Presentation Title

Author and Co-author names
[1]Department or Division Name, Organisation/Affiliation, City, State, Country

Presenter Name

Note: Abstract Description of what will be discussed during the presentation (about 250 - 500 words)

Abstract

Statement of the Problem: Neuromuscular disorders such as Spinal Muscular Atrophy (SMA) and Amyotrophic Lateral Sclerosis (ALS) pose significant challenges due to their genetic basis and limited therapeutic options. Recent breakthroughs in gene therapy have opened new horizons for targeted treatments.

Methodology: This study reviewed data from clinical trials conducted between 2020–2024, focusing on CRISPR-based editing, antisense oligonucleotides (ASOs), and viral vector-mediated gene replacement therapies. Patient outcomes, safety profiles, and biomarker responses were analyzed.

Findings: Results demonstrate significant improvements in motor function scores among SMA patients treated with viral vector therapies, while ASO treatments in ALS showed delayed disease progression in early-phase trials. CRISPR-based approaches remain in exploratory phases but show promise in preclinical models.

Conclusion & Significance: Gene therapy holds transformative potential for neuromuscular diseases, though challenges in equitable access, long-term monitoring, and ethical considerations remain key issues for global health systems.

Biography

Dr. Jane Smith is a neurologist and clinical researcher specializing in neurogenetics and advanced therapies for rare neuromuscular diseases. She has published over 80 peer-reviewed articles and serves as a principal investigator in multiple international gene therapy trials.

Certificate Details

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Recent Photograph: (High Resolution)

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