Speaker Series #5820

Dr. Monkol Lek & Dr. Angela Lek, Yale University

Introduction & Summary Slide for:

Exploring the potential of gene editing as a potential therapeutic for GNE Myopathy.

An Overview of what to expect in this talk:

- Using gene editing (such as CRISPR Cas 9) as a potential therapy for GNE myopathy
- Gene-editing is a form of gene therapy and it offers the potential to correct patients' GNE mutations.

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

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FINAL HIGHLIGHTS:

Exploring the potential of gene editing as a potential therapeutic for GNE Myopathy.

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

•Gene editing provides an alternative option to gene gene replacement therapy

•CRISPR technology is more feasible in targeting common mutations in GNE Myopathy.

• Our preliminary results show that we can edit the GNE gene