



GNEM SYMPOSIUM SPEAKER SERIES

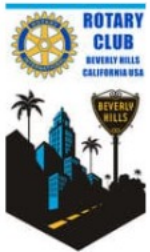
presented by The Neuromuscular Disease Foundation



Katherine Koczwara
Lek Lab, Yale University

"Translational GNE Myopathy Research in Cell Models"

Friday, August 20th 2021 • 10am PST



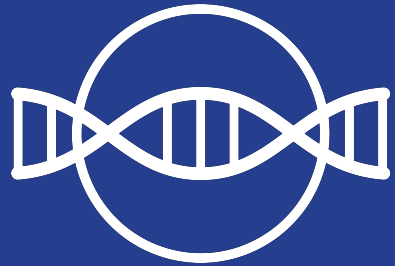
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**Introduction & Summary Slide for:
"Translational GNE Myopathy Research in Cell Models"**

An Overview of what to expect in this talk:

- GNE myopathy background
- Research Aims
 - Develop robust cell model systems
 - Develop reliable assays or disease biomarkers
 - Further our understanding of disease mechanism
- Concluding remarks





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Speaker Series #82721

Katherine Koczwara, Lek Lab, Yale University

FINAL HIGHLIGHTS:

“Translational GNE Myopathy Research in Cell Models”

Key Takeaways

- Cell model systems are a useful tool for GNE myopathy research and therapy development
- *In vivo* assays to measure sialic acid biosynthesis allow us to determine the efficacy of potential therapies
- Additional research into GNE expression levels and pathogenic variants can provide insight into best therapeutic strategies

