UK scientists ready to genetically modify human embryos

Researchers awaiting approval to use gene editing in embryos, which they hope will help them understand early stage life and improve fertility treatment

Kathy Niakan and her team want to use a powerful new procedure called gene editing to disable certain genes in early stage human embryos. Photograph: Francis Crick Institute

Ian Sample Science editor

@iansample

Wednesday 13 January 2016 17.18 GMT Last modified on Thursday 15 December 2016 16.28 GMT

Comments

303

Scientists in Britain are ready to genetically modify human embryos for the first time as part of a research effort to shed light on the root causes of recurrent miscarriages.

Researchers at the Francis Crick Institute in London could receive approval for the controversial work as early as Thursday when the government's fertility watchdog meets to consider the proposal.

If the regulator grants a research licence, the UK team stands to become only the second in the world to modify genes in human embryos. All of the embryos used in the work will be donated with consent by couples who have a surplus after IVF treatment.

Kathy Niakan and her team want to use a powerful new procedure called gene editing to disable certain genes in early stage human embryos. The scientists will then observe how the embryos grow until they are a week old, before destroying them. The experiments should help unravel the complex genetic instructions that turn a freshly-fertilised egg into a healthy embryo.

"The research could lead to improvements in fertility treatment and to a better understanding of the first stages of life," Niakan told reporters at a briefing in London. Under British law, the embryos cannot be transferred to women to achieve a pregnancy.

Gene editing has exploded onto the science scene in the past five years and has since been taken up by hundreds of laboratories around the world. It is so powerful that leading researchers have convened a series of meetings to thrash out guidelines for its responsible use. The most recent took place last month in Washington DC. There, scientists agreed to press on with research that could lead to new medical therapies, but warned that it was too risky to consider genetically modifying human embryos that were destined to become people.

The Human Fertilisation and Embryology Authority could approve Niakan's research on Thursday, but is more likely to request more information from the team before reaching a final decision. A separate ethics committee must then agree to the experiments. With approval from both, Niakan said she could start work in a matter of months.

Human reproduction is staggeringly inefficient. Less than half of new embryos survive the first week to form what scientists call a blastocyst - a ball of 250 or so cells that are on the brink of becoming the different tissues and organs needed to make a healthy foetus. Of those that last a week, only half will implant in the womb, and only a quarter will survive more than three months in pregnancy.

While studies in mice and other animals have helped scientists understand how some embryos grow, Niakan said researchers needed to study human embryos directly to understand the specifics of early human development.

The gene editing procedure, called Crispr-Cas9, works much like the search and replace function on a word processor. It can seek out a target gene and make precise changes to the DNA code. Scientists can use the tool to change a single letter of genetic information, to delete a gene, or replace a faulty one with a healthy copy.

Niakan aims to knock out up to four genes in one-day-old human embryos to see how they affect development over the first week of life. Testing the effects of each gene is expected to require 20 to 30 embryos.

The crucial questions she hopes to answer are about how genes work together to produce three classes of cells in the week-old embryo. The first class is an outer group of cells that forms the placenta and attaches the embryo to the womb. Inside the embryo are two other groups of cells. One forms all of the tissues needed to make the foetus, while the other forms an egg sac that supports the foetus as it grows. Genetic glitches in any of these could prevent an embryo from surviving.

Niakan will not use gene editing to improve IVF embryos. But the research could help doctors understand which embryos have the best chances of producing a live birth, and pave the way for drugs that can boost the chances of poor quality IVF embryos before they are implanted.

“Miscarriages and infertility are extremely common but not very well understood,” Niakan said. “You never can predict where the research will lead but we hope it would be a great benefit for fertility treatments in the long-term.”

Last year, scientists in China became the first to genetically modify human embryos when they tried - and failed - to mend a faulty gene in human embryos. The research was performed on abnormal IVF embryos which could never have formed viable foetuses.

Sarah Norcross at Progress Educational Trust, a charity that works to improve the choices of people affected by infertility and genetic diseases, said: “This is an important piece of basic scientific research. Recurrent miscarriage affects a huge number of people and it isn’t greatly understood. People are just told

to go away and try again. To improve our understanding of something like that, which has a huge impact on people, is really valuable."

A spokesperson for Comment on Reproductive Ethics said the group was "opposed to any form of destructive research on human embryos".

Gene editing: how it works

Gene editing is powerful technique that can be used to change anything from a single letter of DNA code to a whole series of genes inside a living organism. There are different ways to do it, but the most popular, known as Crispr-Cas9, was discovered by accident by scientists studying how bacteria fight off viral infections.

To defend themselves, the bugs release strands of genetic material that stick to the virus's genome and slice it up with enzymes. Gene editing co-opts this defence to produce what has been called "molecular scissors".

To edit a gene in a human embryo, scientists first create a strand of genetic letters, made from Gs, Ts, Cs, and As, which attaches to the gene they want to change, but not to others. Added to the strand are enzymes that cut through DNA on contact. When injected into the embryo, the strand seeks out and lines up with the target gene and makes a cut. This disables the gene.

More advanced forms of Crispr-cas9 carry new genes into cells, which can replace faulty ones that are removed. Gene editing can disable scores of genes at once. In a record-breaking effort, researchers at Harvard last year used Crispr-cas9 to knock out more than 60 genes in pig embryos in work that could make pig organs safe to implant in humans.

https://www.theguardian.com/science/2016/jan/13/uk-scientists-ready-to-genetically-modify-human-embryos