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Stem Cells Hold Promise for Muscular Dystrophy

Injections helped restore damaged muscle in mice, researchers report

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THURSDAY, July 10 (HealthDay News) -- Researchers are reporting that they've managed to repair damaged muscle in mice with a form of muscular dystrophy by injecting them with specialized stem cells from skeletal muscle.

There's no guarantee that the treatment will translate to humans with the disease, said study author Amy Wagers, an assistant professor in Harvard University's Stem Cell and Regenerative Biology Department.

Still, the findings are going to help scientists "move forward very quickly with human stem cells," she said. "It is an important step."

There are more than 30 kinds of muscular dystrophy, all genetic diseases that cause skeletal muscles to degenerate. The most common form, Duchenne muscular dystrophy, mainly affects boys and is caused by the lack of a protein that helps muscles stay intact.

Many children with the disease cannot walk and must live on respirators. The disease is usually fatal by early adulthood.

"The best current treatment is with steroids, which tend to slow the disease progression," said Paul Muhlrad, research program coordinator with the Muscular Dystrophy Association. "However, there are a number of promising experimental therapies under development, including gene and cell therapies and treatment with a high-tech drug."

In the new study, Wagers and her colleagues turned to purified stem cells that create muscle. The cells came from healthy adult mice and were injected into mice with a disease

that scientists consider to be the equivalent to Duchenne muscular dystrophy.

The findings were published in the July 11 issue of *Cell*.

Like a replacement division sent to replace exhausted soldiers, the stem cells restored muscle. "They actually came in and started making muscle," she said.

The stem cells even left a supply of reserve cells for future use, Wagers said. These reserve cells were a bonus: "They can be activated if needed to repair damage later," she explained.

There is one hitch, however. The treatment probably won't help restore muscle in the heart, which some forms of muscular dystrophy target.

For now, the next step is to see if the approach will work in humans, Wagers said.

Muhlrad said the study is exciting, especially because it relies upon stem cells that come from adults, not embryos. Researchers have been working on a similar treatment using embryonic stem cells, but "that method entails some technical and ethical challenges," he said.

As for humans, the potential for the new stem cell treatment is unknown.

"Researchers will have to devise effective methods for isolating the required numbers of cells from human donors, for delivering those cells to all the affected muscles in patients, and for ensuring that the transplanted cells are not rejected by the recipient's immune system," Muhlrad said.

In general, "stem cell treatment is certainly not yet ready for prime time for muscular dystrophy, though there have been a number of promising preclinical studies in animals, which we're hopeful will enter human clinical trials soon."

More information

Learn more about muscular dystrophy from the National Institute of Neurological Disorders and Stroke.

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