

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation



Prof. Stella Mitrani-Rosenbaum
Hadassah-Hebrew
University Medical Center

"From Gene to Therapy: Challenges in
GNE Myopathy-The Need for Animal
Models"

June 5th 2020
9am PDT/12pm EDT



NEUROMUSCULAR
DISEASE FOUNDATION

Speaker Series #6520
Dr. Stella Mitrani-Rosenbaum, Hadassah
Introduction & Summary Slide for:
From Gene to Therapy: Challenges in GNE Myopathy
The need for animal models

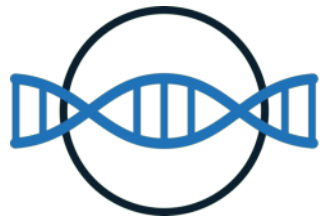
An Overview of what to expect in this talk:

1. GNE has specific roles in MUSCLE
2. Development of Animal models essential to evaluate any treatment
:
Muscle conditional mouse
3. Development of Gne KO zebrafish
4. These studies are supported by NDF grants



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Introduction & Summary Slide for:

**“From Gene to Therapy: Challenges in GNE
Myopathy**

“The need for animal models”

- **Key Takeaways:**

Final slide:

1. GNE has specific roles in MUSCLE
2. Animal models are essential for the full understanding of these functions and for monitoring any potential treatment on muscle pathology. Without animal models, no treatment can be evaluated.
3. Since no animal model which recapitulates GNE Myopathy symptoms is available, and since GNE KO mice are embryonically lethal, muscle conditional Gne KO mice and Gne KO zebrafish could provide useful tools for these purposes.