Gene, stem cell therapy only needs to be 50 percent effective to create a healthy heart

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Heart disease is the leading cause of death in the United States and greatly affects the quality and length of life for individuals with specific forms of muscular dystrophy. Recent discoveries have demonstrated that gene and/or stem cell therapy could help a variety of organs in the body, but until now scientists have been unsure whether the heart could benefit from these treatments.

According to a new study, recently published in *Circulation Research*, a journal of the American Heart Association, University of Missouri-Columbia researchers have demonstrated that a muscular dystrophy patient should be able to maintain a normal lifestyle if only 50 percent of the cells of the heart are healthy.

Patients with Duchenne muscular dystrophy and Becker muscular dystrophy have a gene mutation that disrupts the production of a protein known as dystrophin. Absence of this protein starts a chain reaction that eventually leads to muscle cell degeneration and death. Eventually, the damaged muscle tissue is replaced by fibrous, bony or fatty tissue and loses function. In the heart, this leads to severe heart disease and can place severe limitations on individuals afflicted with the disease.

In the past, scientists believed that the only way to have a healthy heart was to rid the heart of all damaged tissue. The heart is considered to be a “synchronized organ;” therefore, it was believed that the heart needed to maintain 100 percent normal cells in order to stay healthy.

In gene therapy, mutated genes are replaced with healthy genes. In stem cell therapy, diseased cells are replaced with healthy cells. However, in these gene and stem cell therapies, it is not feasible to fix every cell in the heart. Previously, scientists were uncertain whether partial correction could benefit patients.

“In our study, we found that a heart with 50 percent normal cells looks like a normal heart,” said Dongsheng Duan, an associate professor of molecular microbiology and immunology at the MU School of Medicine. “More importantly, it acts like a normal heart. This is the first time that we have concrete evidence that partial gene or cell therapies will be effective for preventing heart disease in a mouse model of muscular dystrophy.”

“It’s important to note that this could improve the quality of life for individuals who have this heart condition,” said Brian Bostick, a doctoral student in molecular microbiology and immunology and the first author of the study. “We’re also looking at this as a possible way to prevent heart disease. If we can treat it early through gene therapy or cell therapy, we know now that it can be very beneficial for patients.”

The MU researchers said that this finding would have a positive impact on the ongoing gene and cell therapy studies in animal models of muscular dystrophy as well as in human patients. It also raises the hope of developing effective gene and cell therapies for patients suffering from other heart diseases.

Source: University of Missouri-Columbia

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