Chinese scientists use gene-editing techniques in humans for first time cnn.com

By James Griffiths and Serenitie Wang, CNN November 15, 2016

Hong Kong (CNN) Chinese scientists have become the first in the world to use the revolutionary CRISPR-Cas9 gene-editing technique in humans.

On October 28, genetically modified cells were injected into a patient at the West China Hospital in Chengdu with aggressive lung cancer, according to the scientific journal Nature. The team of scientists, led by Lu You of Sichuan University, extracted immune cells from the patients and edited them using CRISPR-Cas9.

The technique knocks out a gene that normally acts as a check on the cell's ability to launch an immune response and prevents it from attacking healthy cells. The modified cells were then multiplied and re-introduced into the patients' bloodstream where, it is hoped, they will home in on the cancer and wipe it out.

Liao Zhilin, who handles communications for the team, told CNN "everything is going as planned," but would not go into details. He said information on the results and findings of the study would be released when they are ready.

Gene-editing

CRISPR stands for clustered, regularly interspaced, short palindromic repeats -- regular patterns of DNA sequences which can be edited out of genes. Cas9 is a type of modified protein injected into a body to work on the DNA, like a pair of scissors that can snip the genes.

The technique is based on a decade-old discovery that certain bacterial cells can identify invading viruses and chop up their DNA. CRISPR-Cas9 adapts that technique to allow us to edit genes, removing harmful diseases and even allowing the creation of hybrid human-animal organs to fill the transplant gap.

New space race

Lu's team is not the only one working on using the gene-editing technique in humans. A planned US trial is due to start in early 2017, using CRISPR-edited genes to treat various cancers.

"I think this is going to trigger 'Sputnik 2.0', a biomedical duel on progress between China and the United States, which is important since competition usually improves the end product," Carl June, an immunotherapy specialist at the University of Pennsylvania, and scientific adviser to the US trial, told Nature.

In March 2017, a team at Beijing University hopes to launch three clinical trials using gene-editing against bladder, prostate and renal-cell cancers.

Safety

Lu's team plans to treat 10 patients, and the main purpose of the trial is to test its safety. Patients will be monitored for six months to determine whether there are any negative side effects from the treatment.

Multiple trials on human embryos by Chinese scientists, while providing potentially live-saving insights into the treatment of HIV and other diseases, have raised ethical questions, particularly with regard to the future potential for so-called "designer babies."