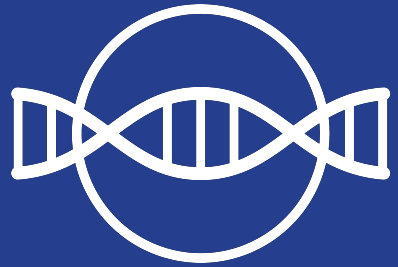


An Overview of what to expect in this talk:

- Summary of GNE myopathy research field
- NDF-funded research projects





GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

Speaker Series #60421

Dr. Angela Lek, Yale University, Lek Lab

FINAL HIGHLIGHTS:

NDF- Sponsored GNE Research Projects

Key Takeaways

- Two main avenues of therapy: gene correction, enzyme supplementation
- Disease modeling: development of a GNE muscle-specific knock-out mouse model
- Importance of assays to measure function of GNE and other biomarkers

