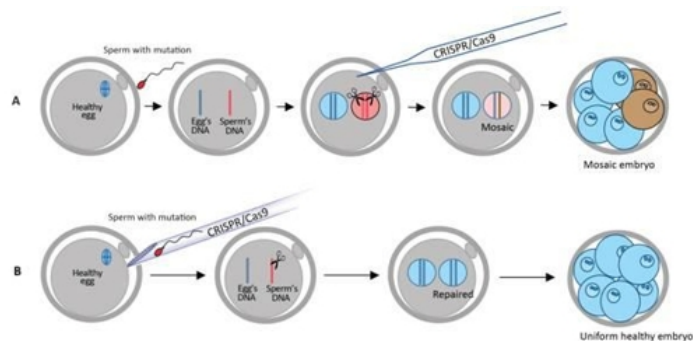




# Breakthrough: Human Embryos Successfully and Safely Gene-Edited

Posted August 03, 2017



According to (<https://www.technologyreview.com/s/608482/gene-editing-study-in-human-embryos-points-toward-clinical-trials/>) a study published Wednesday (<http://nature.com/articles/doi:10.1038/nature23305>), scientists have, for the first time, "successfully edited genes in human embryos" (<https://www.mprnews.org/story/2017/08/02/npr-scientists-precisely-edit-dna-in-human-embryos-to-fix-a-disease-gene>) to fix a common and serious disease-causing mutation", leaving the embryos perfectly healthy and possibly paving the way for children in the future to be born without hereditary conditions. Such mosaic embryos probably arise when the fertilized egg copies its DNA (<http://www.webmd.com/a-to-z-guides/dna-fingerprinting>) before researchers add Cas9, Mitalipov says.

In 2015, scientists in China (<http://irishtimes.com/search/search-7.1213540>) conducted the first known attempt (<http://www.kpbs.org/news/2015/apr/23/critics-lash-out-at-chinese-scientists-who-edited/>) to use CRISPR (<https://techcrunch.com/2016/11/15/chinese-scientists-crispr-a-human-for-the-first-time/>) on human embryos.

But this new (<http://www.nature.com/nature/journal/vaop/ncurrent/full/nature23305.html>) work suggests that gene research is moving faster than the National Academies anticipated, and might soon outpace our efforts to regulate it.

"This is actually very good, and could be very useful in these sorts of cases, but of course it's limiting because it means that that change from the paternal version only goes one way", developmental geneticist Robin Lovell-Badge of the Francis Crick Institute told (<http://www.npr.org/sections/health-shots/2017/08/02/540975224/scientists-precisely-edit-dna-in-human-embryos-to-fix-a-disease-gene>) BuzzFeed News.

A way to fix embryos? But that may require several cycles of IVF, which is expensive and carries with it side effects and complications, before enough genetically healthy embryos are created. Out of 58 embryos, 42 showed the normal gene in every cell. While the US has uniquely lax laws (<https://www.inverse.com/article/34777-human-embryo-crispr-edit-dna-legal-US>) surrounding human genome editing (the government will not fund it, but private funders abound), over 40 countries have laws prohibiting germline gene editing - the kind that can be passed on to future generations - and all European countries are bound by the Convention on Human Rights and Biomedicine, which prohibit the same thing.

There are about 10,000 diseases that are caused by individual mutations and could in theory be fixed with the treatment, which is known as germline gene editing because it involves using chemical scissors to...

Similarly, Anna Middleton, vice chair of the United Kingdom association of genetic nurses and counselors, said while the science is "really elegant" it must be replicated extensively before any firm conclusions can be drawn about the accuracy and safety of the approach.

The researchers concentrated on hypertrophic cardiomyopathy (<http://www.mayoclinic.org/diseases-conditions/hypertrophic-cardiomyopathy/home/ovc-20122102>) (HCM) a heart (<http://www.webmd.com/heart/picture-of-the-heart>) muscle disease that affects about 1 in 500 people and can result in sudden death. In the 1990s he co-developed zinc-finger nucleases (ZFNs), one of the first gene editing technologies, establishing a foundation for modern technologies including CRISPR (<http://time.com/4764488/crispr-genomic-technology/>).

The procedure was most (<https://link.springer.com/article/10.1007%2Fs10815-016-0710-8>) effective when CRISPR-Cas9 was injected into the eggs along with the sperm (<http://www.webmd.com/infertility-and-reproduction/guide/sperm-and-semen-faq>) rather than immediately after.

Mitalipov argues this means that the procedure should not actually be called gene editing, but that it is **more** of an assisted form of natural DNA fix. If even one cell in an early embryo is unedited, "that's going to screw up the whole process", says Mitalipov. This gives parents full control over whether or not their child will carry a disease-associated gene, making it incorrect to think of genome editing in embryos as the only way to prevent genetic disease in kids.

About 72 percent of the embryos were free from the mutation. Normally a sick parent has a 50-50 chance of passing on the mutation.

Beginning the process before fertilization avoided that problem: Until now, "everybody was injecting too late", Mitalipov said. "Really, we didn't edit anything, neither did we modify anything", explained Shoukhrat Mitalipov (<http://www.ohsu.edu/people/shoukhrat-mitalipov/2D760207FF014335B07EC30F3818652F>), lead author and a researcher at the Oregon Health and Science University (<http://www.ohsu.edu/xd/>). But edited embryos are not allowed to be inserted into the uterus and develop into a fetus in any country. The embryos were allowed to grow for only a few days, and there was never any intention to implant them to create a pregnancy. "It's unclear at this point when we would be able to move on", Mitalipov says.

Could we be on our way to designer babies?

Professor Robin Lovell-Badge from the Francis Crick Institute said that the research only appears to work when the father is carrying the defective gene, and that it would not work for **more** sophisticated alterations.

The existence of the testing option means that gene editing of embryos has "a very narrow window of opportunities", says Lluís Montoliu, a genome researcher at the National Centre of Biotechnology in Madrid, Spain.

Otherwise, he said, "this technology will be shifted to unregulated areas, which shouldn't be happening".

Future work will continue to assess the safety and effectiveness of the procedure and efficacy of the technique with other mutations.

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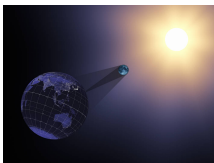
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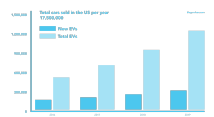
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