

Promise and peril: a primer on gene editing

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Britain's granting of a licence Monday for scientists to alter the genes of embryos for infertility research has thrown the controversial technique under a white-hot spotlight.

But what is it?

Gene editing is the direct, surgical modification at the molecular level of a genome, which is the genetic blueprint for every individual animal or plant.

It is far more precise than conventional selective breeding or an earlier generation of genetic engineering techniques.

Gene editing is already widely used to add desirable traits to food crops or livestock, and in lab animals for research on disease, and Chinese researchers announced last year they manipulated the genomes of non-viable human embryos looking for a way to correct a rare and fatal blood disorder.

Britain is the next country to allow scientists to alter human embryos—restricted to research on the causes of infertility and miscarriages.

The embryos to be used in the research are ones that would have been destroyed, donated by couples receiving In-Vitro Fertilisation (IVF) treatment who do not need them.



The new authorisation requires that these embryos be destroyed within 14 days.

Many scientists welcomed the decision to create a regulatory framework for the technique, but others expressed concern that gene editing may one day be used to make "designer babies" with selected physical or intellectual traits.

There are several forms of gene editing, all of them involving the removal or addition of gene snippets.

The human genome consists of long strings of DNA made up of about three billion "base pairs" of the chemical "letters" A, T, G and C (adenine, thymine, guanine and cytosine) in a particular order.

These comprise about 20,000 to 25,000 genes.

Tinkering with evolution

Gene editing makes it possible to remove or insert snippets of DNA at precise locations using molecular "scissors", like altering a film sequence or using the "find & replace" function in a word-processing software.

This allows researchers to "knock out" genes that cause disease (certain forms of cancer or Tay-Sachs, a very rare condition which destroys nerve cells, for example), or repair a naturally-occurring mutation.

The editing methods in use today are divided into two broad groups. In the first, the engineered changes persist only for the life of the organism, in the second it is passed on to the next generation.

The newest technique—known by it's acronym CRISPR/Cas9—has rapidly dominated the field because it is cheaper and much simpler to



use. It is also the one best suited for editing the genome of still-developing embryos.

But such so-called "germline modification" means that DNA changes, if the embryo were allowed to live and become an adult, would be passed on to its offspring, effectively tinkering with the process of evolution.

CRISPRs—"clustered regularly interspersed short palindromic repeats"—are an immune defence system found in bacteria to protect against viruses.

Relative to other methods, they can be easily engineered to home in on the exact spot where a break in the genome should be made.

Once a sequence of DNA is broken, scientists can trigger one of two kinds of repair mechanisms. One requires using a DNA fragment as a template—including the new sequence to be inserted—for the repair.

The other does not need a template, and simply patches the rupture in the genome.

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