



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.

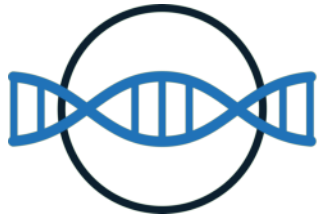
An Overview of what to expect in this talk:

- 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.



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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 diseases with CRISPR Technology
- Gene editing provides an alternative option to 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