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2nd Annual NMD Neuromuscular Drug Development

October 23-25, 2019 | Boston, MA, USA

Developing Second Generation Neuromuscular Therapeutics

Your 18 Expert Speakers Include:

Nicolas Currier
Associate Director, Medical
Biogen

Kenneth Attie
Vice President
Acceleron Pharma

Kirsten Gruis
Global Head of Neuromuscular
Roche

Jane Larkindale
Executive Director, Duchene Regulatory Sciences Consortium
Critical Path Institute

Ralph Kern
Executive Vice President, Chief Operating Officer & Chief Medical Officer
Brainstorm Cell Therapeutics

"Excellent meeting! I learned a lot"

Gayathri Ramaswamy, Associate Director, **Biogen**

Event Partner: The Jackson Laboratory
Leading the search for tomorrow's cures

Tel: +1 617 455 4188 **Mail:** info@hansonwade.com www.nmd-summit.com

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2nd Neuromuscular Drug Development Summit (NMD)

Now in its 2nd year and built with **Biogen**, **Audentes** and **PTC Therapeutics**, the industry-focused **NMD Summit** will overcome the challenges preventing neuromuscular drug developers **bringing truly transformative therapeutics to market**.

With an emphasis on ALS, SMA, DMD and other rare diseases, 18 expert speakers will share their insights through this comprehensive learning and networking forum, enabling you to:

- Explore the approaches being used to treat neuromuscular disorders including **gene therapy, antisense oligonucleotide (ASOs) and small molecules**
- Evaluate the success in the clinical landscape to **determine where improvement is needed**
- Discuss the translational challenges hindering drug developers working on different neuromuscular approaches and **develop strategies to improve translation into the clinic**

Join 90+ other neuromuscular stakeholders at this definitive forum to exchange novel ideas, gather lessons learned and establish new collaborations. Don't miss your opportunity to **be part of the force accelerating change in the wider therapeutic NMD landscape.**

Testimonials from other Hanson Wade CNS events:

"Impressive line-up of industry speakers"

Sean M. Smith, Executive Director, **Merck**

"Very focused meeting, that accomplished a lot in two days. The presentations were high quality and organized into interesting sessions"

Sally Ishizaka, Senior Director, **Eisai**

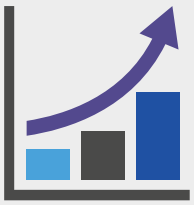
"Excellent networking and learning opportunity for pharma scientists"

Matthew Kennedy, Director, **Merck**

Your Top Reasons to Attend NMD:

Optimize the use of biomarkers and define clinically meaningful endpoints to **improve patient stratification**

Widen your awareness of the exciting progress in the clinical landscape to **benchmark yourself against your competitors**



Address the limited population size and develop strategies to **overcome patient access leading to larger study groups**

Discover the advances in preclinical models and how best to navigate the limitations for **better predictability**

Discuss the regulatory issues surrounding rare disease drug development in order to **improve standard of care**

"Excellent conference with a very good and diverse set of speakers and topics"

Jim Cassella, Chief Development Officer, **Concert Pharmaceuticals**

Your 18 Expert Speakers

Elin Haf Davies
Research Fellow
Bangor University

Eva Chin
Chief Development Officer
NMD Pharma

Jane Larkindale
Executive Director, Duchenne Regulatory Science Consortium
Critical Path Institute

Keith Foster
Chief Scientific Officer
Sutura Therapeutics

Kenneth Attie
Vice President, Medical Research
Acceleron Pharma

Kenneth Rhodes
Chief Scientific Officer
Yumanity Therapeutics

Kirsten Gruis
Global Head, Neuromuscular
Roche

Laurie Conklin
Medical & Regulatory Director
ReveraGen Biopharma

Mathieu Nonnenmacher
Principal Scientist, Vector Engineering
Voyager Therapeutics

Nicolas Currier
Associate Medical Director
Biogen

Rajasekhar Suragani
Director, Preclinical & Translational Exploratory Biology
Acceleron Pharma

Ralph Kern
Executive Vice President, Chief Operating Officer & Chief Medical Officer
Brainstorm Cell Therapeutics

Romesh Subramanian
Chief Executive Officer & Founder
Dyne Therapeutics

Satish Eraly
Medical Director
Biogen

Shazia Ahmad
Director
Patient & Physician Services, UBC

Stuart Peltz
Founder & Chief Executive Officer
PTC Therapeutics

Susan Ward
Head, Clinical Operations
Brainstorm Cell Therapeutics

Thomas Holm Pedersen
Chief Executive Officer
NMD Pharma

Pre-Conference Workshops
Wednesday, October 23

Workshop A
9:00am – 12:00pm

Effectively Utilizing Neuromuscular Biomarkers in Clinical Trials

This workshop will address how to use biomarkers effectively within clinical trials to enrich the patient selection, provide evidence of target engagement and understand which mechanisms reflect an effect and identify responders. Biomarkers can provide additional data to support clinical programs, especially in CNS disorders, that have recently seen a high rate of Phase 3 failures.

By attending this workshop, you will:

- Understand how specific biomarkers interact with your drug/ treatment, including how variables may affect the results (i.e., time of day, processing protocols, gender of subject)
- Learn what processes and training you need in your clinical trial to ensure quality assessments and biomarker results
- Discover what the future holds for novel biomarkers and personalized medicine, including telemedicine and wearable devices and technology

Workshop Leader

Susan Ward
Head of Clinical Operations
Brainstorm Cell Therapeutics

Susan has extensive knowledge of product lifecycle from preclinical to all phases of clinical development, especially in CNS and psychiatric disorders. She has an established track record in the design, leadership, and execution of innovative, clinical development programs. At Pfizer, Biogen and Roche she developed and lead cross-functional teams in translational medicine, biomarkers and complex clinical trials.

Workshop B
12:30pm-3.30pm

Linking the Regulatory and Reimbursement Landscape for Neuromuscular Disorder Drug Development

With the recent approval of multiple neuromuscular therapeutics, understanding of both the regulatory and reimbursement expectations and requirements is essential. The influence of the FDA and EMA on clinical trial design and supporting early approval balanced against the challenges of securing reimbursement will be discussed.

Attend this workshop to:

- Explore what is needed to align your data generation for both regulatory and reimbursement
- Assess the regulatory and economic challenges for neuromuscular therapeutic approval
- Debate the cost of drugs, access and reimbursement

Workshop Leader

Elin Haf Davies
Research Fellow
Bangor University

Elin embarked on a PhD at the Institute of Child Health (UCL) to develop a clinical outcome measure and biomarker of ataxia in children with neuro-metabolic disease and was part of the paediatric team at the EMA, responsible for implementing the Paediatric Regulation in Europe.

> "The event allowed great access to senior people and provided opportunities to collaborate and develop opportunities for further interaction. The speakers provided excellent insight into the state of play in the research areas covered and what still needs to be done. Very worthwhile"
>
> Steven Arnott, Chief Executive Officer, **Perron Institute**

Conference Day One
Thursday, October 24

Nicolas Currier
Associate Medical Director
Biogen

8.00 **Chair's Opening Remarks**

Predicting Future Success for Neuromuscular Drug Development

Nicolas Currier
Associate Medical Director
Biogen

Satish Eraly
Medical Director
Biogen

8.15 **Assessing the Current Clinical Landscape for Neuromuscular Candidates – Is the Future Bright?**

- Antisense oligonucleotides (ASOs) have shown remarkable ability to target motor neurons and as a result efficacy for the treatment of Spinal Muscle Atrophy (SMA)
- Building on the Spinraza success, Biogen is targeting genetically defined ALS with an eye towards high value targets in ALS
- Tools such as radiolabelled ASO, and TE and disease biomarkers such as SOD1, poly(GP), and neurofilament, are helping us de-risk the PK and PD

Kenneth Attie
Vice President, Medical Research
Acceleron Pharma

8.45 **Treating Today's Patients: Moving to Restore Muscle Function in Addition to Slowing Progression**

- Muscle atrophy and weakness are key clinical manifestations of most NMDs, whether myogenic or neurogenic in origin
- Anabolic therapies, such as myostatin inhibitors, have the potential to improve strength and function independent of specific genetic defects
- A locally-acting therapeutic may be well-suited to focal or asymmetric NMDs, while having fewer off-target effects

9.15 **Speed Networking**

This session is the ideal opportunity to meet face-to-face with the key thought leaders working in the neuromuscular field. Specifically designed to connect you with new contacts from the most active companies in the field, the renowned Speed Networking session will be one of the most valuable hours you will spend at NMD.

10.00 **Morning Refreshments**

Translating into the Clinic: Essential Considerations

Laurie Conklin
Medical & Regulatory Director
ReveraGen Biopharma

11.00 **Effectively Integrating Neuromuscular Biomarkers into the Clinic**

- What clinical outcomes are routinely measured in the neuromuscular clinic during care of children with Duchenne muscular dystrophy?
- Which of these have been used as primary outcome measures in drug development trials?
- What biomarkers have been used in drug development and clinically in Duchenne muscular dystrophy?
- Where is this field heading?

Keith Foster
Chief Scientific Officer
Sutura Therapeutics

11.30 **Delivering Antisense Oligonucleotides: Overcoming Translational Hurdles to Enhance Systemic Cargo Delivery**

- Antisense oligonucleotides approaches can be adapted to a wide range of human disease
- Effective delivery strategies of antisense oligonucleotides is urgently required
- Discovering the emerging novel approaches to systemic delivery of antisense oligonucleotides

12.00 **Lunch & Networking**

Preclinical Models of Neuromuscular Diseases: What Can We Expect?

Rajasekhar Suragani
Director, Preclinical & Translational Exploratory Biology
Acceleron Pharma

1.00 Limitations of Preclinical Models: Lessons Learned from Myostatin+ Targeted Therapies for NMD

- Smad2/3 signaling is implicated in negatively regulating muscle mass/growth
- Potential for Myostatin pathway as target for NMD
- Myostatin+ ligand inhibition demonstrates better efficacy in preclinical NMD models
- Endpoints in preclinical NMD models that may/may not translate in patients and clinical trials

1.30 Mastermind: Discussing the Limited Availability & Suitability of Preclinical Models for Rare Diseases & Future Needs

- What preclinical models are currently available and how well do they represent neuromuscular diseases?
- What needs to improve for better predictability of clinical efficacy?
- Are model providers investing enough into developing rare disease models?
- Take this opportunity to share your opinions and benchmark your peers' perspective on the current state of neuromuscular preclinical models available

2.00 Afternoon Refreshments & Networking

Navigating Clinical Trial Design Complications for Neuromuscular Disorders

Ralph Kern
Executive Vice President, Chief Operating Officer & Chief Medical Officer
Brainstorm Cell Therapeutics

3.00 How to Create Exclusion Criteria & Characterize Patient Populations Whilst Maintaining Patient Enrolment Quantity Through Phases

- Learn how to manage clinical heterogeneity in clinical trial populations
- Understand optimal use of inclusion/exclusion criteria
- Determine individual risk stratification using clinical trajectories and biomarkers
- Explore the value of responder analysis

Jane Larkindale
Executive Director, Duchenne Regulatory Science Consortium
Critical Path Institute

3.30 Accelerating Drug Development for Rare Diseases: Development of a Clinical Trial Simulation Tool for DMD

- Discussion of the issues that reduce the efficiency of rare disease drug development, as exemplified by DMD
- Development of a CDISC integrated database of patient-level clinical data from DMD patients, and the value of such a database
- Plans for the development of a clinical trial simulation tool for DMD, data and analysis to date
- The regulatory pathway to approval of this tool
- Discussion of how similar methods could be used to develop tools for other rare diseases and accelerate drug development

4.00 Chair's Closing Remarks

4.15 Scientific Poster Session

The learning and networking continues at the Poster Session, an informal part of the conference agenda, allowing you to connect with your peers in a relaxed atmosphere and continue to forge new, beneficial relationships. You will have the opportunity to present and review presentations displaying new data in neuromuscular research, such as novel targets and advances in clinical development.

Conference Day Two
Friday, October 25

Nicolas Currier
Associate Medical Director
Biogen

8.00 Chair's Opening Remarks

Promising Therapeutic Approaches for Neuromuscular Disorders

Romesh Subramanian
CEO & Founder
Dyne Therapeutics

8.15 Exploring Targeted Delivery of Oligonucleotide Therapeutics to Muscle

- Pioneering targeted therapies for serious neuromuscular disease
- FORCE platform delivers medicines specifically to skeletal, cardiac and smooth muscles
- Demonstrate high potency, specificity and tolerability
- Achieved proof of concept in rodents and primates
- Developing medicines for DM1, DMD and FSHD

Mathieu Nonnenmacher
Principal Scientist, Vector Engineering
Voyager Therapeutics

8.45 Focused Evolution of CNS-targeting AAV Vectors Using Cell Type-Specific RNA Expression

- Blood-brain barrier-penetrant AAV capsids are highly desirable for broad CNS distribution via intravenous injection
- Current AAV vectors do not allow efficient CNS gene transfer following systemic delivery in large mammals
- Voyager Therapeutics developed a new directed evolution platform (TRACER™) combining cell type-specific recovery of AAV capsid libraries and high-throughput enrichment analysis by next-generation sequencing
- Discussing the mouse proof-of-concept experiments determining brain transduction

Kirsten Gruis
Global Head, Neuromuscular
Roche

9.15 Is There Still a Role for Small Molecule Treatments for Neuromuscular Disorders?

- Evaluating the pros and cons of small molecule drug development for rare diseases
- Identifying that small molecules are best positioned when molecular pathophysiology is well understood
- Reviewing two recent small molecule successes in rare disease

9.45 Morning Refreshments & Networking

Supporting Neuromuscular Drug Development Commercial Success

Shazia Ahmad
Director
Patient & Physician Services, UBC

10.45 Patient Advocacy Partnerships to Improve Real World Evidence (RWE) in Neuromuscular Drug Development

- Review and discuss the use of patient advocacy groups in understanding factors in the real world setting, outside of clinical trials, that can help better understand a disease and develop drugs more effectively
- Distinguish the type of information that can be learned from patient advocacy groups that can influence the development of better medications
- Presentation will include panel members who are patient advocates representing neuromuscular diseases such as DMD

Stuart Peltz
Founder & Chief Executive Officer
PTC Therapeutics

Thomas Holm Pedersen
Chief Executive Officer
NMD Pharma

11.15 Panel Discussion: Encouraging Investment into Rare Diseases Research & Securing ROI

- Discussing the cost of drugs, access & reimbursement for rare neuromuscular disorders
- Discovering the collaborations needed for patient treatment success & stakeholders required
- What does an optimal deal structure & pricing scheme look like?

11.45 Lunch & Networking

Improving Drug Development Strategies for Treating Neuromuscular Diseases

12.45 Development of Combination Therapies for Rare Neuromuscular Diseases: The Road Less Traveled

Eva Chin
Chief Development Officer
NMD Pharma

- Rare Neuromuscular Diseases (NMDs) have complex pathologies often without well-defined genetic and molecular causes
- Effective treatment of rare NMDs will require novel therapies targeted at symptomatic treatment as well as disease modifying therapies
- The challenges and opportunities of developing combination therapies will be discussed

1.15 Unbiased Discovery of Novel Targets for ALS & FTD

Kenneth Rhodes
Chief Scientific Officer
Yumanity Therapeutics

- Yeast models of protein misfolding and aggregation offer many advantages for novel target and pathway discovery
- Unbiased phenotypic screening in yeast offers an approach to identification of novel genetic and small molecule modulators of protein folding toxicity
- This presentation will focus on models of TDP-43 and C90rf72 toxicity in yeast and the validation of toxicity modifiers in mammalian cells
- Overall, this discovery strategy has identified several new approaches to disease modification in ALS and FTD

1.45 Afternoon Networking & Refreshments

FDA Approval – Is This Therapeutic Success?

2.45 Discussing the Future of Neuromuscular Therapeutics

Stuart Peltz
Founder & Chief Executive Officer
PTC Therapeutics

- Telling the PTC Story
- Discovering innovation in small molecule treatments
- Rational Gene Therapy approaches
- What's next: The future of NMDs

3.15 Panel Discussion: Assessment of Approval Considerations

Jane Larkindale
Executive Director, Duchenne Regulatory Science Consortium
Critical Path Institute

Ralph Kern
Executive Vice President, Chief Operating Officer & Chief Medical Officer
Brainstorm Cell Therapeutics

Elin Haf Davies
Research Fellow
Bangor University

- Is efficacy or biomarker improvement more important? (DMD vs. SMA approval)
- Aligning clinical expectations with current regulation – what needs to change?
- Supporting clinical scales favoured by clinicians but not recognised by regulation

3.45 Chair's Closing Remarks

"It was a great opportunity to meet like-minded individuals and discuss the common issues that we all face in drug discovery. Really competent speakers and a great overall atmosphere"

Patrick Downey, Director, **UCB**

Partnership Opportunities

Why Partner with NMD?

Partnering with **NMD** is your opportunity to demonstrate your expertise in front of the leading minds in neuromuscular drug development.

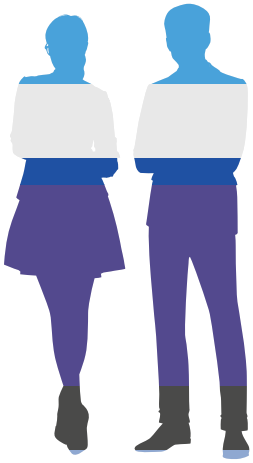
Your brand, your message and your reputation will be showcased to experts from **Biogen**, **Voyager**, **Audentes**, **Roche** and other leading companies in the space as they actively seek partners to help them advance their neuromuscular research.

This forum will be a unique platform for you to demonstrate how your company can enable drug developers to overcome the barriers preventing the development of truly curative neuromuscular therapeutics.

There are a variety of partnership opportunities available and we'll work with you to build a bespoke package to ensure that you meet your 2019 business objectives.

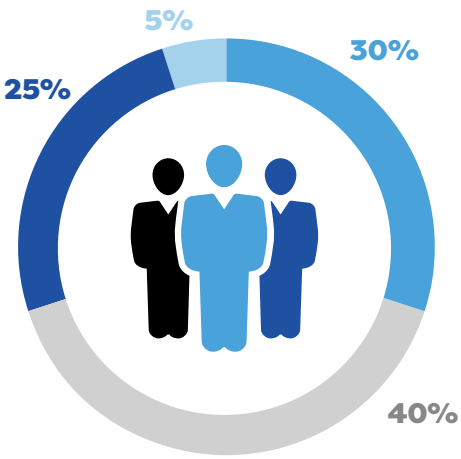
Get in touch today to learn more:
sponsor@hansonwade.com

Audience Seniority



- C-Level: **19%**
- President/VP: **16%**
- Global Head/Head Of: **7%**
- Director: **40%**
- Principal Scientist/ Scientist: **14%**
- Professor: **4%**

Attending Companies



- **Large Pharmaceutical**
- **Small Biotech**
- **Service Providers**
- **Academia**

*Based on market research and previous events' statistics

Event Partner

The Jackson Laboratory
Leading the search for tomorrow's cures

The Jackson Laboratory (JAX) is an independent, nonprofit biomedical research institute combining innovation, reliability, and commitment to customer service to provide the most clinically relevant mouse models and precision services. With an extensive and ever-expanding portfolio of mouse models and services such as target validation and drug efficacy studies, JAX is committed to providing researchers with the most advanced tools to support their neurobiological research and enable efficacious drug design.

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Get Involved

Okeefe Ogholo
Business Development Manager
Tel: +1 617 455 4188
Email: sponsor@hansonwade.com

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3 Easy Ways To Book

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1. Deepen your understanding of the neuromuscular clinical landscape and evaluate the successes of different therapeutic approaches
2. Review the use of patient advocacy groups to understand factors in the real-world setting, outside of clinical trials
3. Learn how to manage clinical heterogeneity in clinical trial populations with optimal use of inclusion and exclusion criteria

Team Discounts*

- **10% discount – 2-3 delegates**
- **15% discount – 4 delegates**
- **20% discount – 5 or more delegates**

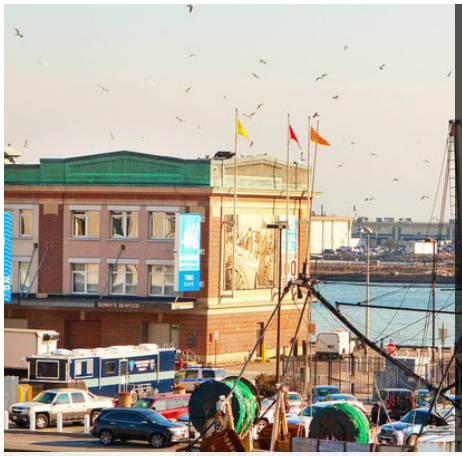
Please note that discounts are only valid when three or more delegates from one company book and pay at the same time.

Discounts cannot be used in conjunction with any other offer or discount. Only one discount offer may be applied to the current pricing rate.

Contact: register@hansonwade.com

SECURE YOUR PLACE

Package Details	Book by Friday, August 16	Standard Prices
GOLD: Conference + 2 Workshops	$3699 **(Save $500)**	$3899 **(save $300)**
SILVER: Conference + 1 Workshop	$3199 **(Save $300)**	$3399 **(save $100)**
BRONZE: Conference Only	$2599 **(Save $200)**	$2799
Workshops (Each)	$699	$699



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Westin Boston Waterfront
425 Summer Street, Boston, Massachusetts
02210 USA

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