Human genome-editing research should proceed, say leading UK science bodies

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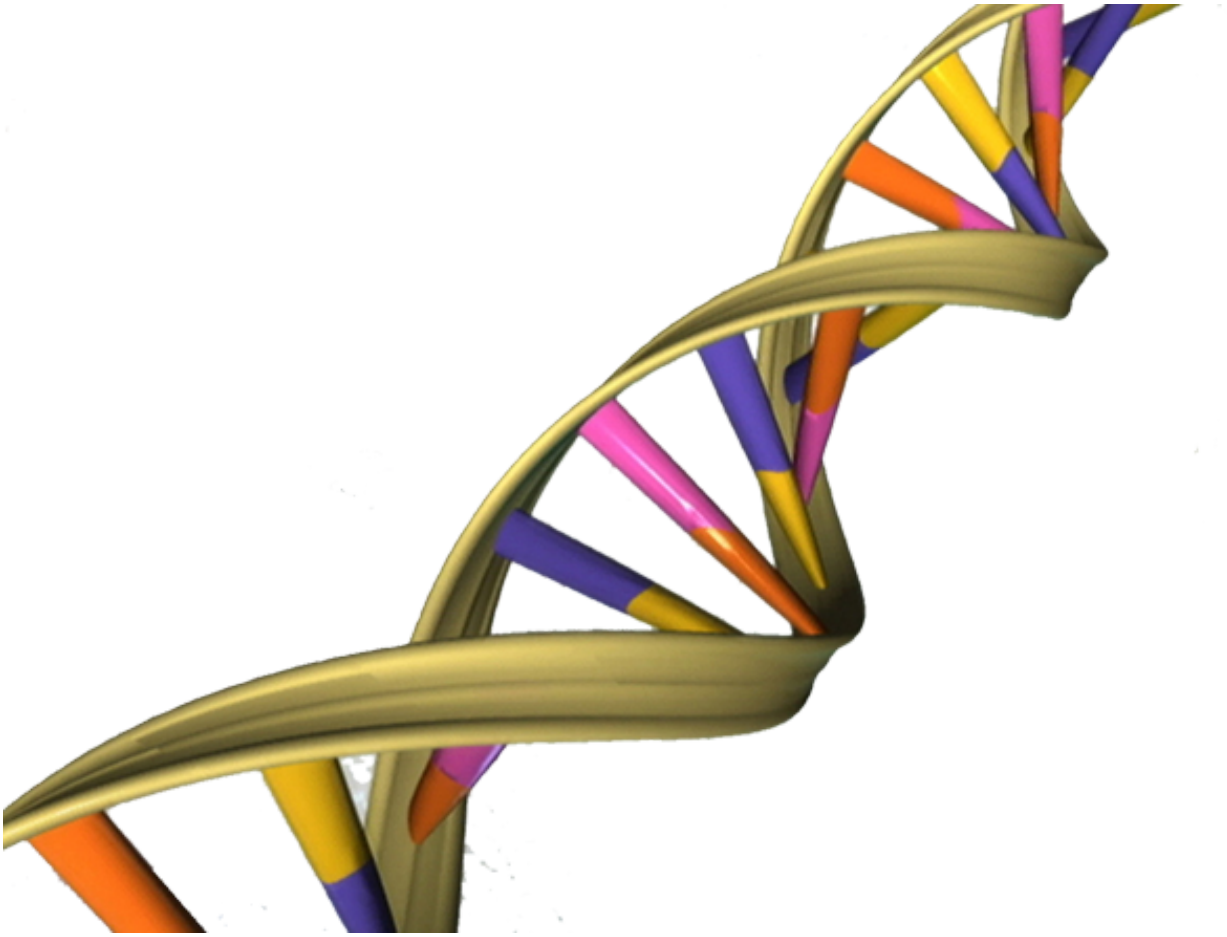

A depiction of the double helical structure of DNA. Its four coding units (A, T, C, G) are color-coded in pink, orange, purple and yellow. Credit: NHGRI

A group of leading UK research organisations has today issued an initial joint statement in support of the continued use of CRISPR-Cas9 and other genome-editing techniques in preclinical research. This includes the use of the technology for research purposes in human reproductive cells and early embryos, where this is fully justified, scientifically and ethically, and within the confines of the law.

The coalition of research funders and learned societies will continue to fund and support research of this kind, as well as studies that further progress and refine these technologies. The group has also called for widespread discussion among scientists, ethicists and the wider public about how these emerging techniques may in future be applied clinically, in human reproductive cells and early embryos, to treat or prevent serious genetic disease.

Genome editing is a powerful research tool that allows sections of DNA in the genome to be precisely removed or replaced using 'molecular scissors'. The concept of genome editing is not new, but rapid developments in the technology – namely the emergence of the CRISPR-Cas9 system – mean that targeted, highly efficient editing of DNA in cells has become relatively simple.

Our understanding of CRISPR-Cas9 is still at an early stage, and any potential treatments based on this technique are many years away. The first clinical applications of CRISPR-Cas9 are likely to involve editing the DNA in somatic (non-reproductive) cells – for example, by removing them from a patient and editing them in a lab to correct a harmful mutation before putting them back into the body. There is already some work in progress to do just this in diseases including HIV, sickle-cell disease and haemophilia, and emerging potential for cancer therapy.

There has also been some suggestion that genome editing could be used clinically in the future to edit the DNA of human eggs and sperm, or

early embryos (known as germline editing). The difference here is that any changes made would be passed on to future generations. This is prohibited under UK law and is unlikely to be allowed in any European jurisdiction at present.

The UK Human Fertilisation and Embryology Act does permit the use of new technologies such as gene editing for non-clinical research purposes in germ cells, including human embryos up to 14 days old, where this is appropriately justified and supported by rigorous scientific and ethical review.

Today's statement – signed by the Academy of Medical Science (AMS), the Association of Medical Research Charities (AMRC), the Biotechnology and Biological Sciences Research Council (BBSRC), the Medical Research Council (MRC) and the Wellcome Trust – says that this type of research to be allowed to continue and calls for a broad and inclusive discussion about genome editing and its future implications.

Professor Sir John Tooke PMedSci, President of the AMS, said: "Genome editing is an evolving technology that has been used in the lab for many years to increase our understanding of disease mechanisms, and may provide the potential means to develop new medical treatments in the future. We believe such therapeutic use should be explored, but several scientific and ethical questions remain to be answered and much more evidence will be needed before an informed decision can be made about a particular application."

Aisling Burnand, Chief Executive of the AMRC, said: "Although currently at an early stage, this technology has the potential, in future decades, to help people with devastating diseases. Patients and their families will clearly want to be involved in discussions to consider the feasibility, safety and desirability of clinical applications as the research matures."

Professor Melanie Welham, BBSRC's Science Director, said: "Genome editing in a preclinical research setting can widen our understanding of basic biology, which is vital to underpin advances in prolonging healthy life and disease management."

Dr Rob Buckle, Director of Science Programmes at the MRC, said: "The UK's place as a global leader for research is underpinned by a strong regulatory framework. As genome editing technologies evolve it's vital that the regulatory framework remains robust and adapts so that the full potential of genome editing can be realised in a scientifically, ethical and legally rigorous way."

Katherine Littler, Senior Policy Advisor at the Wellcome Trust, said: "As with any emerging technology, the potential for genome editing to be applied as a therapeutic tool in future deserves careful consideration. It's essential that we start these discussions early, by engaging in an open and inclusive debate involving scientists, ethicists, doctors, regulators, patients and their families, and the wider public."

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