutic applications are not yet here. However, science is progressing apace and it is necessary to consider the potential future uses of genome editing technology.
7. In the first instance clinical advancements are likely to involve the editing of somatic cells with the aim of repairing or eradicating a mutation that could cause disease, or to engineer beneficial changes to allow cell therapy. An example of this is cancer, where an individual's immune cells might be modified to target cancerous cells.
8. There may be future potential to apply genome editing in a clinical context using germ cells or embryos, although this is currently prohibited in many jurisdictions including the UK. Although we have previously expressed our support for a medical innovation which will introduce germ-line modifications – mitochondria donation – these techniques only replace, rather than alter, a small number of unhealthy genes in the “battery pack” of the cells with healthy ones, and do not introduce heritable changes that alter personal characteristics and traits. We recognise that there are therefore important differences between mitochondria donation and the future potential applications of genome editing, which we discuss further in paragraphs 12-14.

Key areas for consideration

9. Genome editing technologies and their future potential use give rise to important questions, which need to be anticipated and explored in their national and international contexts, in a timely and inclusive manner. It is of paramount importance that scientific, ethical and public consideration of genome editing progress in parallel in order to ensure that all facets of the discussion keep pace with scientific development.
10. It is important that as these discussions progress genome editing is not considered as simply an ‘acceptable’ or ‘unacceptable’ technology; rather there must be a case-by-case approach according to the context. It is necessary to clearly delineate the different ways and contexts in which genome editing might be used, clearly distinguishing the use of this technology in a research context compared with its potential application in a clinical setting, as well as distinguishing the use of these technologies using somatic or germ cells.
11. There have been calls for a moratorium on the use of genome editing as these discussions progress, but we are concerned that framing discussions in this way is not a helpful starting point for the following reasons: we need transparency and would be concerned about any rhetoric that

drives research underground; there is a need for a progressive environment around these techniques; and starting with a ban frames the discussion in unhelpful assumptions and could stifle discussion. In jurisdictions where there are clear regulations, such as the UK, there is no need for a moratorium on editing germ cells in a clinical context as it is already not permissible. In jurisdictions where there isn't clear legislation or regulation, a moratorium would have little effect. We reason therefore that broad bans could have a negative impact and limit our ability to explore these emerging technologies and their potential.

Ethics

12. In considering the ethical acceptability of genome editing, it is important to recognise that the concept of genome editing is not new: for many years, scientists have applied a range of tools to manipulate genetic sequences. This is not therefore the first time we have deliberated the ethics of altering the genome. Recent discussions, for example around mitochondria donation, gene therapy, cloning and genetic screening are all pertinent here and should be drawn on. However, even if many of the ethical questions remain the same, the implications and broader contexts in which the current discussions are taking place will be different and therefore the same ethical questions must be explored as part of this discussion.
13. Whilst there are parallels with previous discussions, there are also differences arising as the speed, ease of use and accuracy of the new genome editing techniques makes it feasible to use them for a wider range of applications. As such, there are also likely to be new ethical questions arising and there is a need for a constant and continual dialogue about where emerging technologies are taking us.
14. It has been suggested that some outcomes of genome editing, such as the introduction of heritable changes that alter personal characteristics, should be automatically ruled out as ethically unacceptable. While we are not necessarily supportive of such applications, we also do not think potential future applications of genome editing should be automatically ruled out before being fully explored. Rather, it is necessary to be genuinely open-minded, welcoming public discussion and deliberation.

Societal engagement

15. Like many scientific innovations and emerging technologies, there is a need to engage with society about genome editing, and that requires openness about how and why the techniques are, and could be, used. It is important to consider a range of approaches according to the purpose of public engagement. For example, engagement may be in order to raise awareness, heighten understanding, or seek public input into the direction of research or the application of technologies and treatments.
16. Responsibility to engage with the public about scientific advancements does not uniquely fall to genome scientists; all scientists, the wider research community and Government, have a responsibility to engage the public in an inclusive and discursive dialogue about where emerging technologies are taking us.

Oversight

17. As with all emerging technologies, robust governance and oversight mechanisms are essential. There are rightly different approaches to achieving this, but in all cases the oversight mechanisms must be proportionate and flexible to keep pace with fast-moving scientific developments. In the UK, this is ensured through separation of legislation and regulation: underlying principles are established by Parliament in legislation, and then responsibility for on-going regulation falls to the expert and independent body – in this case the Human Fertilisation and Embryology Authority when using human gametes or embryos, and the Human Tissue Authority when using other human tissue.

18. We would be pleased to discuss any of the points raised here in more detail.