

## **Human Genome Editing: Science, Ethics, and Governance (NASEM Report)**

Examines and provides recommendations on the scientific, ethical, and social issues raised by human genome editing and assesses the capacity of governance systems to ensure the responsible development and use of this technology.

Updated last **December 1, 2017**  
for the 02/14/2017 report.

### **WHAT IT DOES**

[Human Genome Editing: Science, Ethics, and Governance](#) is the resulting report of a consensus study carried out by the Committee on Human Gene Editing: Scientific, Medical, and Ethical Considerations, convened by the National Academy of Sciences (NAS) and the National Academy of Medicine (NAM). The task of the committee in writing the report was to inform the international community, with additional focus on the United States, on important questions about the human applications of [genome editing](#) technology.

The authoring committee proposed the following seven principles for the governance of human genome editing:

1. Promoting well-being, which means fostering beneficial aspects while preventing unnecessary risks for the recipients of these interventions, as in the bioethical principles of beneficence and non-maleficence;
2. Transparency, which means committing to sharing information broadly in the scientific community and engaging the public in the discourse of policy-making;
3. Due care, which means proceeding prudently at each advancement in technology and its translational applications to ensure proper oversight;
4. Responsible science, which means maintaining the highest standards of scientific rigor and integrity in the conduct of research and presentation of findings;
5. Respect for persons, which means valuing all persons equally and respecting autonomy of decision-making;
6. Fairness, which means striving towards achieving distributive justice of the burdens and benefits of research and equitable access to its applications; and
7. Transnational cooperation, which means organizing a concerted approach to regulation while valuing cultural complexity and differences in national policy-making processes.

These principles were intentionally developed to be broadly applicable for international adoption by national regulatory agencies and the scientific community alike.

Additional recommendations issued by the report considered the following three aspects: (1) whether existing oversight mechanisms provide the capacity for appropriate regulatory action; (2) whether the applications are directed towards editing [somatic cells](#) or [germ cells](#) (i.e., eggs and sperm); and (3) whether the intention of the intervention is to treat or prevent a disease or for enhancement purposes.

Based on these three aspects, the recommendations offered were divided into the following categories:

1. In regard to basic laboratory (i.e., non-clinical) research, the main recommendation was to use the current oversight infrastructure to regulate basic laboratory research involving human genome editing. Other recommendations depend on whether the research uses somatic or germ cells.
2. In regard to clinical uses of somatic cell genome editing, whose purpose is curing or treating diseases, the main recommendation was also to use the existing oversight infrastructure, as long as the following conditions were met:
  1. Clinical trials or therapies are limited to treatment or prevention of disease or disability;
  2. Safety and efficacy should be evaluated with consideration of the risks and benefits of intended use; and

3. Significant public discourse should inform any further uses.
3. In regard to heritable genome editing, the report recommended that clinical trials may receive authorization, so long as they are only for compelling purposes (see below) and are subject to a robust and effective regulatory framework. Specifically, the following conditions were proposed:
  1. absence of reasonable alternatives;
  2. restriction to preventing a serious disease or condition;
  3. restriction to editing genes that have been convincingly demonstrated to cause or to strongly predispose to that disease or condition;
  4. restriction to converting such genes to versions that are prevalent in the population and are known to be associated with ordinary health with little or no evidence of adverse effects;
  5. that credible preclinical and/or clinical data on risks and potential health benefits of the procedures are available;
  6. ongoing, rigorous oversight during clinical trials of the effects of the procedure on the health and safety of research participants;
  7. comprehensive plans for long-term, multigenerational follow-up that still respect personal autonomy;
  8. maximum transparency consistent with patient privacy;
  9. continued reassessment of both health and societal benefits and risks, with broad ongoing participation and input by the public; and
  10. reliable oversight mechanisms to prevent extension to uses other than preventing a serious disease or condition.
4. In regard to genome editing for purposes other than treatment or prevention of disease (enhancement), the report recommended that such applications should not yet receive authorization and that, first, significant public discourse and policy debate should inform the potential extension of human genome editing for these purposes.

The development of [CRISPR/Cas9](#) ([SciPol brief available](#)) as a genome editing tool has generated significant expectations within scientific, commercial, and patient communities, as well as concerns by policymakers. However, the report emphasized the importance of meaningful public engagement to ensure transparency and legitimacy in the debates that surround this technology, especially with regard to interventions in germ cells that can be transmitted to future generations and in applications that go beyond the restoration of healthy functions (enhancement).

#### RELEVANT SCIENCE

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[Human genome editing](#) is a method of making precise changes to DNA in human cells. [Genome editing tools](#), such as meganucleases, zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs), and most notably the [CRISPR/Cas9 system](#), act as molecular scissors that can be programmed to cut out [DNA](#) from the human [genome](#), such as harmful [genes](#), or to insert DNA, such as novel genes. The scientific community has rapidly adopted CRISPR/Cas9 because it is a more versatile, precise, efficient, and less expensive genome editing tool. As a side note, the term “genome editing” is used instead of “gene editing” because tools like CRISPR/Cas9 can be used to edit other parts of the genome besides genes.

CRISPR (clustered regularly interspaced short palindromic repeats) is an antiviral component of the bacterial immune defense system. When a virus injects its DNA into a bacterial cell, the bacterial immune system responds by copying parts of the viral DNA and storing it in the bacterial genome, essentially cataloging information on the virus. CRISPR-associated (Cas) enzymes can then reference the viral DNA copy stored in the CRISPR system to identify and break down similar invading viral DNA in the future, and stop the virus before it establishes an infection.

The application of the CRISPR system to human genome editing involves scientists supplying a specific [RNA](#) sequence that guides the Cas9 enzyme to cut the DNA at a targeted sequence in the human genome. Scientists can then use this cut to remove DNA or insert a new DNA segment of their choosing. The remarkable power of such genome editing lies in the ability of scientists to select with great precision the stretch of DNA intended for editing. Many diseases and disabilities that afflict humans – such as cancer, cystic fibrosis, and muscular dystrophy – are caused by [genomic mutations](#). CRISPR/Cas9 therapies have great potential to ameliorate some of (and perhaps most of) these conditions through editing out the deleterious mutations.

CRISPR/Cas9 can potentially be used in any organism, including plants, animals and humans. However, this report focused on the human applications of this technology. Important ethical concerns are raised with each translation of CRISPR/Cas9 technology to therapeutics. However, especially salient concerns include the downstream effects of heritable genetic changes from germline (i.e., germ cell) editing of embryos and applications of human genome editing outside of the realm of treating disease or disability, often referred to as enhancement.

#### WHY IT MATTERS

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Recent advances in human genome editing technology have projected the field into an ethically uncharted realm of possibilities. It is important to track the emergence of revolutionary scientific developments, such as CRISPR/Cas9, and to review the capacity of current regulatory infrastructures at the national and international level to accommodate such innovations. The *Human Genome Editing* report delivers an impetus for continued international dialogue among relevant stakeholders – from policymakers and biomedical researchers to candidate patients and the general public – to construct a consensus on the applications and limitations of human genome editing technologies. Regulatory bodies, policymakers, and civil society would do well to keep abreast with news from the scientific community, as ethical questions regarding human genome editing rapidly shift from hypothetical to real-world.

#### BACKGROUND

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Though genome editing technology has been in the toolkit of molecular biologists for several decades, the emergence of the CRISPR/Cas9 system in 2012 advanced both the potency and scope of potential applications in humans. To address the lack of international dialogue on this issue, the NAS and NAM established the Human Gene-Editing Initiative and convened the International Summit on Human Gene Editing: A Global Discussion in conjunction with the Chinese Academy of Sciences and The Royal Society of the United Kingdom. The authoring committee of *Human Genome Editing* was then assembled to continue the discussions begun in the International Summit and provide guiding insights on the use of genome editing technology in humans.

#### ENDORSEMENTS & OPPOSITION

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Controversy and public debate surrounding the report is primarily associated with the committee's position on moving forward with germline editing within the aforementioned set of qualifying criteria.

Endorsements:

- Dr. Eric Lander, [Broad Institute of MIT and Harvard, quotation](#), February 14, 2017: "They have closed the door to the vast majority of germline applications and left it open for a very small, well-defined subset. That's not unreasonable in my opinion."

Opposition:

- Edward Lanphier, [Sangamo Therapeutics, quotation](#), February 14, 2017: The report "changes the tone to an affirmative position in the absence of the broad public debate this report calls for."
- [Center for Genetics and Society, press release](#), February 14, 2017: "We're very disappointed with the report. It's really a pretty dramatic shift from the existing and widespread agreement globally that human germline editing should be prohibited."

#### RELATED POLICIES

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[PL 114-113 \(SciPol brief available\)](#), titled "Consolidated Appropriations Act, 2017" and more commonly known as the federal budget, prohibits in [Section 749](#) the use of any federal funding "to notify a sponsor or otherwise acknowledge the receipt of a submission for an exemption for investigational use of a drug or a biological product... in research in which a human embryo is intentionally created or modified to include a heritable genetic modification." This provision stands in contrast to the recommendations made in the

report on the reasonability of conducting research that involves germline editing.

This provision was also included in the 2017 budget ([PL 115-31](#)) as [Section 736](#).

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**ORGANIZATIONS**

This consensus study report was sponsored by the National Academy of Sciences and the National Academy of Medicine based in Washington, D.C. and was authored by the Committee on Human Gene Editing: Scientific, Medical, and Ethical Considerations. The committee was co-chaired by:

- [Dr. Alta Charo](#), Sheldon B. Lubar Distinguished Chair and Warren P. Knowles Professor of Law and Bioethics, University of Wisconsin–Madison; and
- [Dr. Richard O. Hynes](#), Investigator, Howard Hughes Medical Institute; Daniel K. Ludwig Professor for Cancer Research, Massachusetts Institute of Technology.

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