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Targeted Gene Therapies

Gene therapy offers potential treatments for numerous diseases including sickle cell disease, hemophilia, cystic fibrosis, and enzyme deficiencies. Gene therapies attempt to insert useful genes into an organisms existing genetic material, or genome. Many standard methods insert the gene in a random part of the organism's genomes; this process can interrupt useful genes and increase the risk of cancer. Our experiment seeks to address this issue by attempting to target the insertion of the new gene into a specific, safer, part of the genome. I have built a DNA construct that will be specially packaged to target liver cells in a mouse model. The DNA construct itself contains a mobile genetic cargo containing the gene we want to insert, a gene that allows that cargo to move, and a region that specifically associates with a part of the organism's genome. That region is what may provide the ability to target gene insertion. After the animals receive the gene therapy, we analyze the insertions sites compared to a control and several other constructs to test the theory. This method of targeting can lead to new lines of research and could eventually reach patients with diverse medical problems.



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