Gene Therapies

May 2019 Rare-Ed: Rare Genomic's Rare Disease Education Campaign

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EXON SKIPPING

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Exon skipping is a method of copying only some of a gene's information into messenger RNA (mRNA) to bypass harmful mutations as if they are typos.

To do this, splice sites are chemically shielded in ways that enable an mRNA to form while ignoring a mutation, or altering how the RNA or protein folds. An "antisense" molecule binds to a specific sequence in the mRNA to provide the shielding.

The antisense molecule keeps the cell from including the exon with the mutation when it is reading the genetic instructions, thus restoring the reading frame. Skipping the exon with the mutation results in a shorter but "in-frame" set of RNA instructions, leading to a shorter, but still relatively functional protein

GENE INACTIVATION

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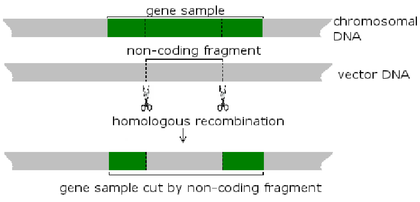
Gene Inactivation is a genetic therapy that involves using RNAi (RNA interference) to silence gene expression.

This silencing can be achieved by (1) using short interfering RNAs (siRNAs)

(2) transcription of short hairpin RNAs (shRNAs) from expression vectors.

RNAi effect is capable of discriminating between the wild-type and mutant alleles, and remains local to the target sequence.



CAR T CELL THERAPY

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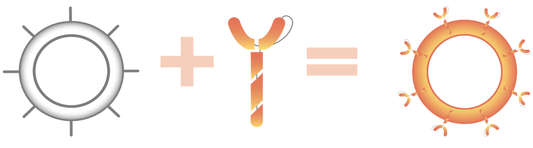
CAR T cell therapy is created by adding a new receptor (or hook) to a patient's T cells. This receptor is called a chimeric antigen receptor (CAR) and transforms the T-Cell into a Cart T Cell. The new CAR T cells work within the body to find their match on specific cells, which include normal cells and cancer cells allowing therapies to be made specific to the patient.



T CELL
A key fighter in your immune system

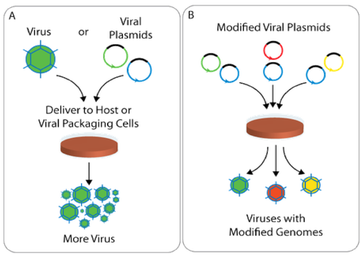
CAR
A specific receptor is added to your T cell

CAR T CELL
The T cell with the CAR added helps find and fight specific targeted cells

VIRAL VECTORS

VIRAL VECTORS

Viruses are used vehicles to carry 'good' genes into a human cell. First, the genes in the virus that cause disease are removed. Then the genes are replaced with genes encoding a desired effect (for instance, insulin production in the case of diabetics).



Sources:

Exon Skipping: https://www.mda.org/quest/article/exon-skipping-dmd-what-it-and-whom-can-it-help
Cart-T Cell Therapy: https://www.explorecarttherapy.com/about-CAR-T-therapclid=EAIaIQobChMInaSt8P_D4gIVj47ICh1iqQ0EEAAYAiAAEgIArvD_BwE
Viral Vectors: http://www.genetherapynet.com/viral-vectors.html
Gene Inactivation: https://www.researchgate.net/profile/Slawomir_Orzechowski/publication/271052389/figure/fig1/AS:295097993318400@1447368252120/Gene-inactivation-by-homologous-recombination.png