

## **GDEM SYMPOSIUM SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*

### "Building the Tools to Develop and Optimize a Gene Therapy Program for GNE Myopathy"



#### **Dr. Paul Martin**

Friday September 11, 2020  
9am PDT/12pm EDT

Principal Investigator, Center for Gene Therapy  
Nationwide Children's Hospital



Speaker Series #91120

**Dr. Paul Martin, Principal Investigator  
Nationwide Children's Hospital**

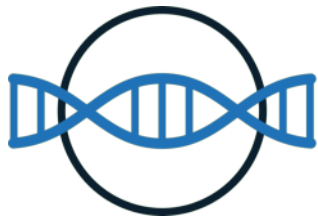
Introduction & Summary Slide for:  
**Building the Tools to Develop and Optimize a  
Gene Therapy program for GNE Myopathy**

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

- 1. What is your relationship to NDF? A.** We have been funded by NDF, beginning in June of 2020, to perform experiments on GNE gene therapy.
- 2. What we will learn from this presentation today? A.** Learn about how AAV works as a gene therapy. **B.** Learn about the potential of gene therapy to treat muscle diseases. **C.** Learn about research on GNE gene therapy potency assays. **D.** Learn about new GNE bicistronic AAV vector technologies.
- 3. How does this study fit in with our bigger scientific mission?**  
**A.** Development of GNE AAV vector potency assays will speed gene therapy development and optimization.



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Introduction & Summary Slide for:

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- **Key Takeaways:**

- 1. What did you show/teach us? A.** Gene Therapy has enormous potential to treat GNE myopathy. **B.** A number of steps must still be accomplished to bring gene therapy to patients. **C.** New gene therapies to prevent disease while building new muscle strength are being developed.

- **2 . What problems have you solved so far? A.** We are developing cell line and *in vivo* potency assays to describe GNE activity derived from AAV gene therapy vectors.

- **3. What's next A.** Optimize AAV.GNE potency assays, **B.** Characterize new second generation gene therapy vectors.