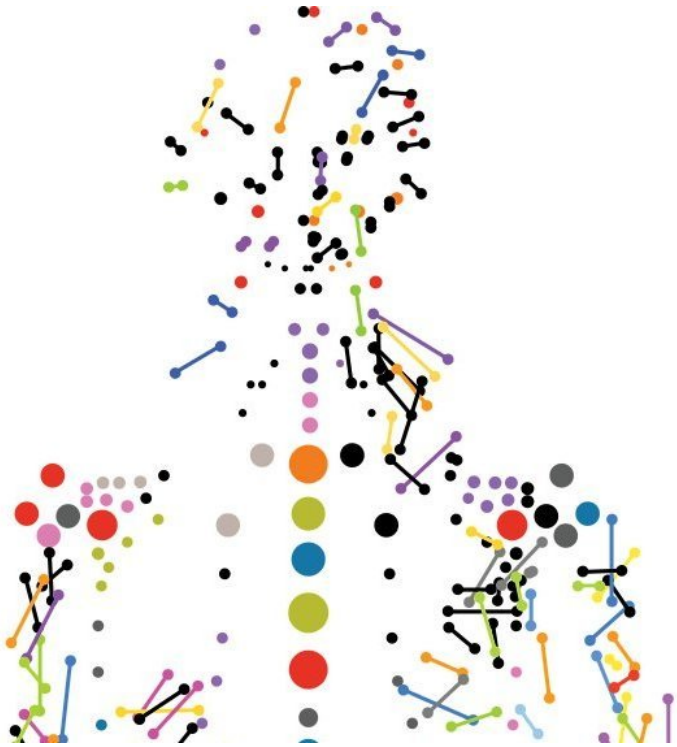
Report: Heritable genome editing technology is not yet ready for clinical use

September 7 2020, by Katherine Fenz



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Gene editing technologies developed in the past decade have proven to be extraordinarily powerful and hold the potential to someday cure or prevent a range of diseases. While applications that introduce edits in somatic cells are being used in the clinic, changes that could be transmitted to subsequent generations (heritable edits) raise additional

technical, ethical and societal issues. Gene editing technologies are not yet ready for applications entailing heritable edits, an international commission has concluded. A range of scientific and medical issues have yet to be addressed, the committee determined, and many ethical, moral, and societal concerns remain.

The commission was convened by the U.S. National Academy of Medicine, the U.S. National Academy of Sciences, and the U.K.'s Royal Society in response to a 2018 announcement by Chinese researchers that twins had been born following gene editing on early embryos. The commission included 18 members from 10 countries, with expertise in science, medicine, bioethics, and law. Rockefeller University President Richard P. Lifton served as co-chair, along with Kay Davies, Professor of Genetics at University of Oxford. The commission was charged with evaluating the state of the science and identifying technical criteria that would need to be met before considering clinical use, and to define a translational pathway for initial clinical use if the technology were demonstrated to be efficient in making intended changes without unintended edits.

"It is very clear that the science is not ready for clinical application, as the ability to make intended edits with high efficiency without off-target edits in human zygotes has not been demonstrated," said Lifton. "In particular, the ability to efficiently make specific single base pair substitutions in zygotes is particularly challenging, and demonstrating that no potentially deleterious unintended changes have been made in the genome is similarly not established. There are many gaps that would need to be addressed before contemplating any responsible clinical effort."

"Moreover, any initial use of heritable human gene editing should proceed cautiously, starting with the most favorable balance of potential benefits and harms, recognizing that potential parents often have a

reasonable alternative for having a biologically related child without a disease-causing genotype, or have alternative means for mitigating effects of the inherited disease," said Lifton. "Our commission defined a responsible clinical translational pathway extending from rigorous preclinical research to demonstrate technical reliability to clinical application. Nonetheless, these criteria would not be sufficient to proceed. This pathway should only be pursued if a country with a robust mechanism for regulatory oversight deemed it was permissible for a specific application after broad discussion of ethical and social issues."

The report also provides guidance on essential elements of national and international scientific governance and oversight, which includes an international scientific advisory panel to continuously monitor the state of relevant science, a body to convene and consider the potential benefits and harms, as well as the ethics of proposed applications, that can inform deliberations of individual countries, and a robust mechanism for reporting of unauthorized uses of the technology.

More information: International Commission on the Clinical Use of Human Germline Genome Editing: www.nationalacademies.org/our- … mline-genome-editing

Provided by Rockefeller University

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