

## 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.

It is important to emphasise that the science is still at a relatively early stage and potential therapeutic applications are not yet here. It is also important to clearly delineate the different ways and contexts in which this technology 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 (non-reproductive) or germ (reproductive) cells.

Research using genome editing tools holds the potential to significantly progress our understanding of many key processes in biology, health and disease and for this reason we believe that responsibly conducted research of this type, which is scientifically and ethically rigorous and in line with current legal and regulatory frameworks, should be allowed to proceed. We will continue to support the use of genome editing in preclinical biomedical research as well as studies that progress and refine these technologies. 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.

We believe that genome editing technologies may hold significant potential for clinical application in the future; and we would be open to supporting the development of new therapeutic approaches should the evidence from research advance sufficiently to justify their use. Primarily these advancements have involved the editing of human somatic cells with the aim of repairing or eradicating a mutation that could cause disease or to engineer beneficial changes to allow cell therapy, for example in cancer, where an individual's immune cells might be modified to target cancerous cells.

We also recognise, however, that there may be future potential to apply genome editing in a clinical context using human germ cells or embryos, though this is prohibited by law in the UK and unlikely to be permissible in other European jurisdictions at present. This raises important ethical and regulatory questions, which need to be anticipated and explored in a timely and inclusive manner as the basic research proceeds and prior to any decisions about clinical application. Active early engagement with a wide range of global stakeholders will therefore be needed, which should include, but not be limited to, biomedical and social scientists, ethicists, healthcare professionals, research funders, regulators, affected patients and their families, and the wider public. Many of these considerations will not be unique to this specific technology, but will apply across a number of future developments in science for the benefit of health.

Frameworks clearly demarcating research use of genome editing from potential clinical use, and carefully distinguishing use of somatic and germ cells, will ensure that the research community remains at the forefront of this novel area, while exploring the complex issues around different clinical applications in a robust and inclusive manner.

The signatories are committed to supporting discussion around genome-editing and its implications. We are progressing this through a number of activities and we will continue to develop and refine our position, informed by the outcomes from such activities.