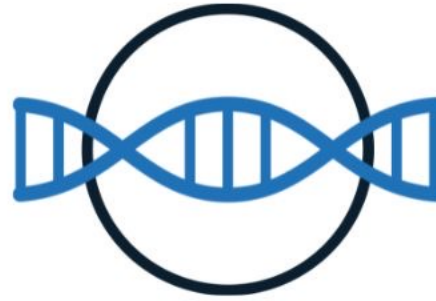




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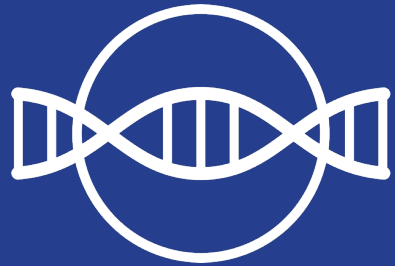
**GNEM SYMPOSIUM  
SPEAKER SERIES**

*presented by The Neuromuscular Disease Foundation*

# Activation of Endogenous Mutated GNE Product by Small Compound

- **Activation of GNE**
  - Continuous sialic acid supply
  - Effective to **treat GNE myopathy**
- Introduction of **new approach** to find **small molecules** which **activate GNE enzyme**
- **Funded by the NDF**





## GNEM SYMPOSIUM SPEAKER SERIES

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NDF GNEM Speaker Series

**Dr. Wakako Yoshioka-** National Center of Neurology and Psychiatry (NCNP), Japan

### **FINAL HIGHLIGHTS:**

“Activation of Endogenous Mutated GNE Product by Small Compound””

### **Key Takeaways**

1. Activation of GNE/MNK kinase would enable continuous supply of sialic acid which can provide better treatment.
2. *In-silico* screening is an efficient approach to find activator from known compounds.
3. Stabilization of oligomerization by small compounds have potential to be new therapeutic strategy.

