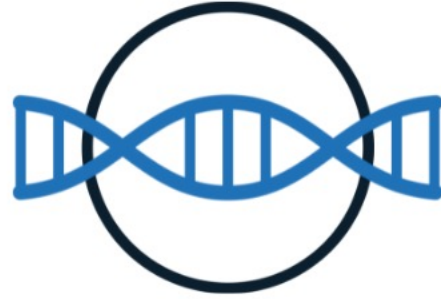




Dr. Stella Mitrani-Rosenbaum
Hadassah-Hebrew
University Medical Center



**GNEM SYMPOSIUM
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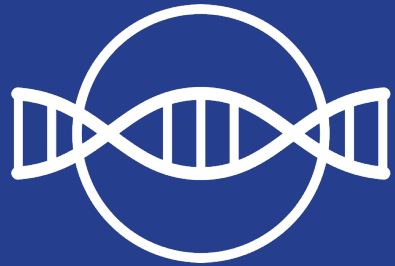
GNE Myopathy Treatment: Developing An Animal Model

Sunday, June 26th 2022 • 9am PT

Animal Models for GNE Myopathy

- ✓ Zebrafish Gne KO model
- ✓ Mouse Gne KO model
- ✓ ProDGNE European Consortium





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Speaker Series #62622

Dr. Stella Mitrani-Rosenbaum

FINAL HIGHLIGHTS:

“GNE Myopathy Treatment: Developing An Animal Model”

Key Takeaways

1. We have developed a zebrafish *gne* KO model that shows clear muscle defects, thus, ready to be used for treatment evaluation.
2. We have developed a muscle *Gne* KO mouse we are now characterizing for muscle defects
3. We have developed a muscle/liver *Gne* KO mouse we are now characterizing for muscle defects.

The availability of these new tools are uncovering novel pathways involving GNE, leading to a better understanding of GNE functions, to the definition of novel biomarkers for therapy assessment

