Dr. Julie Crudele
University of Washington

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

Developing An AAV Gene Therapy For GNEM

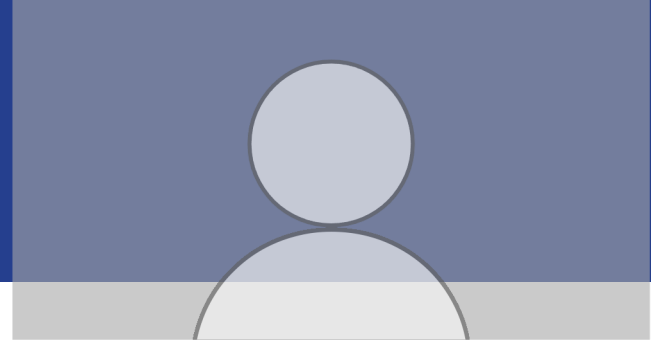
Sunday, September 25th 2022 • 9am PT

Speaker Series #092522

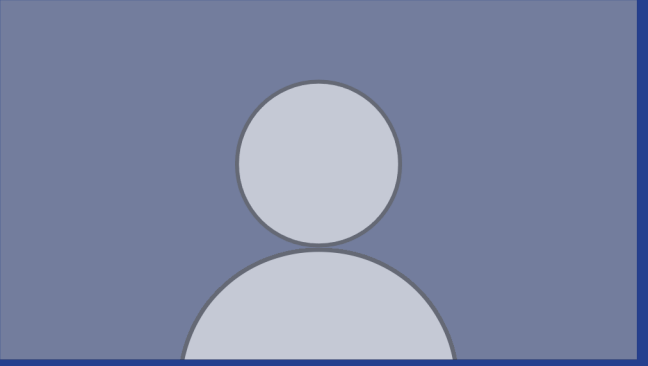
Dr. Julie Crudele, University of Washington

Introduction & Summary Slide for:

"Developing An AAV Gene Therapy For GNEM"



- AAV gene therapy for GNE myopathy
  - Promoter development
  - AAV serotype screening



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FINAL HIGHLIGHTS:

“Developing An AAV Gene Therapy For GNEM”

Key Takeaways

1. We have designed a promoter that has dual liver and muscle expression to be used for AAV gene therapy for GNE myopathy
2. We have confirmed that AAVMyo can be used at 10x lower doses than AAV9 to treat body-wide muscle disease
3. We are now testing AAVMyo-TEC4.8-GNE1 in mice that have mutant GNE