



GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

"Evaluating GNE Myopathy Gene Therapy: Movement towards a Biomarker"

Friday, June 18th 2021 • 10am PST



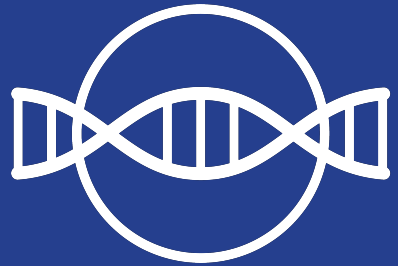
Dr. Kelly Crowe
Mount St. Joseph University



Sponsored by: Rotary Club of Beverly Hills

- **Dr. Crowe is funded by the NDF to study biomarkers for GNEM gene therapy**
- **Dr. Crowe will be talking about her lab’s work on biomarker discovery for GNEM gene therapy**
- **Biomarkers are a critical component of moving towards a gene therapy for GNEM**





GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

Speaker Series #61821

Dr. Kelly Crowe, Mount St. Joseph University

FINAL HIGHLIGHTS:

**“Evaluating GNE Myopathy Gene Therapy:
Movement towards a Biomarker”**

Key Takeaways

1. Finding a reliable biomarker is critical for assessing a GNEM gene therapy
2. Several lectins have shown promise in detecting changes in sialic acid levels
3. We will continue testing our candidate lectins in models of GNEM

