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Gene-editing technique heals mice with muscular dystrophy

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Duke University researchers reported December 31 in the journal Science that mice with muscular dystrophy have responded well to a gene-editing therapy devised to treat Duchenne muscular dystrophy

AFP

WASHINGTON — Researchers have succeeded for the first time in reversing a degenerative illness in mice, using a revolutionary gene-editing technique that one day may help cure similar diseases in humans.

Three studies published in the journal Science said researchers employed a technique known as CRISPR —clustered regularly interspaced short palindromic repeats—to cure an adult mouse model suffering Duchenne muscular dystrophy, a disorder that affects one in every 5,000 baby boys.

“This marks the first time that CRISPR has successfully treated a genetic disease inside a fully developed living mammal with a strategy that has the potential to be translated to human therapy,” researchers said.

Duchenne muscular dystrophy causes progressive weakness because of genetic mutations that interfere with the production of dystrophin, a protein needed to form healthy muscle.

The novel gene-editing technique fixes mutations that cause genetic diseases by making precise changes to the DNA of the ailing subject, using non-pathogenic viruses as delivery vehicles for the modified genes.

Charles Gersbach, associate professor of biomedical engineering at Duke University and the leading scientist on one of the studies, recently began focusing on CRISPR/Cas9—a modified version of a bacterial defense system that targets and slices apart the DNA of familiar invading viruses.

After refining the technique, Gersbach and his team first delivered the therapy directly to a leg muscle in an adult mouse, resulting in an increase in muscle strength as well as some correction of muscles throughout the body, including in the heart.

“There is still a significant amount of work to do to translate this to a human therapy and demonstrate safety,” Gersbach cautioned.

“But these results coming from our first experiments are very exciting.”

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