

## **GNEM SYMPOSIUM SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*



Dr. Nuria Carrillo  
NDF Chief Medical Office

“GNE Myopathy: Two Decades of  
Progress – Much Accomplished,  
Much Still to Do”

Friday September 25, 2020  
9am PDT/12pm EDT



NEUROMUSCULAR  
DISEASE FOUNDATION

Speaker Series #92520  
**Dr. Nuria Carrillo, NDF Chief Medical Officer**  
Introduction & Summary Slide for:  
**“GNE Myopathy: Two Decades of Progress – Much  
Accomplished, Much Still to Do”**

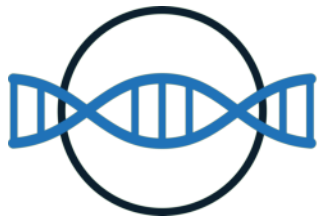
**An Overview of what to expect in this talk:**

Intro:

1. As NDF’s Chief Medical Officer, one of my goals is to expedite genetic therapies for GNE Myopathy.
2. I will give an overview of scientific advances since the discovery of the gene, including current research programs, highlighting those needed for a gene therapy program.
3. Help us identify further research needed to advance gene therapy to clinical trials.



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- **Key Takeaways:**

- Final slide:

- 1. Significant advances have been made since the discovery of the gene in 2001. However, more scientific resources and preclinical studies are needed to support gene therapy clinical trials.
- 2. NDF-funded studies are addressing some of these needs.
- 3. We will identify scientific priorities for 2021 to facilitate the development of gene therapy for GNE Myopathy.