

“An Initiative to Guide Decision Making on Human Gene-Editing Research”

Statement of Victor J. Dzau  
President, National Academy of Medicine  
National Academies of Sciences, Engineering, and Medicine

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Good afternoon, Chairwoman Comstock, Ranking Member Lipinski, and members of the Subcommittee. I'm Victor Dzau, president of the Institute of Medicine, which will become the National Academy of Medicine on July 1. I'm pleased to be here today on behalf of the National Academies of Sciences, Engineering, and Medicine. The Academies operate under a congressional charter signed by Abraham Lincoln in 1863 to provide advice to the nation on matters where science, technology, and medicine can solve complex challenges and thereby improve peoples' lives.

Thank you for the opportunity to speak with you today about the important matter of human gene editing and the major initiative we have launched to help guide decision making in this area. The Academies have an established track record of providing leadership in emerging and often controversial areas of genetic research. Our initiative is marshalling the best available expertise to help you and the nation obtain a thorough understanding of gene editing and its potential benefits and risks, which will provide a solid foundation for informed decisions and sound policies on this research.

### Potential Benefits and Challenges

As you will hear from other witnesses today, gene-editing technologies hold great promise for advancing science and improving human health. Powerful new tools such as CRISPR-Cas9 developed by Dr. Doudna and others allow researchers with basic knowledge of molecular genetics to precisely modify the genetic makeup of any living organism. The possible applications for such technologies are many. The genomes of plants and animals could be modified to boost agriculture and food production. Genes of disease-carrying insects could be edited to reduce the spread of malaria, West Nile virus, or dengue fever. In humans, the technologies could offer a cure to often devastating genetic diseases such as Huntington's disease and sickle cell anemia, and help improve understanding and treatment of many other illnesses.

However, these new avenues of research also present many complex challenges, both to the scientific and medical communities and to society as a whole. Research that attempts to alter human genes raises important questions about safety, uncertainties, risks, and ethical considerations. Of particular concern is the potential to make permanent modifications to human DNA in the nuclei of cells in eggs, sperm, or human embryos that are then passed down to succeeding generations. This is known as human germline editing.

Although much remains to be done before these technologies could be deployed safely and efficiently, the availability of these new technologies has certainly intensified debate among scientists and physicians about such research. Recently, through an editorial in a prominent scientific journal, several researchers, including Dr. Doudna, have called for a suspension of studies that attempt to modify the human germline for clinical application until the potential risks, benefits, and ethical concerns are thoroughly explored.

Here in the U.S., there are legislative prohibitions on the use of federal funds for research on human embryos, and constraints on such research when it is subject to oversight by government agencies such as the U.S. Food and Drug Administration. These laws and regulations do not apply to work done internationally without federal funds and without the intent to seek federal approval of any products of that research. In April, Francis Collins, the director of the National Institutes of Health, stated that NIH "will not fund any use of gene-editing

technologies in human embryos.” Collins cited strong arguments against such research, including “serious and unquantifiable safety issues, ethical issues presented by altering the germline in a way that affects the next generation without their consent, and a current lack of compelling medical applications justifying the use of CRISPR/Cas9 in embryos.” And in May, John Holdren, the director of the White House Office of Science and Technology Policy, said that “the Administration believes that altering the human germline for clinical purposes is a line that should not be crossed at this time.”

### The Academies Initiative

It’s clear that the advent of these technologies has brought us to a critical juncture in genetic research. What is needed now is guidance – guidance that is based on an in-depth review of the science underlying gene editing and an understanding of the potential benefits as well as the valid concerns raised by this research. This is exactly the type of leadership for which the National Academies of Sciences, Engineering, and Medicine are known.

Toward that end, on May 18, we announced a major initiative on human gene-editing research. Our work is already well-underway. Just last week, we met with a multidisciplinary advisory group that will help steer our initiative. Their names are appended to my testimony. This group will be instrumental in counseling Ralph Cicerone, president of the National Academy of Sciences, and me to ensure that the Academies’ efforts in this area are comprehensive, inclusive, and transparent.

As with science and medicine in general, gene-editing research is truly an international endeavor, and any future applications will likely be felt around the world. To gather the multinational, multidisciplinary perspectives critical to the success of this initiative, the Academies will convene a global summit to examine recent scientific developments in human gene editing and the range of associated ethical and governance issues. Summit participants will examine:

- The current state of the science and available technologies;
- The rationale for conducting gene-editing research in humans;
- Existing national and international regulatory principles, standards, and guidance for such research and areas where more direction is needed; and
- Ethical and legal considerations in such research.

Concurrently, the Academies will appoint an expert committee to conduct a comprehensive study on human gene-editing research. Like all of our committees, this study committee will represent a wide range of expertise and be carefully screened for bias and conflict of interest. Although the study’s statement of task is still being finalized, some of the questions the committee will likely address include:

- What is the state of the science of gene editing and how rapidly is it advancing?
- What is the evidence on the efficacy and risks of gene editing in humans?
- What are the potential clinical applications and how should their risks and benefits be weighed for current and future generations?
  - What principles and frameworks should be applied for determining which, if any, applications should go forward?
  - What are the ethical, legal, and social implications?

- What oversight mechanisms are needed and which safeguards should be in place to guard against misuse of gene-editing techniques?

Of course, the study will also be informed by our international summit.

### Advances to Benefit Humankind

I am confident that the Academies' initiative will help the nation and the world make sound, evidence-based decisions about this research. Allow me to briefly highlight a few examples of when the Academies have been of similar service

In 1975, the National Academy of Sciences convened what is now known as the Asilomar conference, a landmark turning point for recombinant DNA research. The conference ultimately led to voluntary guidelines to ensure the safety of what was then a new technology. Our 1988 study on mapping the human genome helped steer what has become an incredible source of new scientific advances. In 2005, we issued guidelines for human embryonic stem cell research, which were widely adopted by research institutions, and international scientific societies. And most recently, an international workshop on research of dangerous pathogenic viruses – known as “gain of function” research – will inform new policies for the study of avian influenza, Severe Acute Respiratory Syndrome (SARS), and Middle East Respiratory Syndrome (MERS).

These examples underscore how quickly genetics and biomedical science have advanced over just the past few decades. It's no wonder that all of us – scientists, physicians, policymakers, and the public – want to do everything possible to ensure that these advances continue and that scientific and medical breakthroughs such as gene editing benefit all of humankind. With that goal in mind, the National Academies of Sciences, Engineering, and Medicine are ready to provide a comprehensive understanding of human gene editing and its implications to help guide decisions about its use in the years to come.

Thank you for inviting me to testify. I would be pleased to address questions from the Subcommittee.