New genome-editing technology to help treat blood cancers

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Australian researchers have developed a new genome editing technology that can target and kill blood cancer cells with high accuracy.Dr Brandon Aubrey, Dr Gemma Kelly and Dr Marco Herold (L-R) from the Walter and Eliza Hall Institute of Medical Research in Melbourne, Australia, led the research.Using the technology they were able to kill human lymphoma cells by locating and deleting an essential gene for cancer cell survival. Credit: Walter and Eliza Hall Institute of Medical Research.

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Using the technology, researchers from the Walter and Eliza Hall Institute were able to kill human lymphoma cells by locating and deleting an essential gene for cancer cell survival.

The research, published in the journal *Cell Reports*, provides a 'proof of concept' for using the technology as a direct treatment for human diseases arising from genetic 'errors'.

Dr Brandon Aubrey, Dr Gemma Kelly and Dr Marco Herold adapted the technology, called CRISPR, to specifically mimic and study blood cancers. The Walter and Eliza Hall Institute has one of the most advanced CRISPR laboratories in Australia, established and led by Dr Herold.

Dr Aubrey, who is also a haematologist at The Royal Melbourne Hospital, said the team used the CRISPR technology to target and directly manipulate genes in blood cancer cells.

"Using preclinical models, we were able to kill human Burkitt lymphoma cells by deleting MCL-1, a gene that has been shown to keep cancer cells alive," he said. "Our study showed that the CRISPR technology can directly kill cancer cells by targeting factors that are essential for their survival and growth. As a clinician, it is very exciting to see the prospect of new technology that could in the future provide new treatment options for cancer patients."

The CRISPR/Cas9 system works by efficiently locating and targeting particular genes of interest in the whole genome. It can either target the gene to introduce mutations that make the gene non-functional, or introduce changes that make mutated genes function normally again.

Dr Herold said pharmaceutical companies around the world were already investing millions of dollars to develop CRISPR as a tool for treating genetic diseases such as cancer.

"There is a lot of excitement and a significant amount of resources being invested worldwide to use CRISPR technology for treating patients," Dr Herold said. "The technology can directly target any gene in the person's genome, therefore overcoming many common drug development problems.

"In our study, we showed for the first time that it is possible for CRISPR technology to be used in cancer therapy, however CRISPR is a unique approach that could potentially be used for treating any disease that is caused by genetic mutations. The speed at which we are now able to make specific changes in the DNA will also accelerate basic research discoveries in the lab," Dr Herold said.

More than 50 research groups from around Australia have sought Dr Herold's expertise and are working with the laboratory to adapt the technology for their own research.

Dr Herold said CRISPR was a very new technology with many advantages over existing tools. "CRISPR is a rapid, easy and efficient technology with the best results for genome editing," he said.

"In addition to its very exciting potential for disease treatment, we have shown that it has the potential to identify novel mutations in cancer-causing genes and genes that 'suppress' cancer development, which will help us to identify how they initiate or accelerate the development of cancer.

"The technology dramatically shortens the time frame for fundamental research, allowing us to speed up the discoveries that could be translated

to better diagnostics and treatments for the community."

Provided by Walter and Eliza Hall Institute