

Oligonucleotide-based gene therapy for Muscular Dystrophy

Leonidas A. Phylactou

Muscular Dystrophy is a group of inherited diseases with common characteristics the progressive weakness and loss of muscle. Among the most known muscular dystrophies is Duchenne Muscular Dystrophy and Myotonic Dystrophy. Antisense oligonucleotides (AON) are short pieces of nucleic acids which can be designed to target cellular mRNA and inhibit or modulate endogenous gene expression. In our laboratory we design and use AON in order to correct the genetic defects and consequently the disease phenotype of muscular dystrophies. During the presentation, data will be shown which support that AON are good candidates for gene therapy of muscular dystrophies.