

# **GNEM SYMPOSIUM SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*



"NN109-MaGiNE trial: Background  
and updates in the time of COVID-19"

June 12th 2020  
12pm PDT/3pm EDT

David Kelments  
Clinical Trials Project Manager II at the  
Neurological Clinical Research Institute,  
Massachusetts General Hospital



Speaker Series #61220

**David Klements, Neurological Clinical Research  
Institute (NCRI)**

Introduction & Summary Slide for:  
NN109-MaGiNE trial: Background and updates in the  
time of COVID-19

**An Overview of what to expect in this talk:**

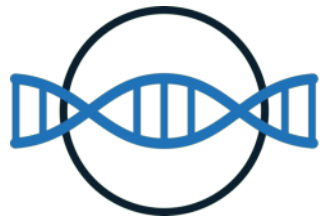
Intro:

- 1. What is your relationship to NDF?** I have been working for the past two years to oversee the development and initiation of this clinical trial to test the use of ManNAc in people diagnosed with GNE Myopathy.
- 2. What we will learn from this presentation today?** Today, I will share some background on the ManNAc trial, and an update on the impacts of COVID-19 and our plans for the future.
- 3. How does this study fit in with our bigger scientific mission?** We hope that this trial will provide solid evidence to support whether ManNAc is a helpful therapy for people with GNE Myopathy, to help support the potential development of this treatment.



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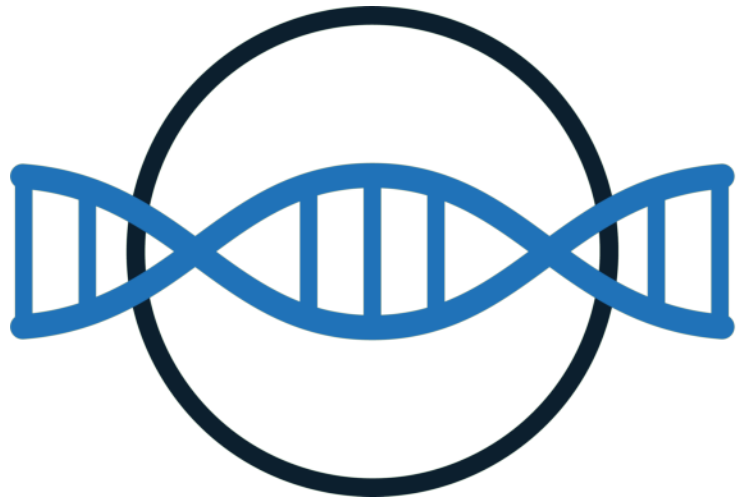
David Klements, Neurological Clinical Research Institute (NCRI)

NN109-MaGiNE trial: Background and updates in the time of COVID-19

- **Key Takeaways:**

- Final slide:

- ManNAc has been studied in early phase trials, and shows promise as a potential treatment to slow the progression of GNE Myopathy.
- This larger trial (NN109-MaGiNE) hopes to provide more definitive answers about the effectiveness of this treatment, to support its development if the study shows positive results.
- The study team, clinical sites, and industry sponsor have put in a great deal of effort in the past 2 years to build a robust trial, which will give us the best possible chance to answer the question of whether ManNAc is a useful treatment for GNEM.
- Although the COVID-19 pandemic has slowed our timetable to begin enrollment, we are closely monitoring the situation at our sites around the country, and standing by to begin enrolling when it is safe and feasible to do so.



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