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Genetic editing that could cure muscle disease

By **Sarah Knapton** SCIENCE EDITOR

A MOUSE has been genetically engineered to be free of muscular dystrophy in a major breakthrough which could eventually see many genetic diseases cured in a similar way.

It is the first time that researchers have proven that the technique can work in a mammal and they are hopeful it will be replicated in humans.

This kind of genetic editing is likely to prove less controversial than tinkering with the DNA of a human embryo, as the patient would be able to choose the treatment.

Around 70,000 people in the UK suffer from Duchenne muscular dystrophy, with many needing a wheelchair by the age of 10 and few surviving past their 30th birthday.

The disease is caused by problems with the body's ability to produce dystrophin, a protein chain which binds the interior of a muscle fibre to its surrounding support structure. In Duchenne muscular dystrophy gene, mutations stop the protein chain being built and muscle tends to shred and slowly deteriorate, eventually causing fatal damage to the heart and lung muscles.

But geneticists at Duke University, North Carolina, US, have shown it is possible to snip away the bad DNA code using a molecular tool known as CRISPR, which acts like tiny scissors.

Without the gene mutation, the mouse began to produce dystrophin again and muscles repaired themselves. "We are very excited, and I agree that this is a milestone in how gene editing, and CRISPR in particular, can be used to treat genetic diseases," said Charles Gersbach, associate professor of biomedical engineering at the university.

"Our study was designed to use an approach that could be extended to human clinical trials."

The researchers used a virus, which had been purged of its harmful genes, to carry the therapeutic genes into the mouse's cells.

The research was published in the journal *Science*.