Dr. Sudha Bhattacharya
World Without GNE Myopathy

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

Potential Therapeutic Options For GNE Myopathy: Lessons From Other Muscle Diseases

Sunday, August 28th 2022 • 9am PT

Speaker Series #082822

Dr. Sudha Bhattacharya, World Without GNE Myopathy

Introduction & Summary Slide for:
"Potential Therapeutic Options For GNE Myopathy: Lessons From Other Muscle Diseases"

1- GNE myopathy is a rare genetic disorder that causes muscle wasting due to mutations in the GNE gene.

2- Current efforts to bring treatments for GNE myopathy are centered around gene delivery using AAV vector, and supplementation with the sialic acid precursor, N-acetyl-D-mannosamine (ManNAc). These are our most promising options at present.

3- Other therapeutic options, like the use of cell-based therapies to regenerate muscle tissue, or delivery of mRNA (in place of DNA) to the muscles are being tried for Duchene muscular dystrophy (DMD), a more commonly occurring genetic disease.

4- It is prudent to keep track of these developments as some of these could prove beneficial for GNE myopathy as well.

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FINAL HIGHLIGHTS:

"Potential Therapeutic Options For GNE Myopathy: Lessons From Other Muscle Diseases"

Key Takeaways

- Many technological innovations are in the pipeline for muscle diseases
- Each technology has its advantages and limitations
- Cell-based therapies are giving promising results for DMD and LGMD, but clinical data is still small
- mRNA-based therapies are rapidly progressing and, once developed for DMD, could be simple to adapt to GNEM