

Dr. Francis Rossignol
National Institutes of Health



**GNEM SYMPOSIUM
SPEAKER SERIES**

presented by The Neuromuscular Disease Foundation

ManNAc As A Potential Therapy for GNE Myopathy— Advances And Future Directions

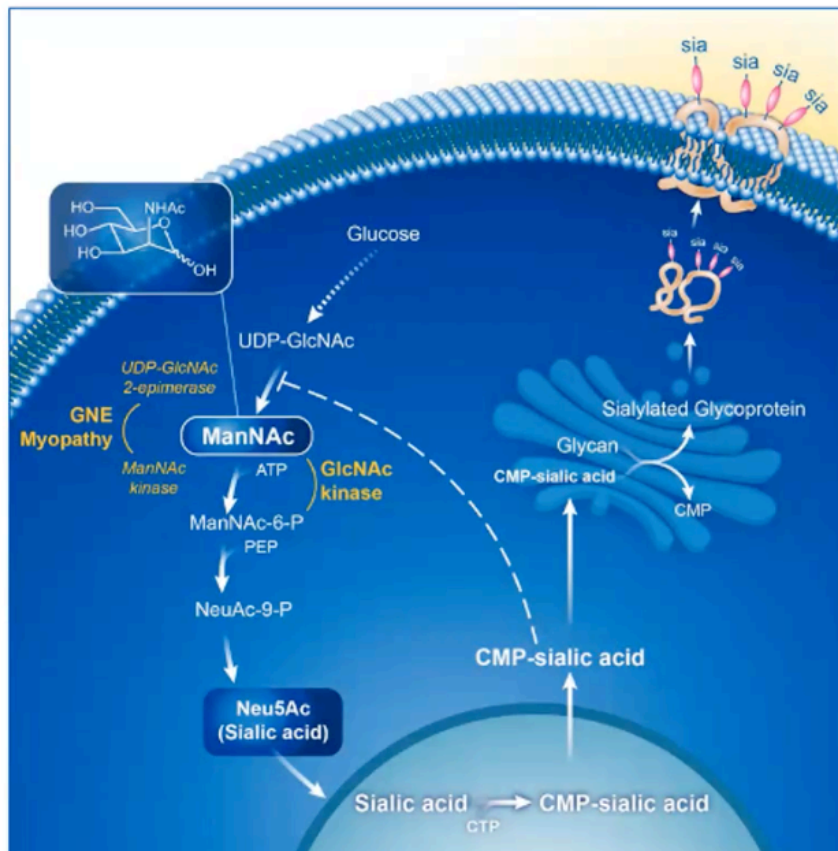
Sunday, December 18th 2022 • 9am PT

Speaker Series #121822

Dr. Francis Rossignol

Introduction & Summary Slide for:

“ManNAc As A Potential Therapy for GNE Myopathy- Advances And Future Directions”



ManNAc is an uncharged precursor of sialic acid

Administration of ManNAc in individuals with GNEM increases sialic acid production (seen in both plasma and muscle), regardless of genetic variants

Clinical data from prior studies suggest efficacy, but more studies are currently ongoing to gather further evidence





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FINAL HIGHLIGHTS:

**ManNAc As A Potential Therapy for GNE Myopathy–
Advances And Future Directions”**

Key Takeaways

1. Innovative study designs developed using natural history data are important to demonstrate changes in outcomes
2. ManNAc in GNEM increases sialic acid production in muscle
3. Further studies of ManNAc are ongoing to address the question of clinical outcomes

