

GNEM SYMPOSIUM SPEAKER SERIES

presented by The Neuromuscular Disease Foundation

"Development of therapy for GNE myopathy: Possibilities and Global Access"




Dr. Alok Bhattacharya

Friday August 21, 2020
9am PDT/12pm EDT



NEUROMUSCULAR
DISEASE FOUNDATION



Speaker Series #82120
Dr. Alok Bhattacharya,
Introduction & Summary Slide for:
**"Development of Therapy for GNE myopathy:
Possibilities and Global Access"**

An Overview of the Talk

What do patients expect from drug developers

Current status

Drug development process

Potential approaches towards GNE myopathy drug development

The way forward

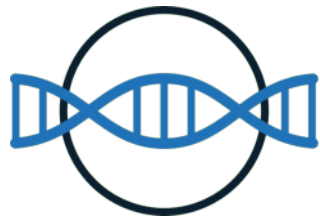
Situation in developing countries and accessibility to drugs

Suggestions for making drugs accessible



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Conclusion

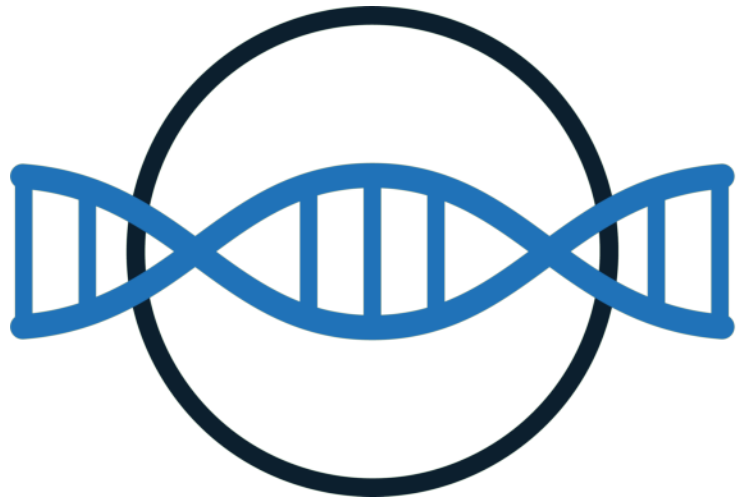
At present there is only one potential therapy in the horizon that can reduce progression of the disease, but not stop, in the upper part of the body

We need to increase our effort towards understanding the pathobiology of the disease, thereby, increasing prospect of finding an effective therapy

Meanwhile, we need to keep working towards developing gene and cell based therapies, and small molecule screens, that do not require understanding of the disease.

We also need to find ways to make the drugs accessible to everyone in the world

Patient organisations, such as NDF and WWGM could bring all stakeholders in the quest for finding a cure available to everyone.



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