Hope for Spinal Muscular Atrophy Patients

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Introduction

• Spinal muscular atrophy is a genetic disorder of motor neurons, the cause of most forms of SMA is the deficiency of SMN protein.
• full-length, functional SMN protein using genetic instructions carried in the SMN1 gene & identical neighboring gene, called SMN2, typically direct cells to produce a short, partially functional version of the SMN protein.

• The great deal of SMA research is aimed at increasing SMN levels, improving muscle strength and function through development of various strategies.[1]

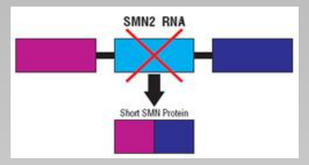
Studies

1. Raising SMN levels through gene therapy.
2. Prenatal transplantation of human amniotic fluid stem cells.
3. Raising SMN levels using antisense oligonucleotides.

Data Analysis

- **Gene therapy**

Scientists have successfully used gene therapy to treat very young mice with SMA-like disease.[2]



- **Stem cells transplantation**

Engrafting enriched neural cells help produce neurotrophic factors that are responsible for the growth of mature neuron cells.[3]

- **Making sense of antisense**

Antisense oligonucleotides are small molecules that can bind to RNA to change how it is spliced. A potential therapy for SMA is to design ASOs that will change the splicing of SMN2 to make more functional SMN protein.[4]

Conclusion

Researches have focused on strategies to increase the body's production of SMN protein, lacking in the chromosome 5-related forms of the disease.
o The approaches include methods to help motor neurons survive in and to maximize the child's independence and quality of life.

Results

- A non-replicating adeno-associated virus capsid is used to deliver a copy of human SMN gene (AVXS-101 provided by Avexis) to the patient's own cells on May.2019.[5]
- Prenatal stem-cell therapy preserves the time window to treat disease in utero with much less cell number.[3]
- FDA on Dec. 23, 2016, approved the antisense therapy Nusinersen (Spinraza) for the treatment of SMA.[4]

References

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