GENETICS, **BIOINFORMATICS, AND** SYSTEMS BIOLOGY COLLOQUIUM



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IONIS PHARMACEUTICALS

ANTISENSE OLIGONUCLEOTIDES (ASOS) FOR THE TREATMENT OF NEUROLOGICAL DISEASES

Antisense oligonucleotides (ASOs) have emerged as a viable therapeutic modality for treating previously intractable diseases of the central nervous system (CNS). RNA therapeutics, like ASOs, can target often undrugable, genetically validated, disease modifying targets. ASOs delivered to the cerebral spinal fluid modulate their target RNAs throughout the CNS. The advancements, opportunities, challenges, and lessons learned in the development of ASOs for the treatment of neurological diseases will be discussed.

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