

Statement from the Organising Committee of the Third International Summit on Human Genome Editing

The Third International Summit on Human Genome Editing, convened by the UK Royal Society, UK Academy of Medical Sciences, US National Academies of Sciences and Medicine, and The World Academy of Sciences, was held to discuss progress, promise, and challenges in research, regulation, and equitable development of human genome editing technologies and therapies.

After listening to three days of thoughtful and inclusive discussion, the members of the [Organising Committee](#) offer the following conclusions:

Remarkable progress has been made in somatic human genome editing, demonstrating it can cure once incurable diseases. To realise its full therapeutic potential, research is needed to expand the range of diseases it can treat, and to better understand risks and unintended effects. The extremely high costs of current somatic gene therapies are unsustainable. A global commitment to affordable, equitable access to these treatments is urgently needed.

Heritable human genome editing remains unacceptable at this time. Public discussions and policy debates continue and are important for resolving whether this technology should be used. Governance frameworks and ethical principles for the responsible use of heritable human genome editing are not in place. Necessary safety and efficacy standards have not been met.

Governance mechanisms for human genome editing need to protect ongoing, legitimate research, while preventing clinics or individuals from offering unproven interventions in the guise of therapies or ways to avoid disease.

Somatic human genome editing¹

Numerous clinical trials using somatic human genome editing are in progress or soon to be initiated, with preliminary but encouraging results that point to future therapies. The dramatic improvement following CRISPR-based research interventions for sickle cell disease offers hope for patients. Many techniques, including base, prime, and epigenetic editing, may also prove to be useful interventions for a broad range of both genetic and acquired diseases and disorders. However, as with other gene therapies, extended long-term follow-up is essential to fully understand the consequences of an edit and to identify any unanticipated effects, should they occur.

Improved techniques have enhanced the efficiency, precision, and accuracy of the editing process, yet effective delivery and editing remains difficult for many tissues of the body. Further research to diversify and increase the efficiency, specificity, and safety of editing-delivery systems is essential for improving potential treatment options and promoting equitable access.

Equitable access for somatic human genome editing

As interventions based on somatic genome editing become more widespread, a commitment to equitable, financially sustainable, and accessible treatments becomes more urgent. In many cases, costs and infrastructure needs of current gene therapy treatments are not manageable for either patients or healthcare systems. Correcting this will require appropriate planning from the earliest stages of the research and development for each potential application. Ensuring research includes

¹ Somatic human genome editing refers to the editing of somatic cells, which are non-reproductive cells, and changes made in these cells affect only the person who receives the genome editing.

more genetically diverse populations and expanding the range of those who conceive and conduct the research, play a vital role in achieving equitable outcomes.

With sickle cell disease (as well as other genetic diseases), a large percentage of patients live in underserved countries and communities or in settings without adequate infrastructure. Moving from ex-vivo to “one and done” in-vivo somatic human genome editing can partially address this problem. But knowledge transfer between nations, improved clinics and research facilities, and strong oversight are also needed to establish sustainable access to safe interventions for research participants and patients.

Health care systems and the global health community should prepare to provide patients with cost-effective, affordable, proven therapies. Therapies based on somatic genome editing that could help meet these needs should be a priority for research investment.

Human germline genome editing for research (not for reproduction)²

Basic research using genome editing in human embryos has continued, with the aim of either understanding aspects of early human development or exploring how the methods might be used to correct gene variants leading to genetic disorders. There has also been significant progress in basic research on deriving functional gametes from stem cells. Basic research in this field should continue.

Heritable human genome editing

Preclinical evidence for the safety and efficacy of heritable human genome editing has not been established, nor has societal discussion and policy debate been concluded. (In some cases, preimplantation genetic testing is among the alternatives.) Heritable human genome editing should not be used unless, at a minimum, it meets reasonable standards for safety and efficacy, is legally sanctioned, and has been developed and tested under a system of rigorous oversight that is subject to responsible governance. At this time, these conditions have not been met.

Ongoing international collaboration and discussions

The Organising Committee calls for on-going dialogue and continued international collaboration on innovative approaches to governance and regulation of human genome editing technologies, the state of the science, and innovation in the treatment of genetic diseases.

² In this statement, germline human genome editing refers to the editing of human embryos or gametes in a research setting, with no plans for those embryos or gametes to be used for human reproduction. Heritable human genome editing refers to the editing of human embryos or gametes to be used for human reproduction.