Stem cell therapy shows promise in small clinical trial for rare lung disease

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Dr. Duncan Stewart of The Ottawa Hospital and the University of Ottawa has published promising results of the first clinical trial in the world of a genetically-enhanced stem cell therapy for pulmonary arterial hypertension. Credit: Dwayne Brown

Canadian researchers have published promising results of the first clinical trial in the world of a genetically-enhanced stem cell therapy for pulmonary arterial hypertension (PAH). This rare and deadly disease mainly affects young women, and is characterized by very high pressure in the arteries supplying blood to the lungs. In some cases, PAH is caused by a defective gene, but in many cases the cause is unknown. Currently available drugs can modestly improve symptoms and exercise capacity (at best), but cannot repair the blood vessel damage to the lungs or cure the disease.

The trial, published in *Circulation Research*, was designed to test the feasibility and side effects of a genetically-enhanced stem cell therapy to repair and regenerate lung blood vessels in PAH. Seven patients underwent a blood cell selection process (apheresis) to harvest a certain population of their white blood cells. These cells were then grown in the laboratory under special conditions to select for stem-like cells called endothelial progenitor cells. These cells were genetically engineered to produce greater amounts of nitric oxide, a natural substance that widens blood vessels and is essential for efficient vascular repair and regeneration. The genetically enhanced cells were then injected directly into the lung circulation of the same patient.

Although the study was not designed to rigorously assess benefits of the therapy, the researchers did observe that patients had improved blood flow in the lungs in the days following the therapy, and enhanced ability to exercise and better quality of life for up to six months after the therapy. However, there was no placebo group in this study, so it is impossible to know for sure if the effects observed were due to the cells or to psychological effects.

The therapy was generally well-tolerated, however one patient who had very severe and disease and signs of poor prognosis died one day after treatment. This was not unexpected, given the patient's declining condition prior to treatment.

"Pulmonary arterial hypertension is a deadly and incurable disease that often strikes people in the prime of their life," said Dr. Duncan Stewart, senior author of the paper, senior scientist at The Ottawa Hospital and professor of medicine at the University of Ottawa. "We desperately need new therapies for this disease, and regenerative medicine approaches have shown great promise in laboratory models and in clinical trials for other conditions."

"This trial shows that genetically-enhanced stem cell therapy is a promising treatment approach for pulmonary arterial hypertension," continued Dr. Stewart, who is also a practicing cardiologist and Executive Vice-President of Research at The Ottawa Hospital. "Although this is an important start, we will need to do larger studies to establish whether this therapy can produce important and durable benefits for people suffering from this

challenging disease."

Dr. Stewart is also leading the first clinical trial in the world of a genetically-enhanced stem cell therapy for heart attack.

Provided by Ottawa Hospital Research Institute

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