Splice-switching oligonucleotide therapeutics is new method for editing gene transcript

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In splice-switching, an innovative therapeutic approach, targeted oligonucleotide drugs alter the editing of a gene transcript to produce the desired form of a protein. Developments in this rapidly advancing field have already led to promising treatments for such diseases as Duchenne Muscular Dystrophy and spinal muscular atrophy, as described in an article in *Human Gene Therapy*.

In "Development of Therapeutic Splice-Switching Oligonucleotides," Petra Disterer and coauthors from University College London, University of London, and Queen Mary University of London, UK, and Medical University of Warsaw, Poland, present an overview of the many possible therapeutic applications for splice-switching oligonucleotides. The authors discuss the design and chemical modification of these novel compounds to increase their stability and effectiveness, and emphasize the need to develop efficient solutions on a case by case basis.

"This is an emerging therapeutic area with promising clinical results," says James M. Wilson, MD, PhD, Editor-in-Chief of *Human Gene Therapy*, and Director of the Gene Therapy Program, Department of Pathology and Laboratory Medicine, University of Pennsylvania Perelman School of Medicine, Philadelphia.

More information: online.liebertpub.com/doi/full … 10.1089/hum.2013.234