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Antisense Oligonucleotide Approaches to the Treatment of Disease

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Antisense oligonucleotides (ASOs) have proven to be an effective and important therapeutic platform. ASOs are having success clinically and pre-clinically for a number of human conditions, including diseases of the central nervous system, but have not yet been widely explored as a treatment option for addiction. ASOs are short, single-stranded, modified nucleotides that function by base-pairing with the complementary sequence of the targeted RNA and modulating gene expression in a manner that is dependent on the ASO design and targeting site. ASOs can be used to normalize aberrant gene expression associated with disease and also to manipulate normal expression in ways predicted to be therapeutically beneficial. We have employed a number of different approaches to modulate gene expression of disease targets in models of Alzheimer's, Parkinson's and Batten disease as well as cystic fibrosis and Usher syndrome. Each of our antisense approaches is specifically designed to either increase or decrease activity of the targeted gene product. I will present an overview of antisense oligonucleotide technology and its targeting and regulatory capabilities with a focus on our work manipulating gene expression in the central nervous system, general strategies that may also be considered for the development of new treatments for addiction.