New gene editor used to fix disease in embryos: study

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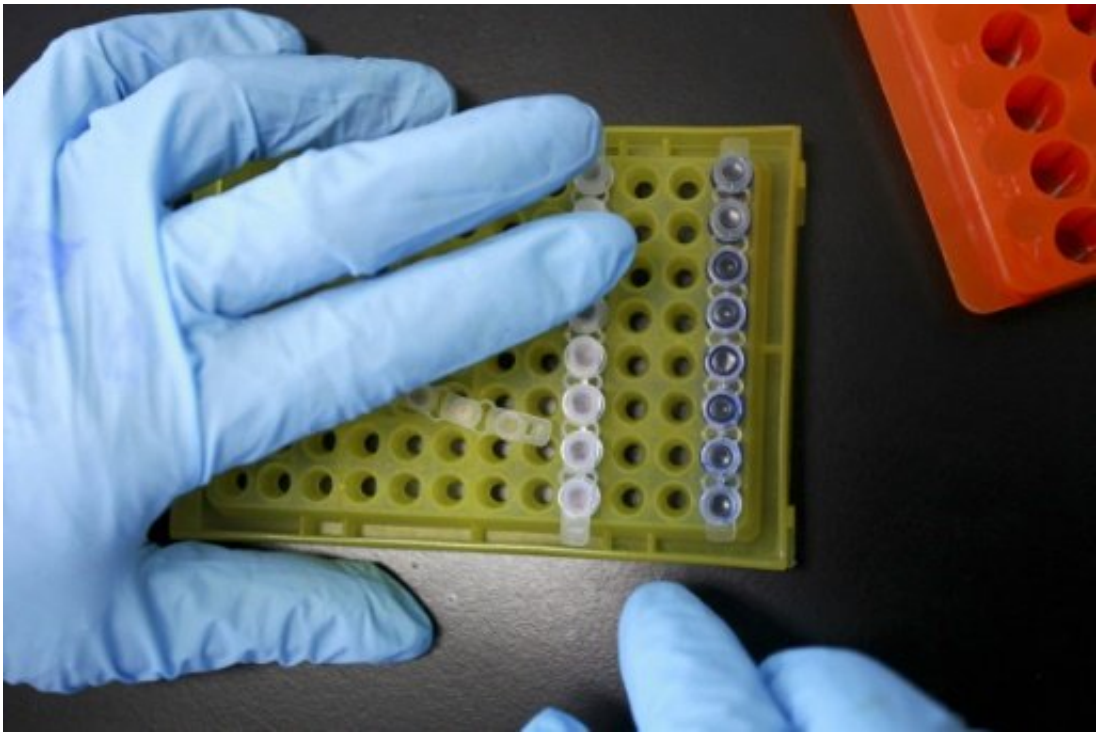

A team of Chinese scientists used a so-called "base editor" to correct a single, mutated "letter" among about three billion in the coding of the human genome

Chinese scientists used an adapted version of a controversial gene-editing technique to correct a disease-causing mutation in human embryos, a medical first cautiously hailed by other experts Thursday.

The team used a so-called "base editor"—an adaptation of the CRISPR-

Cas9 DNA snipping tool—to correct a single, mutated "letter" among about three billion in the intricate coding of the human genome.

The targeted mutation can cause humans to be born with beta-Thalassaemia, a potentially fatal genetic blood disorder.

"This study demonstrated the feasibility of curing genetic diseases in human... embryos by base editor system," the team wrote in the specialist journal *Protein & Cell*.

The journal sparked controversy when it published a paper in 2015 in which the same authors reported on experiments with CRISPR-Cas9 to modify the thalassaemia gene.

That paper led to calls for a halt to experiments involving the genetic editing of human embryos.

Many fear such technology could lead to so-called "designer babies" with desired features such as intelligence engineered into their genes.

For the new study, Puping Liang of the Sun Yat-sen University in China, and a team used a technique based on CRISPR-Cas9, which allows scientists to remove and replace a faulty strand of DNA with pinpoint precision.

Instead of using the Cas9 protein as "scissors" to eliminate the mutated "letter", they used an enzyme to change it.

Can it be improved?

DNA is the instruction booklet for cells to make and sustain life.

It resembles a zipper-like spiral—the teeth on each strand are "base

pairs" of encoding "letters" that chemically match with each other.

The mutation targeted by the Chinese team can cause humans to be born with beta-Thalassaemia, a potentially fatal genetic blood disorder

Adenine teams up with thymine to create the A-T base pair, while cytosine pairs with guanine for the C-G pair.

Thalassaemia can be caused by an "A" base letter converted to a "G" in a specific location of the gene.

For the latest study, the team sought to chemically change the "C" partner of the mutant "G", to a "T".

This would cause the errant "G" to automatically convert to an "A",

expert Robin Lovell-Badge of the Francis Crick Institute, who was not involved in the study, explained via the Science Media Centre.

The method eliminated the need to cut DNA, and the team were successful about one in five times.

They worked with cloned embryos that were kept alive for just a few days for the purposes of lab experimentation

Observers said the technique appeared to be an improvement on standard CRISPR-Cas9.

"This powerful study sheds new light on precise gene correction for single gene disorders," commented Helen Claire O'Neill of University College London.

"It remains to be seen whether the efficiency... can be improved upon."

For Darren Griffin, a genetics professor at the University of Kent, the paper showed that "the ethical implications of gene manipulation in embryos need a thorough examination where safety is of paramount concern."

US-based scientists in August reported using CRISPR-Cas9 to repair a disease-causing mutation in the DNA of early-stage human embryos.

Last week, British scientists said they had used CRISPR-Cas9 to reveal the role of a key gene in the early development of human embryos.