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https://www.gesundheitsindustrie-bw.de/en/article/press-release/gene-scissors-show-promise-treating-rare-immune-disorder

Gene scissors show promise for treating a rare immune disorder

Medical Center – University of Freiburg demonstrates a fundamental therapeutic approach for hereditary immune system defects in a mouse model. The safety profile of genome editing varies significantly depending on the method and cell type.

Researchers at the Faculty of Medicine – University of Freiburg have demonstrated in the laboratory that a hereditary defect in the immune system can be specifically modified using gene scissors. In a mouse model of a rare immune disorder, this approach restored the function of key immune cells and protected against a life-threatening inflammatory response. At the same time, the scientists demonstrate that the safety of gene scissors depends on their type and site of application. The findings were published on May 13, 2026, in the journal *Cell Stem Cell*.

"Our results show that genome editing can be a promising approach for correcting rare hereditary diseases," says Prof. Dr. Toni Cathomen, last author of the study and director of the Institute of Transfusional Medicine and Genetherapy at the Medical Center – University of Freiburg.

How gene scissors work in the genome

Gene scissors are molecular biology tools that allow researchers to specifically modify individual sites in the genome. They recognize a specific DNA sequence and cut the DNA strand or specifically alter individual building blocks of the genetic information. The cell then repairs this site on its own. In this way, disease-causing genetic errors can be specifically modified directly in the genome.

Faulty immune response can be corrected using gene scissors

The study focused on familial hemophagocytic lymphohistiocytosis. In this rare genetic disorder, certain immune cells can no longer effectively eliminate infected body cells. As a result, the immune system can spiral out of control. Using gene scissors, the Freiburg researchers specifically modified the disease-causing defect in the genetic material of mice. As a result, the cells were able to function correctly again, and the animals were protected from the severe inflammatory reaction.

Safety Must Be Tailored to the Procedure

At the same time, the team compared various genome editing methods across different cell types. They found that unwanted genetic changes vary significantly depending on the method and cell type. This is a key insight for the development of new therapies. "The safety of genome editing methods must always be evaluated within the specific clinical context," says Cathomen.

Publication:
Genotoxicity profiling reveals distinct platform- and cell type–specific effects in therapeutic gene editing for genetic hyperinflammation
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Further information

- University Hospital Freiburg