Lou Gehrig’s disease might be treatable using CRISPR

University of California, Berkeley scientists have for the first time used CRISPR-Cas9 gene editing to disable a defective gene that causes amyotrophic lateral sclerosis, or Lou Gehrig’s disease, in mice, extending their lifespan by 25 percent.

The therapy delayed the onset of the muscle wasting that characterizes the disease, which results in progressive weakness and eventually proves fatal when the muscles that control breathing fail.

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The UC Berkeley research team used a virus that Schaffer’s team engineered to seek out only motor neurons in the spinal cord and deliver a gene encoding the Cas9 protein into the nucleus. There, the gene was translated into the Cas9 protein, a molecular scissors that cut and disabled the mutant gene responsible for ALS.

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The researchers found that, at death, the only surviving motor neuron cells in the mice were those that had been “infected” with the virus and contained Cas9 protein, said Thomas Gaj, a postdoctoral fellow who led the study, now at the University of Illinois at Urbana-Champaign.

“The treatment did not make the ALS mice normal and it is not yet a cure,” Schaffer cautioned. “But based upon what I think is a really strong proof of concept, CRISPR-Cas9 could be a therapeutic molecule for ALS.”

Read full, original post: First Step Toward CRISPR Cure for Lou Gehrig’s Disease