Tiny Gold Spheres Improve Efficiency of CRISPR Gene Editing

This finding may eventually make HIV gene therapies more affordable and accessible.

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Researchers found that using microscopic gold spheres to deliver the CRISPR gene-editing mechanism to blood stem cells proved more efficient than the traditional use of a deactivated virus as the transport mechanism. This finding may lead to gene therapies for HIV and inherited blood disorders that are less expensive and more accessible to people in need around the world.

“As gene therapies make their way through clinical trials and become available to patients, we need a more practical approach,” said the new study’s senior author, Jennifer Adair, PhD, an assistant member of the Clinical Research Division at Fred Hutchinson Cancer Research Center in Seattle. “I wanted to find something simpler, something that would passively deliver gene editing to blood stem cells.”

To prompt cells to accept CRISPR gene-editing tools, researchers must deliver a small electric shock. This can damage cells and even kill them. Additionally, when an experimental treatment requires precise editing of a cell’s genes, this demands the engineering of additional molecules to properly deliver editing tools—an extra burden that increases the overall time and cost needed to complete such an effort.

Publishing their findings in Nature Materials, Adair and her colleagues determined that the optimal size of their gold sphere was 19 nanometers in diameter—about one billionth the size of a grain of table salt. They packed onto these gold particles a host of gene-editing tools, including CRISPR plus either of two proteins that act as scissors that cut the cellular genome, Cas12a or the more commonly used protein Cas9.

Next, the scientists isolated blood stem cells—those cells that give rise to all blood and immune cells. In laboratory experiments, they found that it took just six hours for the cells to take up the fully loaded gold nanoparticles, without the need for an electric shock. The gene editing was evidently occurring within the cells within 24 to 48 hours. Cas12a proved better than Cas9 at delivering very precise edits to cells.

The researchers then injected the edited stem cells into mouse models, finding that the gene-editing effect peaked eight weeks later. The edited cells remained in the mice 22 weeks after the

injection. There were also edited cells in the animals' bone marrow, spleen and thymus. This finding is an indication that a retreatment with more edited cells perhaps would not be necessary.

Further research is required to increase the level of gene editing seen in the cells. In this current study, about 10% to 20% of the stem cells successfully received the edits to their genes. The goal is to achieve a cell-edit rate of 50% or greater in order for gene therapies for HIV or inherited blood disorders to stand a good chance of success.

To read a press release about the study, click here.

To read the study abstract, click here.

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