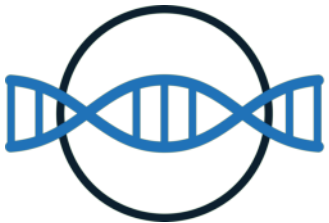


Welcome to our Series  
Please turn off your cameras and mute your audio  
We will get started shortly



**GNEM SYMPOSIUM  
SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*



Dr. Monkol Lek  
&  
Katherine Koczwara



"Research Update on Collected  
GNEM Patient Samples"

June 26, 2020  
12pm PDT/3pm EST



NEUROMUSCULAR  
DISEASE FOUNDATION

# Introduction – (Who, What, Why)

- **Who (are we?)**

- Monkol Lek – Scientist funded by NDF to develop a patient biobank for the GNE research community
- Katherine Koczwarra – PhD student (at Yale) working on this project

- **What (you will learn?)**

- Patient biobank and what goes into it
- How patient biobanks can further our understanding of disease
- Discover new biomarkers
- Develop and test therapies on patient samples



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# Introduction – (Who, What, Why)

- **Why (are we doing this?)**
  - Biomarkers are important to determine if a therapy is working, particularly in slower progressing diseases!
    - RNA sequencing and other characterization of patient cells has the potential of discovery of new sensitive biomarkers.
  - Rapid way of testing gene editing approaches
    - Gene editing approaches are still new and require many rounds of optimization
    - Current applications limited to only correcting cells for research purposes

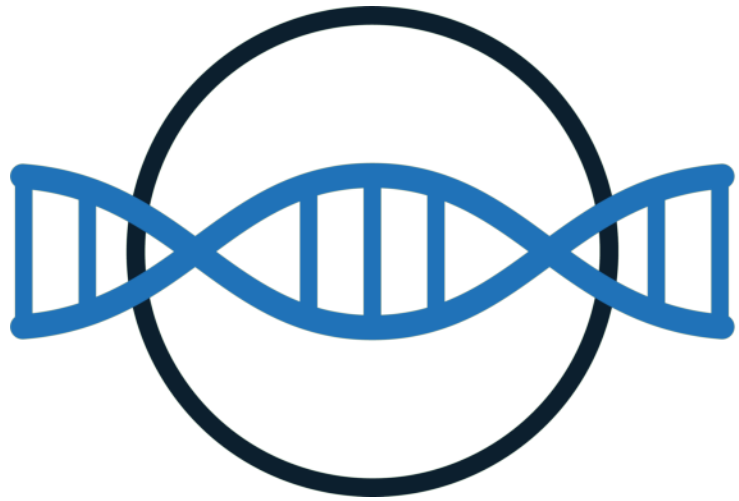


# Key takeaways

1. Developing patient biobanks takes time but is a valuable resource.
2. Biobanks allow for the discovery of genetic biomarkers
3. Rapidly test different therapeutic approaches
4. Patient samples can drive the development of customized therapies



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