

Gene therapy helps dogs with muscle dystrophy, humans next?

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Researchers have used gene editing to reverse symptoms in dogs of Duchenne muscular dystrophy (DMD)—a muscle-wasting and lifeshortening disease that affects one in 5,000 baby boys.

The dogs' symptoms remained in check two years after receiving a single injection, a team reported Tuesday in the journal *Nature Communications*.



Successful treatment of a large mammal is a crucial step on the road to finding a treatment for human sufferers of DMD.

The results, said the team, were "paving the way for human clinical trials."

Duchenne muscular dystrophy is caused by genetic mutations that interfere with production of dystrophin, a protein needed for healthy muscles.

The international team developed a new type of <u>gene transfer therapy</u> to restore dystrophin expression in affected dogs.

They injected the dogs with "microdystrophin"—a compressed version of the dystrophin gene which is one of the largest in the human body with 2.3 million base pairs of coding "letters".

Given its size, it has been impossible to fit the distrophin gene into an emptied virus, which is used to deliver modified <u>genes</u> to patients.

Dogs given the treatment showed "significant restoration of muscle function with a stabilisation of clinical symptoms for more than two years after injection," the researchers said in a statement.

Geneticist Darren Griffin of the University of Kent commented that this was "really a very exciting study indeed".

"The disease has long been a target for <u>gene therapy</u> and it is only to be hoped that sufficient funds can be awarded for this research to reach its natural conclusion and go into full clinical trials," he said via the Science Media Centre.

Boys affected by Duchenne muscular dystrophy develop muscle



weakness in their first year of life, and generally become wheelchairbound before the age of 15.

Muscle wasting and breathing and heart problems follow, and patients rarely survive into their 40s.

More information: Caroline Le Guiner et al. Long-term microdystrophin gene therapy is effective in a canine model of Duchenne muscular dystrophy, *Nature Communications* (2017). DOI: 10.1038/ncomms16105

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