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Dystrophin Gene Targeting Method and Tools (20140040, Dr. Perlingeiro, Dr. Daniel Voytas)

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Genetic Therapy for Duchenne Muscular Dystrophy and Similar Genetic Disorders

A new method and tool have been developed for the genetic therapy of Duchenne muscular dystrophy (DMD) and related genetic disorders. The dystrophin gene sequence has been genetically corrected by inserting a synthetic exon transcript into the gene code prior to the first exon in the natural coding sequence. The inserted construct ends in a stop signal that keeps transcription of mutated exons from occurring, and is able to successfully code for dystrophin. The inserted synthetic exons bypass the mutated genes and allows for stronger muscle tissue and reversal of muscular dystrophy to occur. This method may also be a viable option for treatment in similar genetic disorders, where the exons that cause the disease are spread out across the megabase.

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Duchenne Muscular Dystrophy

DMD is a recessive genetic disorder that causes muscle degeneration and eventual death through a mutation that makes the body unable to code for the protein dystrophin. In healthy bodies, dystrophin is an integral component to the structure of muscle tissue, and the lack of this protein causes the muscles to break down. Researchers have previously found the specific exons in the genetic code that are responsible for the production of dystrophin, and it is known that it is the mutation of these exons that causes the genetic issues related to DMD. However, the exons are spread across the megabase of the human genome, and it is therefore difficult to correct the DMD locus. There has been little success in genetic treatments to correct the mutation responsible for the protein coding error that causes DMD.

BENEFITS AND FEATURES OF EXON INSERTION IN DYSTROPHIN GENE:

- Exon insertion bypasses mutated genes and allows for proper coding of the dystrophin protein
- Can be applied to other genetic mutations that cause harmful effects in humans
- Method and tool has not been used before in the treatment of DMD

Phase of Development Prototype Development

Researchers

Rita C. Perlingeiro, PhD
Associate Professor, Cardiovascular Division, University of Minnesota Medical School
External Link (www.dom.umn.edu)

Michael Kyba, PhD
Associate Professor, Pediatrics, University of Minnesota Medical School
External Link (www.pediatrics.umn.edu)
Fabrizio Rinaldi, PhD
Antonio Filareto, PhD
Daniel Voytas, PhD
Associate Professor, Director of the Center for Genome Engineering at the University of Minnesota
External Link (cbs.umn.edu)