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Study of gene transfer for erectile dysfunction shows promise

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The first human study using gene transfer to treat erectile dysfunction (ED) shows promising results and suggests the potential for using the technology to treat overactive bladder, irritable bowel syndrome and asthma, according to the researchers.

"In the small pilot study, this new therapy was well tolerated and safe," said George Christ, Ph.D., senior researcher and a professor at the Institute for Regenerative Medicine at Wake Forest University School of Medicine. "It provides evidence that gene transfer is a viable approach to treating ED and other diseases involving smooth muscle cells."

The results of the study, which included 11 men with ED, are reported online today in Human Gene Therapy. The technology was developed by Christ and Arnold Melman, M.D., when they worked together at Albert Einstein College of Medicine in the Bronx, New York.

Unlike traditional gene therapy, the gene transfer approach being pioneered by Christ and Melman does not change the DNA or genetic code of cells. Instead, small pieces of DNA reach the nuclei of cells and this causes them to increase production of particular proteins. These proteins help relax smooth muscle cells, the type of muscle found in the penis as well as in hollow organs such as the bladder. Relaxing the tissue allows the penis to fill with blood and become erect.

Previous research has shown that more than 50 percent of men between 40 and 70 years old and 70 percent over age 70 may have ED. The new

therapy is a potential alternative to oral medications, such as Viagra, which are not effective for an estimated 30 to 40 percent of men with ED.

A possible advantage of gene transfer is that a single treatment could last for months. In the current study, improvements were maintained through the 24 weeks of study.

The study was conducted from May 2004 to May 2006 at Mount Sinai School of Medicine and New York University School of Medicine. Men ranged from 42 to 80 years old with a mean age of 59. Six subjects were white, four were black and one was Hispanic. In half of the subjects, the cause of ED was diabetes or cardiovascular disease – both of which can interfere with the ability of smooth muscle cells to relax.

The primary goal of the study was to determine the safety and tolerability of the new therapy. However, the results also showed that at the highest doses, men reported highly significant improvements in erectile function.

The DNA segments – mixed into plasma – were injected into the corpus cavernosum, expandable tissue along the length of the penis that fills with blood during erection. A variety of clinical and laboratory tests were used to assess safety. In addition, effectiveness was measured using the International Index of Erectile Function scale, a questionnaire that is commonly used to measure ED. Patient responses were validated by their partners.

Researchers identified no safety issues with the treatment. Participants who received the highest two doses had apparent sustained improvements in ED as measured by the questionnaire. The researchers said that a larger study that includes a "control" group treated with a placebo is needed to confirm the safety and effectiveness of the

treatment.

Other researchers on the project were Melman, Natan Bar-Chama, M.D., with Mount Sinai School of Medicine, Andrew McCullough, M.D., with New York University School of Medicine, and Kelvin Davies, Ph.D., with Albert Einstein College of Medicine.

The technology is being developed by Ion Channel Innovations (ICI), a development stage biotechnology company, in which Christ and Melman are co-founders and directing members. The therapy is known as ion channel therapy because the proteins it targets are potassium channels, "gates" within cells critical for contraction and relaxation of smooth muscle.

At the Wake Forest Institute for Regenerative Medicine, Christ is continuing to pursue the therapy in collaboration with ICI, and is also exploring the potential of combining gene transfer with traditional oral medications to further increase the clinical utility of the technology. The Albert Einstein College of Medicine at Yeshiva University owns the ICT patents and has granted the company exclusive, worldwide rights.

Source: Wake Forest University Baptist Medical Center