Gene editing

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Genome editing for heritable diseases not yet safe, report states

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Powerful genome editing procedures that could prevent parents from passing on heritable diseases to their children are far from ready for clinical use, and must be proved safe and effective before nations permit them, leading scientists have warned.

In a major report on the procedure, an international commission said no human embryos that have had their DNA edited should be used to establish pregnancies until a substantial body of work shows genetic faults can be corrected precisely and reliably with no harmful consequences.

Even once these considerable hurdles have been cleared, countries should initially only consider allowing human embryo editing to prevent serious, life-shortening diseases caused by flaws in single genes, the report states.

“It came out very clearly that the science is not ready for clinical application, in fact far from it,” Richard Lifton, the president of Rockefeller University in New York and co-chair of the commission, told the Guardian. “There are many gaps that need to be addressed before we believe it would be reasonable to go forward with any clinical effort.”

Genome editing has been hailed as a potential game-changer for combating a slew of heritable disorders such as muscular dystrophy, cystic fibrosis and Tay-Sachs disease. In principle, the faulty genes can be rewritten in IVF embryos allowing prospective parents to have healthy babies.

But the research is at an early stage and numerous studies have shown how genetic tweaks can go awry by changing the wrong strands of DNA, causing fresh genetic mutations, and editing some cells but not others. Such glitches are all the more problematic because they would affect every cell in the child's body and would be passed on to their own children.

While scientists have largely steered clear of gene editing in human embryos, in 2018, the Chinese researcher He Jiankui sparked a global furore when he announced the birth of two baby girls after editing their embryos. He disabled a key gene using a procedure called Crispr in the hope of making the girls immune to HIV. He was later jailed for three years.

The incident prompted the US National Academy of Medicine and the National Academies of Science, along with the UK's Royal Society to convene a commission to assess the state of the science and set out how, if at all, genome editing in embryos might be done responsibly.

In many cases, people who are at risk of passing on genetic diseases can have IVF embryos screened for harmful mutations before going ahead with pregnancy. But in some cases, all of the embryos will be affected, leaving the parents few other options for having genetically-related children.

The report urges countries to hold "extensive societal dialogue" before deciding whether or not to green light so-called "germline" editing. Before proceeding, studies on cells, animals and human embryos must demonstrate the procedure works and does not cause other medical problems. Any edited human embryos must be carefully screened before they are implanted into a woman, and resulting children followed up with medical checks, provided they consent, the report adds.

"We think the bar should be high, and appropriately so," Lifton said. "If you are going to be creating human beings, you want to know that you can reliably make the edits you're intending. If you can't do it reliably, without introducing unintended effects, you shouldn't be going forwards."

Most common diseases are affected by scores of genes and their multiple roles throughout the body are too poorly understood to edit them safely, the report adds. And while genome editing raises the spectre of designer babies, the science is too poorly understood to consider attempting now. For example, Lifton notes that attempts to boost red blood cells to improve endurance might raise the risk of stroke. "There are very strong reasons to be very sceptical about any efforts at enhancement," he said.

Among the report's recommendations are calls for a three-pronged governance structure that monitors genome editing research, its potential to prevent diseases, and puts in place a mechanism for whistleblowers to warn of rogue scientists working on humans. One of these would make recommendations on whether proposed new uses for gene editing were appropriate.

The report came in for immediate criticism, both for being too restrictive, and for not being restrictive enough. Sarah Norcross, director of the Progress Educational Trust, said the commission had gone too far, with a narrow definition of "serious" disease, and an emphasis on single gene disorders.

Meanwhile Marcy Darnovsky, director of the Centre for Genetics and Society in Berkeley, California, said if heritable human genome editing was approved, even for the limited uses, it was "unlikely that any boundaries would hold".

"We could see gene editing for embryos marketed as an enhancement technique, and from there the emergence of a market-based eugenics that would exacerbate already existing discrimination, inequality, and conflict," she said.

Prof Kay Davies, co-chair of the commission and a geneticist at Oxford University, said an ongoing discussion that included all the different views was planned and crucial. "It's not just the science, it's the social and ethical issues. We may decide that germline genome editing should never happen."