





## "Update on Model Systems for GNE Myopathy"

Friday, July 30th 2021 • 10am PST





## Speaker Series #73021 Dr. Stella Mitrani-Rosenbaum Introduction & Summary Slide for: "Update on Model Systems for GNE Myopathy"

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

- Why do we need model systems to develop GNE Myopathy treatment
- Update on Cellular models
- Update on Animal models
- These projects are supported by NDF





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Dr. Stella Mitrani-Rosenbaum

FINAL HIGHLIGHTS:

"Update on Model Systems for GNE Myopathy"

## **Key Takeaways**

- We have developed a Gne KO muscle cell lineage
- We have developed a muscle Gne KO mouse lineage
- These novel tools will allow:
- to further understand Gne functions in muscle
- to define novel biomarkers
- to assess treatment evaluation

