THE EFFICACY OF NUSINERSEN TREATMENT IN SPINAL MUSCULAR ATROPHY: A SYSTEMATIC REVIEW

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INTRODUCTION

Spinal muscular atrophy (SMA) is an autosomal recessive neuromuscular disorder, affecting 1 in 10000 live births, of which presentation varies from early infantile death to progressive weakness of the muscles. A therapeutic agent Nusinersen is being used to treat SMA.

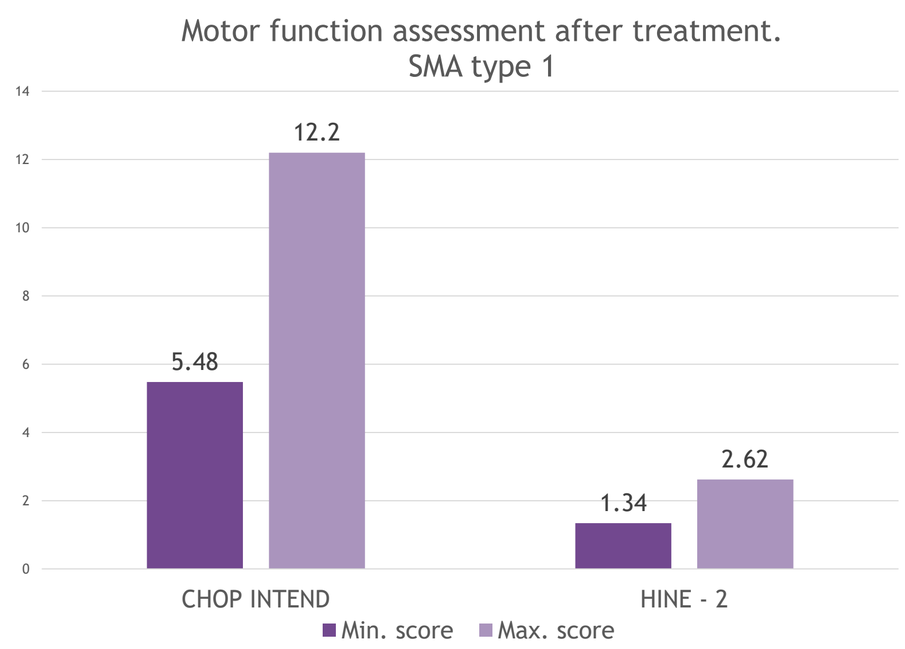
AIM

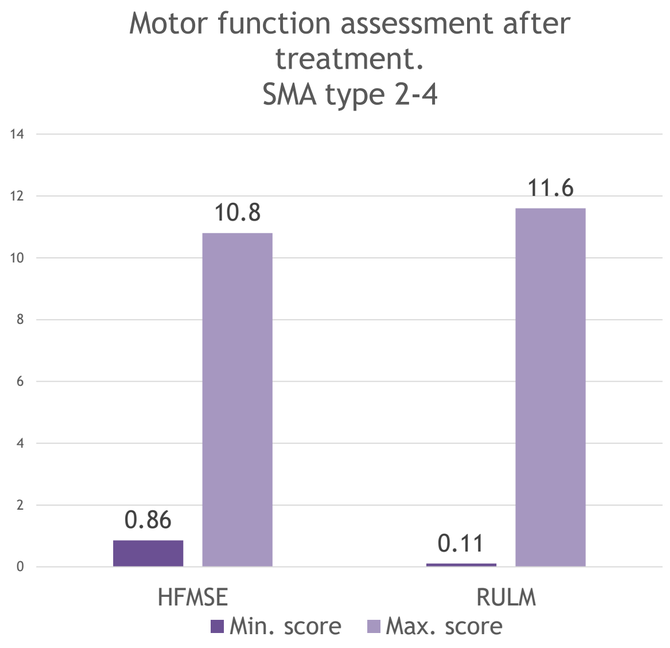
To review the literature analyzing the efficacy of Nusinersen in treating children and adults with SMA.

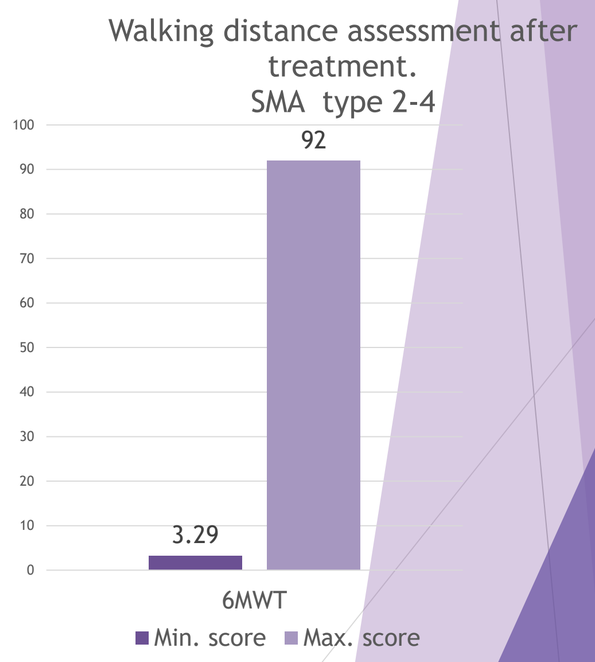
METHODS

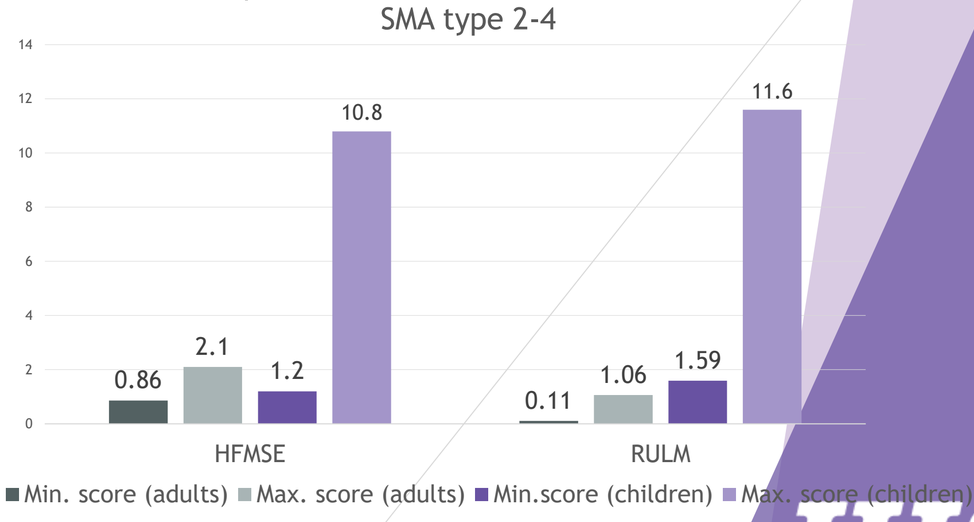
The systematic review was conducted complying with PRISMA reporting standards. 508 articles were identified using key-words: “Nusinersen“, “Spinraza“. After excluding ineligible reports – 23 studies were included in the review.

RESULTS









**Min. score* = average minimum improvement in scale score from baseline to motor function assessment.
**Max. score* = average maximum improvement in scale score from baseline to motor function assessment.

CONCLUSIONS

Nusinersen had a positive effect on the motor function of patients diagnosed with SMA across all types. The best response to medication is exhibited in the youngest children, less apparent in adults.

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