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October 29-31, 2018 | Boston, MA, USA
www.nmd-summit.com

NMD
Neuromuscular Drug Development

Solving the Challenges in Developing Second Generation Neuromuscular Therapeutics

21 Expert Speakers Including:

Greg La Rosa
Chief Scientific Officer,
Senior Vice President,
Rare Disease Research & Development
Pfizer

Brian Kaspar
Chief Scientific Officer
AveXis

Rajeev Sivasankaran
Head Rare Diseases,
Neuroscience Division
Novartis

Meg Wood
Director of Development
CureDuchenne

Brian Bronk
Head of External Innovation & Rare Diseases
Sanofi

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hansonwade

Welcome to **Neuromuscular Drug Development**

Built with **Pfizer, Roche, PTC Therapeutics and 17 other expert companies** the inaugural industry-focused Neuromuscular Drug Development summit (NMD) will **solve the challenges preventing drug developers bringing truly transformative therapeutics to market.**

This summit is the first of its kind, uniting thought leaders in neuromuscular drug development to discuss their challenges and opportunities, with the aim to transform and accelerate the field.

With an emphasis on ALS, SMA, DMD and other rare diseases, this discussion-led forum will enable you to:

- Deepen your understanding of the exciting advancements in **gene therapy** and **how this can be used in combination to increase efficacy**
- Define **clinically meaningful endpoints** to enhance efficacy in clinical trials
- Overcome barriers limiting your **patient access** leading to larger patient populations in clinical trials

Join your peers at this definitive forum to exchange novel ideas, gather lessons learned and create new connections. Don't miss your opportunity to **be a part of the force accelerating the change in the wider NMD landscape.**

Testimonials from World CNS Summit, a sister Summit:

"I was impressed by many of the speakers and learnt a significant amount, which I was able to bring back to our discovery team"
Eisai

"One of the best CNS therapeutics meetings to attend"
P2D Bioscience

"Essentially every speaker was excellent. A very informative and thought provoking series of presentations"
National Institute Neurologic Disease & Stroke

Your Top 5 Reasons to Attend NMD

1. Explore the use of biomarkers and defined endpoints to improve clinical trial design to **avoid failure** to **progress to the next stage of clinical development with** Summit Therapeutics

2. Maximize the data available to **overcome patient access** resulting in **larger studies with** Sarepta

3. Delve into the evolving landscape of neuromuscular clinical trials and discuss issues **defining clinically meaningful endpoints** with Columbia University

4. Discuss the use of gene and cell therapies in neuromuscular diseases and their potential benefit in combination with drugs to **improve efficacy** and **maximize delivery with** AveXis

5. Examine novel targets and mechanisms of action for **necessary efficacy** in developing next generation NMD therapeutics **with** Novartis

21 Expert Speakers

Greg La Rosa
Chief Scientific Officer, Senior Vice President, Rare Disease Research & Development
Pfizer

Brian Kaspar
Chief Scientific Officer
Avexis

Rajeev Sivasankaran
Head Rare Diseases
Novartis

John Tinsley
Chief Scientific Officer
Summit Therapeutics

Brian Bronk
Head of External Innovation & Rare Diseases
Sanofi

Salvador Rico
Vice President, Clinical Development
Audentes

Nikolai Naryshkin
Executive Director
PTC Therapeutics

Gregory Petsko
Chief Scientific Officer
MeiraGTx

Eva Chin
Director
Cytokinetics

Daniel Cohen
Chief Executive Officer
Pharnext

Chikwendu Ibebunjo
Senior Scientist
Novartis

Kim Long
Senior Scientist
Scholar Rock

Sanjay Rakhade
Senior Director, Medical
Sarepta

Meg Wood
Director of Development
CureDuchenne

Jason Zhang
Director, Biology
Wave Life Sciences

Fred Cohen
Chief Medical Officer
Strongbridge Bio

Jinsy Andrews
Director of Neuromuscular Clinical Trials
Columbia University

Lale Welsh
Chief Executive Officer
Neuromuscular Foundation

Joanne Donavon
Chief Medical Officer, Senior Vice President, Clinical Development
Catabasis Pharmaceuticals

Alon Ben-Noon
Chief Executive Officer
NeuroSense Therapeutics

Niren Murthy
Department of Bioengineering
University of California, Berkley

"The quality of talks was high and the discussion very interesting"
University of Rome

Conference Day One | Monday, October 29

Greg La Rosa
Chief Scientific Officer, Senior Vice President, Rare Disease Research & Development
Pfizer

8.30 Chair's Opening Remarks

Fred Cohen
Chief Medical Officer
Strongbridge Bio

8.40 Overview of the Investigational Landscape in Neuromuscular Drugs
- Review the current drug development landscape, with a focus on drugs in clinical stage
- Identify themes across program characteristics, drug targets and technologies
- Summarize disease areas of high unmet need not being addressed

Evaluating the Challenges & the Future of Gene Therapy for Neuromuscular Disorders

Greg La Rosa
Chief Scientific Officer, Senior Vice President, Rare Disease Research & Development
Pfizer

9.10 Gene Therapy in Duchenne Muscular Dystrophy
- Duchenne Muscular Dystrophy is a devastating muscle degenerative disease caused by the loss of function mutations in the muscle structural protein dystrophin
- Dystrophin, being the largest known human gene, poses problems for gene delivery via AAV given the limits this delivery vehicle has in packaging gene payloads
- Using shortened version of the dystrophin coding region, animal models studies, and now human clinical trials have begun to show promising results

Brian Kaspar
Chief Scientific Officer
Avexis

9.40 Translation of Gene Therapeutics in Neurological & Neuromuscular Diseases
- This talk will cover basic and translational studies that have led to Phase 1/2 clinical trials for first in human studies in Spinal Muscular Atrophy
- Understand dose ranging studies to determine dose and preclinical efficacy
- Safety studies to enable first in human studies

10.10 Speed Networking

This session is a great opportunity to introduce yourself to the attendees that you would like to have more in depth conversations with. This session is the ideal opportunity to get face-to-face time with many of the brightest minds working in the neuromuscular drug development field and establish meaningful business relationships.

11.00 Morning Refreshments

Exploring Preclinical Models for Improved Translatability into Clinical Trials

Kim Long
Senior Scientist
Scholar Rock

11.30 Selective Inhibition of Myostatin Activation is Beneficial in Mouse Models of SMA Therapy
- SRK-015 is a monoclonal antibody that specifically inhibits activation of myostatin
- Inhibition of myostatin activation significantly increases muscle mass and function in multiple mouse models of SMA
- Inhibition of myostatin activation improves bone phenotypes in SMA mice

Chikwendu Ibebunjo
Senior Scientist
Novartis

12.00 Improving the Translational Validity of Animal Models of Disease

- Less than 12% of all drugs (<5% for CNS drugs) evaluated in clinical trials make it to registration in part because efficacy in animal models did not translate to the clinic
- Although some of these failures in translation reflect unforeseen intrinsic differences between the model and humans, others could have been avoided by systematic evaluation and validation of the model
- This presentation will review classic and atypical criteria to increase the translational validity of animal models of diseases including neuromuscular diseases

12.30 Lunch & Networking

Identifying & Validating the Role of Biomarkers & How they can be Used to Enhance Clinical Trial Design

John Tinsley
Chief Scientific Officer
Summit Therapeutics

2.00 Development of New Muscle Biomarkers to Support Functional Endpoints in Neuromuscular Disease

- Scientific rationale for choosing appropriate muscle biomarkers in DMD
- Use of nonclinical and clinical biomarker data to support future biomarker use
- Use to support functional endpoints in trials

2.30 Think Tank Roundtable Sessions

More practical and highly interactive breakout roundtables where attendees can crowd-source solutions and share opinions around pre-assigned topic areas. A valuable chance for attendees to unite around hot topics and debate best practice. No more sitting quietly, this is a dedicated opportunity for you to voice your experiences and identify unique solutions.

3.00 Moderator Feedback & Audience Debate

Moderators will be assigned to each roundtable to facilitate discussion and collate the findings. Following the roundtables, they will present back to the entire delegation as part of a panel discussion for information dissemination.

3.30 Afternoon Refreshments

Addressing the Limited Population Size in Neuromuscular Diseases

Meg Wood
Director of Development
CureDuchenne

4.00 Building a Robust Strategy to Overcome Limited Patient Access to Clinical Trials

- The 'right' way to communicate with small but mighty patient communities
- Methods to increase reach into patient communities through innovative strategic partnerships

Sanjay Rakhade
Senior Director, Medical
Sarepta

4.30 Clinical Development for Rare Disease Therapies: Making Trials Compelling for Key Stakeholders

- Unique challenges in designing clinical trials for orphan disorders
- Balancing scientific questions and trial constraints
- Integrating patient/ caregiver feedback esp. in pediatric studies

5.00 Chair's closing remarks

Scientific Poster Session

After the formal presentations have finished, the learning and networking carries on. The Poster Session is allows you to connect with your peers in a relaxed atmosphere and continue to forge new and existing relationships. During this session scientific posters will be presented on the latest advancements in next generation therapeutics, preclinical models and identification of biomarkers in neuromuscular drug development.

Conference Day Two | Tuesday, October 30

Greg La Rosa
Chief Scientific Officer, Senior Vice President, Rare Disease Research & Development
Pfizer

8.30 Chair's Opening Remarks

Investigating Combination Therapies & Overcoming Various Challenges that Comes with Them

Daniel Cohen
Chief Executive Officer
Pharnext

8.40 Showcasing the Natural History of PXT 3003, a Pleodrug for CMT1A

- PXT 3003 is a synergistic combination of 3 drugs each at a 1/10 of approved doses in their canonical indications
- The various paths from inception to approval will be over viewed: discovery, clinical, regulatory IP and pricing
- Pleotherapy is a fast, safe and efficient approach that can be easily be combined with other therapeutic means. It will therefore have an important impact in the treatment of neuromuscular disorders

Salvador Rico
Vice President, Clinical Development
Audentes

9.10 Gene Therapy Clinical Development Program for X-Linked Myotubular Myopathy (XLMTM), a Rare, Severe Neuromuscular Disease

- Describe the unique challenges in designing a gene therapy development program for a rare neuromuscular disease
- Rationale of the clinical development program for AT132 (AAV8-Des-MTM1) in XLMTM
- Present interim efficacy and safety data of ASPIRO, a phase 1/2 clinical trial to assess AT132 in XLMTM

Brian Bronk
Head of External Innovation & Rare Diseases
Sanofi

Eva Chin
Director
Cytokinetics

Salvador Rico
Vice President, Clinical Development
Audentes

9.40 Panel Discussion: Exploring the Benefits and Challenges of Combining "Next Generation" & Existing Therapeutics

- The idea of a using a 'cocktail' drug and the challenges Surrounding this
- Combining drugs can enhance drug efficacy and delivery
- What is the future of this and how can we improve this technique

10.10 Morning Refreshments & Networking

Reviewing Novel Targets & Mechanisms of Action for the Development of Neuromuscular Therapeutics

Rajeev Sivasankaran
Head Rare Diseases
Novartis

11.00 Developing a Potential Therapy for an Orphan Neuromuscular Disease

- Phenotypic drug discovery
- Looking at mechanism of action studies
- Sharing preclinical studies and clinical path

Nikolai Naryshkin
Executive Director
PTC Therapeutics

11.30 Evolving Therapeutic Approaches for Treatment of Neuromuscular Disorders

- The potential of small molecules for therapeutic targeting of neuromuscular disorders
- Targeting SMN2 splicing for treatment of spinal muscular atrophy
- Targeting mutant IKBKAP splicing for treatment of familial dysautonomia

Eva Chin
Director
Cytokinetics

12.00 Fast Skeletal Muscle Troponin Activators as Treatments for Neuromuscular Diseases
- Reldesemtiv is a fast skeletal troponin activator that increases muscle force production
- Reldesemtiv increases muscle function in preclinical models of SMA and ALS
- Reldesemtiv improves 6 min walk time and respiratory function in adults with SMA

12.30 Lunch & Networking

Reviewing Novel Targets & Mechanisms of Action for the Development of Neuromuscular Therapeutics

Niren Murthy
Department of Bioengineering
University of California, Berkley

1.30 In vivo Delivery of Cas9 Ribonucleoprotein & Donor DNA with Gold Nanoparticles
- Nonviral delivery of Cas9, gRNA and donor DNA to muscle tissue and gene correction via HDR
- Nonviral delivery of Cas9 and gRNA to the brain and gene editing in the striatum and hippocampus, as a treatment for autism
- New delivery strategies for Cas9 RNP

Jason Zhang
Director, Biology
Wave Life Sciences

2.00 Stereopure Exon Skipping Approach for the Potential Treatment of DMD
- Wave's chemistry platform allows precise design, optimize and manufacture stereopure oligonucleotides
- Stereopure molecules significantly enhanced the efficiency of exon skipping comparing to regular randomers, with mechanism to be discussed
- In vivo NHP target engagement has been achieved and clinical trial well underway

2.30 Afternoon Refreshments

Exploring How to Ensure You Receive a Return on Investment Within Neuromuscular Drug Development & Patient Advocacy Perspective

Lale Welsh
Chief Executive Officer
Neuromuscular Foundation

3.30 Rethinking Nonprofits & Patient Advocacy
- Providing the NFP view on neuromuscular drug development
- How patient advocacy can be rethought

Alon Ben-Noon
Chief Executive Officer
NeuroSense Therapeutics

4.00 ALS Drug Re-purposing - Upsides & Downsides of the Business Model
- Does successful development promise an investment?
- Drug pricing and reimbursement
- Incentives for investment are greater than ROI

Joanne Donavon
Chief Medical Officer, Senior Vice President, Clinical Development
Catabasis Pharmaceuticals

Brian Bronk
Head of External Innovation & Rare Diseases
Sanofi

4.30 Panel/Round table: Looking into Payment Models to Ensure Commercial Success when Bringing Neuromuscular Therapies to Market
- Validating methods to decide where the next exciting investment lies
- How to ensure commercial success
- Lessons learned from failures

5.00 Chair's closing remarks

Post-Conference Workshop Day | Wednesday, 31 October

Workshop A

Evolving landscape of neuromuscular clinical trials

8:00am – 11:00am

New approvals of therapy, the development of gene directed therapy and changing regulatory landscape have created challenges and opportunities to conducting clinical trials. In this workshop, attendees will discuss key issues in conducting clinical trials in neuromuscular diseases including:

- Current clinical trial landscape
- Share perspectives on clinical trial recruitment hurdles
- Clinical Trial Design
- Discuss issues defining clinically meaningful endpoints

Workshop Leader

Jinsy Andrews
Director of Neuromuscular Clinical Trials
Columbia University

Workshop B

Exploring New Approaches to The Treatment of Neuromuscular Diseases Starting from Genetic & Biochemical Studies in a Model Organism

12:00pm – 3:00pm

Gene therapy is a very exciting approach for the treatment of neuromuscular diseases, but not without its challenges.

Attendees will discuss in detail:

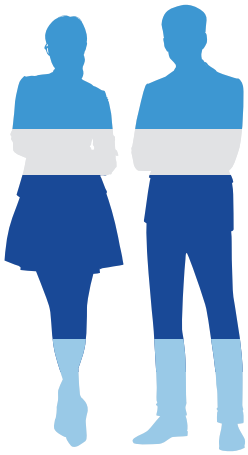
- An unbiased genetic screen in a model organism can identify therapeutic possibilities that could never be discovered by work in animals or even human cells
- ALS is not a single disease; there are several sub-types and therefore treatment of ALS will require precision medicine
- Gene therapy has considerable advantages over other approaches for the treatment of ALS and probably all neurodegenerative diseases

Workshop Leader

Gregory Petsko
Chief Scientific Officer
MeiraGTx

Partnership Opportunities

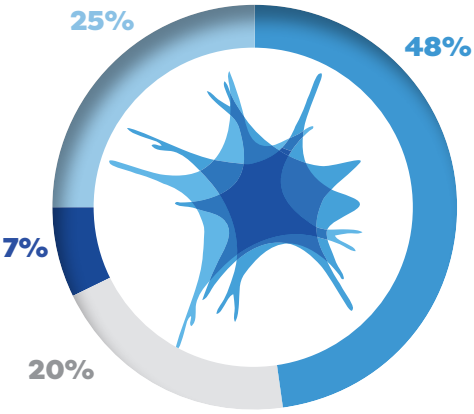
Audience Seniority*



- C-Level: 26%
- VP: 10%
- Senior Director/ Director: 40%
- Scientist: 24%

*Based on previous events' statistics (World CNS)

Attending Companies*



- Drug Developers
- Solution Providers
- NFP & Government Organizations
- Academics & Research Institutions

*Based on previous events' statistics (World CNS)

Companies that Attend Hanson Wade Events

abbvie, Boehringer Ingelheim, Eisai, gsk, Johnson & Johnson, Lundbeck, MERCK, NOVARTIS, Otsuka, Pfizer, Roche, Shire, Takeda, TEVA

WHY SPONSOR

Partnering with NMD is your opportunity to demonstrate your expertise in neuromuscular drug development and elevate your company's standing and influence on the community.

Your brand, your message, your reputation showcased in front of the leading minds of neuromuscular drug development.

This forum will be a unique platform for you to demonstrate how your company can enable drug developers overcome the barriers preventing the development of truly curative neuromuscular therapeutics. This could involve speaking on the agenda, hosting networking sessions or exhibiting. **We'll work with you to build a bespoke partnership** to ensure that you meet your 2018 business objectives.

Get in touch today to learn more about how we can support your business objectives within the neuromuscular field. Email us at **sponsor@hansonwade.com**

Get Involved Today

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Ready to Register?

1. Hear from patient advocacy groups to understand how to reach a wider patient audience and gain the patient perspective
2. Gain an understanding of the clinical landscape of neuromuscular diseases and the exciting future of the field
3. Learn from industry leaders and the lessons they have learned during their neuromuscular drug development

3 Easy Ways To Register

www.nmd-summit.com

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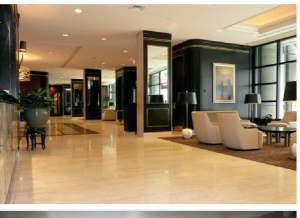
SECURE YOUR PLACE

Package Details	Register & Pay before Friday July 27	Standard Pricing
GOLD Conference + 2 Workshops	$3597 **(save $600)**	$3897 **(save $300)**
SILVER Conference + 1 Workshop	$3098 **(save $400)**	$3398 **(save $100)**
BRONZE Conference Only	$2499 **(save $300)**	$2799
Workshops (each)	$699	

Team Discounts*

- **10% discount – 3 delegates**
- **15% discount – 4 delegates**
- **20% discount – 5 or more delegates**

Please note that discounts are only valid when three or more delegates from one company book and pay at the same time.
Contact: register@hansonwade.com



VENUE

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