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NUSINERSEN, NEWER DRUG FOR SPINAL MUSCULAR ATROPHY

"NUSINERSEN, AN EXON 7 INCLUSION DRUG FOR SPINAL MUSCULAR ATROPHY: A MINIREVIEW"

Abstract

Spinal muscular atrophy (SMA) is an autosomal recessive neuromuscular disease with incidence of 1 in 5000 to 10,000 Live births and is produced by homozygous deletion of exons 7 and 8 in the SMN1 gene. The SMN1 and SMN2 genes encode the SMN protein, a crucial protein for the preservation of motor neurons. Newer drug, Nusinersen, from early infancy has shown improvement in clinical outcomes of SMA patients.

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Abstract. The reduction of survival motor neuron (SMN) protein causes **spinal muscular atrophy** (SMA), an autosomal recessive neuromuscular disease. Nusinersen is an **antisense oligonucleotide,** approved by the FDA, which specifically binds to the repressor within SMN2 exon 7 to enhance exon 7 inclusion and augment production of functional SMN protein. Nusinersen is the first new oligonucleotide-based drug ...

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Nusinersen

Nusinersen, marketed as Spinraza, is a medication used in treating spinal muscular atrophy, a rare neuromuscular disorder. In December 2016, it became the first approved drug used in treating this disorder.

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