The Use of CRISPR to Treat Duchenne Muscular Dystrophy

Duchene muscular dystrophy (DMD) is a genetic disease that leads to the degradation of muscle cells over time, currently affects around 1 in 5,000 boys, and has average life expectancy is 26.

CRISPR offers a potential cure through genetic modification.

What is CRISPR?
- CRISPR is a system of proteins that function as an adaptive immune response in bacteria against viruses.
- A protein called Cas9 and RNA segments target viral DNA, cutting it, and duplicating select parts as a ‘memory’ of past infection.
- This image below shows the process for a typical CRIPSR immune response.
- The CRISPR system used for genetic modification includes Cas9, RNA segments, and a DNA template to modify specific parts of a genome.

Current Research
- Currently there are no human trails for curing DMD with CRISPR but experiments with mice and human stem cells are promising.
- The image below shows dystrophin levels in CRISPR treated mice and shows how only a small percentage of corrected cells can created dystrophin levels close to normal mice.
- Human stem cells have been successfully cured of DMD, however the cells must then be reintroduced to the body and nearly all parts of the body since muscle cells are 40% of ones body weight.

Curing DMD
- CRISPR is a promising cure for DMD as the disease is caused by a deletion mutation in exon 44 (exons are the function parts of the gene).
- Since DNA is read in sets of three, a deletion mutation ruins every subsequent set of three. A small point mutation is perfect for CRIPSR to target and change.
- Additionally more deletions could be made to restore the sets of three creating a still not perfect, but somewhat functioning dystrophin gene.

- CRISPR is not the only method of genetic modification two other methods called TALENs and zinc fingers are also used. Both are manufactured and are custom made to the targeted gene. This makes them less flexible then CRISPR and harder and more expensive to produce.
- CRIPSR is generally more efficient but for some segments of DNA TALENs may prove more efficient then CRISPR.

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<tr>
<th>CRISPR</th>
<th>TALENs and Zinc Fingers</th>
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<td>Simpler to Produce</td>
<td>Are harder to produce and each must be made unique for the targeted gene</td>
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<td>Specifically Cuts between two base pairs</td>
<td>TALENs cleaves anywhere from 12 base pairs</td>
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<td>Up to 70% of desired mutations</td>
<td>Can range between having 1%-50% of desired mutations</td>
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