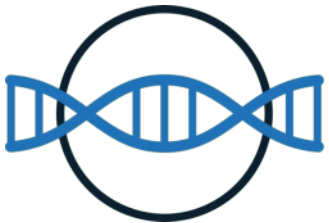


Welcome to our Series  
Please turn off your cameras and mute your audio  
We will get started shortly



**GNEM SYMPOSIUM  
SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*



Dr. Carmen Bertoni  
NDF Scientific Director

“Progress toward developing a cure  
for GNE Myopathy:  
Building a strong program toward  
achieving a common goal”

Friday August 28, 2020  
9am PDT/12pm EDT



NEUROMUSCULAR  
DISEASE FOUNDATION



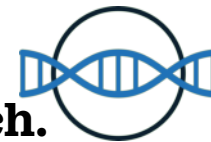
Speaker Series #82820

**Dr. Carman Bertoni, NDF Scientific Advisor**

Introduction & Summary Slide for:

**“Progress Toward Developing a Cure for GNE Myopathy:  
Building a strong program toward achieving a common goal”**

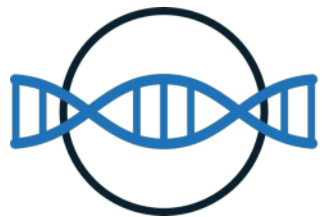
- 1. The presentation will explain the major goals that NDF has set toward the development of an effective treatment for GNE Myopathy**
- 2. Details will be provided on:**
  - Basics of the disease;
  - Scientific strategic approach used to identify key aspects that need to be tackled and better understood;
  - Projects funded by NDF to address the lacunas in the field;
  - Goals and expectations for the upcoming years.
- 3. The aim is to provide a better understanding of the “Big Picture” and the role of NDF in supporting preclinical and clinical research.**



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**“Progress Toward Developing a  
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## **Key Takeaways:**

**Many promising approaches at the horizon;**

### **What we are working on:**

- I. Identify therapeutic approaches and targets likely to achieve sustained beneficial effects in muscle;
- II. Establish/enrich biobanks and patient registries to understand disease progression;
- III. Generate robust animal models to optimize therapy;
- IV. Identify biomarkers to be used to assess efficacy;
- V. Identify valid outcome measures to assess efficacy.

***NDF will continue to work non-stop for the  
community until a cure is found!***