



Developing Therapies in Rare Neuromuscular Disease

A Symposium by The Advisory Committee for Therapeutics

Boston

31st October 2024

Welcome to the Developing Therapies in Rare Neuromuscular Disease

Dear Colleagues and Friends,

Welcome to the inaugural symposium by the TREAT-NMD Advisory Committee for Therapeutics, Developing Therapies in Rare Neuromuscular Disease.

Over the past 15 years, TACT has convened over 80 meetings, providing expert advice and guidance to academic and industry drug developers that have de-risked countless NMD therapeutic programs across various disease areas. To celebrate this remarkable journey and share the invaluable knowledge gained during this time, we are honored to present this special event.

This Masterclass offers a unique opportunity to learn directly from the most experienced and knowledgeable members of the TACT committee. Each expert will bring their unique perspective and expertise, enriching our collective understanding of NMD therapeutic development.

For individuals involved in developing NMD treatments, this symposium presents a valuable opportunity to gain valuable insights and strategic guidance to help chart a course towards success.

On behalf of the TACT committee and the entire TREAT-NMD community, we extend our warmest regards.

With sincere gratitude,

Annemieke Aartsma-Rus

(Chair, TREAT-NMD Advisory Committee for Therapeutics)



Programme

09:00-09:15	Introduction and Overview Annemieke Aartsma-Rus PhD & Volker Straub PhD, MD
09:15-09:45	Learnings and FAQs
09:45-09:55	Q&As from audience Volker Straub PhD, MD & Annemieke Aartsma-Rus PhD
9:55-10:15	Patient Voice
10:15-10:20	Q&As from audience Lindsay Alfano PT, DPT, PCS / Sharon Hesterlee PhD
10:20-10:40	Coffee and Networking break
10:40-11:10	Pre-Clinical learnings
11:10-11:20	Q&As from audience Annemieke Aartsma-Rus PhD
11:20-11:50	Standard Operating Procedures & Good laboratory practices
11:50-12:00	Q&As from audience Kanneboyina Nagaraju PhD
12:00-12:30	Ultrarare disease challenges
12:30-12:40	Q&As from audience Brian Tseng, PhD
12:40-13:10	Outcome Measures
13:10-13:20	Q&As from audience Lindsay Alfano PT, DPT, PCS
13:20-14:10	Lunch
14:10-14:30	Trial Design
14:30-14:40	Q&As from audience Chris Weihl MD, PhD
14:40-15:10	De-risking Priorities/Statistic Models
15:10-15:20	Q&As from audience Heather Gordish-Dressman PhD
15:20-15:40	Coffee and Networking break
15:40-16:10	Regulatory
16:10-16:20	Q&As from audience Pavel Balabanov, MD, PhD
16:20-17:00	Key Areas for review from TACT Experience
17:00-17:15	Q&As from audience Mike Kelly PhD



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