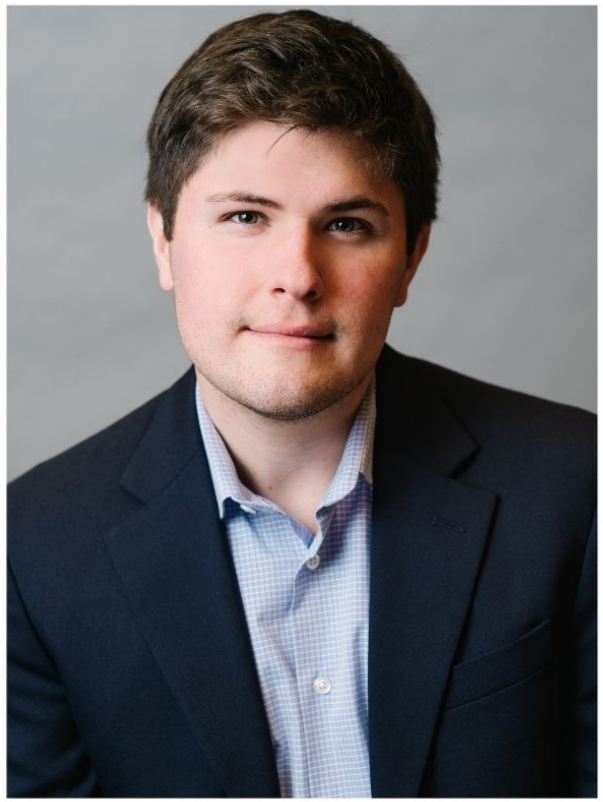

Rich Horgan
Founder & President
Cure Rare Disease

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

Engineering a Gene Therapy for Monogenic Rare Diseases

Friday, June 11th 2021 • 10am PST

Speaker Series #61121

Rich Horgan, Founder & CEO Cure Rare Disease

Introduction & Summary Slide for:
Engineering a Gene Therapy for Monogenic Rare Diseases

Agenda

- Brief overview of N=1 therapeutic development for Duchenne muscular dystrophy
- Translating the gene therapy framework to develop a potential therapy for GNE myopathy

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FINAL HIGHLIGHTS:

Engineering a Gene Therapy for Monogenic Rare Diseases

Key Takeaways

• CRISPR technology is a form of gene therapy with the potential to change DNA to correct faulty genes

•Researchers have demonstrated the correction of other genetic genetic diseases with CRISPR Technology

•The development of a gene therapy framework has the potential to help other monogenic diseases.

• Our preliminary results show that we correct the GNE gene