CRISPR treatment for dogs with muscular dystrophy could one day lead to a cure for humans

Dogs suffering from muscular dystrophy are having their genomes edited with CRISPR, and the results are “mind-blowing.” Researchers from Texas … unveiled data showing how CRISPR, a new tool for modifying genes, could reverse the molecular defect responsible for Duchenne muscular dystrophy, a devastating illness that affects about one in 5,000 boys.

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If the treatment can stop muscular dystrophy in dogs, that would set the stage for giving the experimental treatment to boys, according to the research team.

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[D]uring a conference at the National Institutes of Health, researcher Leonela Amoasii reported how the team had infused CRISPR into the bloodstream of one-month old puppies bred to suffer from the disease. It widely repaired the cells of their muscles and hearts, she said.
…

[T]he CRISPR strategy entails using a virus to add the editing tool to a person’s body, where it can repair the genetic problem.
Restoring the function of a person’s own gene could be an advantage, though the challenge for gene editing is that more than 3,000 different mutations that can cause muscular dystrophy. Therefore, more than one CRISPR treatment will be needed to fix them all.

The teams at Exonics and in Texas are initially targeting one region of the gene, called exon 51, where a repair could help about 13 percent of patients.

Read full, original post: A CRISPR cure for Duchenne muscular dystrophy is closer after a trial in dogs