WELCOME
SPEAKERS
AGENDA
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REGISTRATION

REGISTER BY FRIDAY, OCTOBER 30 & SAVE UP TO $600

Muscular Disorders Drug Development Summit

Propelling R&D Across DMD, FSHD, DM, LGMD, Pompe Disease & More

January 26-28, 2027 | Boston, MA

Strengthening Translational & Clinical Development of a New Wave of Genetic, Stem Cell, Small Molecule & RNA-Based Muscle Disease Therapies

Your Expert Speakers Include:



Jiyeon Denninger
Medical Director, Clinical Development
Sarepta Therapeutics



Nitza Thomasson
Executive Director, Global Head of Research and Development Neurology
Servier



Jun Shi
Director of Muscle Disease Research
Biogen



Barbora Malecova
Director of Biology
Avidity Biosciences



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi



Tom Natoli
Senior Director, Neuromuscular Research
Dyne Therapeutics

"I'm looking forward to listening, learning and sharing to better serve the muscular dystrophy communities"

Nitza Thomasson, Executive Director, Global Head of Research & Development, **Servier**

Proud to Partner

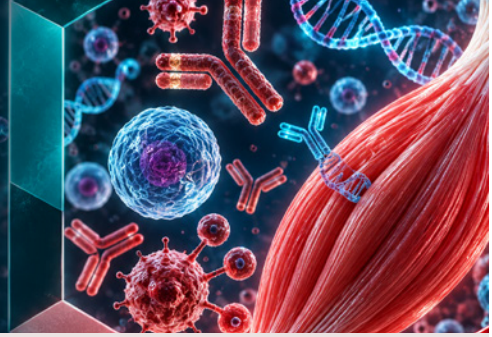
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Uniting the Industry for Cross-Modality Insights Spanning DMD, FSHD, DM & More



After a year marked by uncertainty around **Sarepta**'s **Elevidys**, the field is on the verge of new era shaped by cross-modality innovation and meaningful progress, with FDA submissions anticipated from Regenxbio, Capricor and Avidity that could transform therapeutic options for muscular dystrophies.

With investments including **Servier**'s **$2.65 billion buyout of Edgewise** signposting reignited pharma interest and funding opportunities, muscle disease drug developers are poised to double down on investments across their preclinical, translational and clinical programs in 2026 and beyond.

Take advantage of this timely opportunity to join **60+ industry experts** spanning muscle disease biology, translation, clinical development and more at the **Muscular Disorders Drug Development Summit** for three days of focused collaboration designed to:

- **Explore** emerging modalities beyond gene therapy
- **Discover** lessons learnt from developers of approved drugs
- **Enhance** biomarker measurements of muscular function and activity
- **Innovate** more targeted delivery to specific muscles while avoiding off-target tissues
- **Ensure** clinical trial readiness and leverage real-world data to uncover patient-relevant endpoints

With momentum building into the pipeline, don't miss your chance to learn from **Servier**, **Dyne Therapeutics**, **Edgewise Therapeutics**, **Sanofi**, **Biogen**, **Avidity Biosciences** and more at the only forum dedicated solely to addressing biopharma's most pressing translational and clinical challenges in advancing novel therapies for muscular dystrophies, Pompe disease and related myopathies.

Hear From Your Industry Peers:

"High quality of talks and excellent focus on networking. Good size for discussion and making new connections."

Lundbeck
*7th CNS Drug Delivery Summit Past Attendee

"Very open environment for collaboration and communication. First time I've been to an industry focused event and I was positively impressed."

Athira Pharma
*5th Oligonucleotides for CNS Summit Past Attendee

"This meeting exceeded my expectations. I benefited greatly from both the presentations and the networking opportunities, gaining valuable scientific knowledge and professional insights."

Stoke Therapeutics
*5th Oligonucleotides for CNS Summit Past Attendees

Explore Emerging Modalities for Muscular Disorders R&D

Evaluate the benefits and drawbacks of small molecules, gene therapies and RNA-targeting therapeutics. Consider the future of combination therapies with **Edgewise Therapeutics**, and understand how different modalities can affect immunogenicity, biodistribution and efficacy with **Bonito Biosciences**.

Shape Your Clinical Trial Design with Tested Expert Strategies

Learn how **Sarepta** navigates patient heterogeneity in rare disease trials for **DM1** and **FSHD**, and how **Avidity** begin trial design considerations in the preclinical phase with exon skippers for **DMD**, to increase the likelihood of clinical success.

Learn to Think Like an Investor & Derisk Drug Development

Understand the investor mindset with insights from venture philanthropic organization, **Solve FSHD**, to build confidence in your drug development program, whilst developing stronger investor relationships.

Investigate Novel Biomarkers & Endpoint Selection Tools

Discover emerging biomarkers uncovered by **Dyne Therapeutics** to innovate the delivery of RNA therapeutics and enhance their real-world impact for patients.

Enhance Specificity & Biodistribution in Muscle Delivery

See how **NuCyRNA Therapeutics** is engineering novel targeting technologies to enhance payload delivery for DM1 to overcome challenges with long-term accumulation and toxicity.

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Your Expert Speakers

Muscular Disorders Drug Development Summit
January 26-28, 2027 | Boston, MA

Pharma Thought Leaders



Jun Shi
Director of Muscle Disease Research
Biogen



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi



Nitza Thomasson
Executive Director, Global Head of Research & Development Neurology
Servier



Ricardo Mondragon Gonzalez
Principle Scientist, Muscle Biology & Rare Diseases
Sanofi



Seemin Ahmed
Principal Scientist and Gene Therapy Leader
Novartis

Biotech & Academic Innovators



Barbora Malecova
Director of Biology
Avidity Biosciences



Damon Wang
Chief Executive Officer
NuCyRNA Therapeutics



Domi Stickens
Chief Scientific Officer
Arrakis Therapeutics



Jim Breitmeyer
Chief Executive Officer
Altay Therapeutics



Jimmy Weterings
Vice President Oligonucleotide Therapeutics
Bonito Biosciences



Jiyeon Denninger
Medical Director, Clinical Development
Sarepta Therapeutics



Marla Weetall
Senior Vice President, Pharmacology & Biomarkers
PTC Therapeutics



Matthias Schmidt
Chief Executive Officer
JCR Pharmaceuticals



Natalie Hawryluk
Vice President, Drug Discovery & Early Development
Edgewise Therapeutics



Paige Martin
Associate Scientific Director
C-Path Institute



Sweta Girgenrath
Vice President, Therapeutic Research & External Innovation Lead
Entrada Therapeutics



Thomas Natoli
Senior Director, Neuromuscular Research
Dyne Therapeutics

Impact Investor Perspective



Eva Chin
Executive Director
Solve FSHD

"Good size of meeting to enable networking and robust discussion in the workshops. The pre-conference workshop day enabled a lot of important and thoughtful discussions"

Origami Therapeutics

*5th Annual ALS Drug Development Summit Past Attendee

Pre-Conference Workshop Day
Tuesday, January 26

Morning Coffee & Workshop Check-In — 9.00

Workshop A — 10.00

Moving Beyond Variable & Subjective Muscle Function Assessments: Selecting Endpoints with Clinical Approval in Mind

As the landscape of therapeutic approaches for muscular disorders expands, so must the endpoints used to assess them. Demonstrating meaningful clinical benefit remains one of the biggest barriers to successful approval, and while functional assessments continue to play an important role, sponsors are exploring novel biomarkers, digital measures and disease-specific endpoints that can provide earlier, more sensitive evidence of treatment effect. Attendees will gain practical insights into endpoint selection, validation and implementation from experts navigating today's evolving regulatory landscape.

This workshop will:

- Explore the shortfalls of traditional functional assessments such as the 6-minute walk test and the North Star Ambulatory Assessment to identify the key challenges like variability in a largely heterogeneous patient population
- Discuss aligning endpoint selection with disease biology to demonstrate clinically meaningful benefits
- Discuss novel strategies including digital endpoints to detect treatment effects earlier and generate evidence that supports regulatory decision-making

Workshop Leaders



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi



Marla Weetall
Senior Vice President, Pharmacology & Biomarkers
PTC Therapeutics

Lunch & Networking — 1.00

Workshop B — 2.00

Exploring Combining Modalities for Muscular Dystrophies to Enhance Efficacy Without Increasing Toxicity

As current treatments reach the limit of their therapeutic potential, combination approaches are emerging as a promising strategy to address the complexities of treating muscular disorders. Combining complementary MOAs may enhance efficacy, durability and patient outcomes while minimizing the need for higher doses, limiting toxicity and improving safety. Attendees will learn how leading developers are identifying synergistic combinations and navigating the challenges of demonstrating added benefit while maintaining acceptable safety profiles.

This workshop will:

- Combine complementary therapeutic modalities to target multiple drivers of disease and maximize treatment efficacy
- Utilize translational and clinical evidence to identify synergistic combinations and limit cross-modality interactions that lead to toxicity
- Explore combination therapy successes for neuromuscular diseases: triple drug combination for sporadic ALS

Workshop Leader



Natalie Hawryluk
Vice President, Drug Discovery & Early Development
Edgewise Therapeutics

End of Pre-Conference Workshop Day — 5.00

Conference Day One
Wednesday, January 27

8.00 **Check-In & Light Breakfast**

8.50 **Chair's Opening Remarks**



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi

Overcoming the Complexities of Clinical Trial Design: Derisking Development for Rare Muscular Disorders to Improve the Likelihood of Success

9.00 **Pursuing Accelerated Approval in Rare Diseases: Leveraging Novel Biomarkers & Endpoints to Support Regulatory Decision Making**

- Evaluating the use of validated biomarkers and surrogate endpoints as early indicators of treatment benefit to generate rapid evidence for regulatory bodies
- Exploring regulatory expectations for novel endpoint qualification in rare muscular disorders
- Learning from successful accelerated approval case studies to identify best practices and increase the likelihood of regulatory success



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi
PHARMA THOUGHT LEADER

9.30 **Derisking Drug Development with a Disease-Focused Approach to Gain Investor Confidence for the Evolving Muscular Disorder Landscape**

- How Solve FSHD are positioned as strategic investors to provide early funding, scientific guidance, and credibility that advance pipelines for disease-focused progress
- Building and validating key drug development tools, including prognostic biomarkers, molecular biomarkers, and clinically meaningful outcome measures, to reduce uncertainty and strengthen development pathways
- Demonstrating how foundation-led partnerships can bridge critical funding and knowledge gaps, creating a stronger path from discovery through clinical development for muscular disorder therapies



Eva Chin
Executive Director
Solve FSHD
INVESTMENT INSIGHTS

10.00 **Roundtable Discussion: Statistically Significant vs Clinically Meaningful: Discussing the Shortfalls & Future Direction of Clinical Endpoints for Muscular Disorders**

- Examining the disconnect between statistical significance and meaningful patient benefit in muscular disorder trials to deliver more tangible improvements for patients
- Assessing emerging functional, digital and patient-centered endpoints alongside conventional measures to focus on treatment effects which are relevant to disease burden
- Defining a clinically meaningful change for patients, clinicians and regulators to collaboratively guide endpoint selection and increase confidence in development outcomes



Jiyeon Denninger
Medical Director, Clinical Development
Sarepta

10.30 **Morning Break & Speed Networking**
Join our speed networking session tailored for muscular dystrophy professionals, like yourselves, to connect with fellow industry peers to facilitate rapid yet meaningful exchanges of insights and expertise. Elevate your networking experience during this session designed for impactful connections within the space.

Novel Biomarkers & Endpoints to Strengthen Trial Design & Reduce Late-Stage Failure Risk

11.30 **Using Modeling & Simulation in Duchenne to Build More Efficient, Informative Clinical Trials & Reduce Development Uncertainty**

- Characterizing disease progression in Duchenne using longitudinal natural history and clinical trial data to better understand variability, trajectories, and relationships across clinically meaningful outcomes
- Using disease progression models and clinical trial simulation to inform decisions around endpoint selection, eligibility criteria, study duration, sample size, and other key trial design assumptions
- Applying model-informed drug development approaches to reduce development uncertainty, support more evidence-based decision-making, and strengthen regulatory interactions



Paige Martin
Associate Scientific Director
Critical Path Institute
TRIAL DESIGN INNOVATOR

Conference Day One
Wednesday, January 27

12.00 **Delivering RNA Therapeutics for FSHD: Innovations in DUX4 Silencing & Biomarkers for Clinical Development**

- Exploring the use of RNA therapeutics for the treatment of FSHD to deliver efficacious patient outcomes
- Uncovering novel DUX4 silencing technologies and biomarkers to tackle challenges with patient heterogeneity and asymmetric disease progression
- Strengthening end point selection to reduce trial uncertainty and increase confidence in development decisions



Tom Natoli
Senior Director, Neuromuscular Research
Dyne Therapeutics
NOVEL TECHNOLOGY

12.30 **Lunch Break & Networking**

Advancing Targeted Muscle Delivery to Enhance Efficacy & Minimize Toxicity

1.30 **Improving Targeting with Antibody & AAV-Mediated Delivery of Nucleic Acid Drugs for Muscular Disorders to Reduce Off Target Effects & Enhance Efficacy**

- Utilizing AAV delivery systems to improve targeting of therapeutic payload to relevant tissues with efficacy
- Evaluating antibody-mediated delivery to overcome biodistribution challenges and reduce toxicity
- Enhancing targeting to improve therapeutic index of nucleic acid drugs and support safer and more efficacious clinical outcomes



Mathias Schmidt
Chief Executive Officer
JCR Therapeutics

2.00 **Engineering Novel Targeted Muscle Delivery Technologies to Enhance Oligonucleotide Delivery & Biodistribution**

- Moving towards delivery platforms that enhance payload delivery to increase efficient drug delivery
- Addressing poor specificity and the consequences of long-term drug accumulation
- Exploring how we can achieve more accurate delivery and trafficking characteristics to the muscle for DM1 therapies



Damon Wang
Chief Executive Officer
NuCyRNA Therapeutics
NEW DATA

2.30 **Afternoon Break & Poster Session**
Take this opportunity to showcase your latest research and innovations with your peers and understand the strategies of your fellow muscular dystrophy experts.

Reviewing the Emerging Cross-Modality Landscape for Muscular Disorder Drug Development

3.15 **Exploring the Lessons Learnt from Gene Therapies to Optimize the Efficacy & Delivery of Next-Generation Therapeutics**

- Examining clinical and regulatory findings from early gene therapy programs to understand where the field is moving to advance safety, patient selection and efficacy
- Assessing how historical limitations are being addressed to improve therapeutic performance and enable the development of safer and more effective treatments
- Translating real-world and clinical trial insights into future modality development strategies to guide better decision making and increase the chances of clinical and commercial success



Seemin Ahmed
Principal Scientist and Gene Therapy Leader
Novartis

Conference Day One
Wednesday, January 27

3.45 **Panel Discussion: Evaluating the Strengths & Limitations of Genetic, Stem Cell, Small Molecule & RNA-Based Muscle Disease Therapies**

- Examining the use of different modalities to address the underlying drivers of muscle degeneration and guide development decisions
- Debating the practical trade-offs between administration, manufacturing and patient accessibility to understand the implications of each modality
- Sharing cross-modality learnings from clinical and translational programsto identify common barriers to pipeline advancement

LEADING PHARMA VOICES



Jun Shi
Director of Muscle Disease Research
Biogen



Jim Breitmeyer
Chief Executive Officer
Altay Therapeutics



Ricardo Mondragon Gonzalez
Principal Scientist, Muscle Biology & Rare Diseases
Sanofi



Nitza Thomasson
Executive Director, Global Head of Research and Development Neurology
Servier

4.15 **Chair's Closing Remarks**



Kristina An Haack
Senior Global Head, Clinical Development
Sanofi

4.30 **End of Conference Day One**

"I'm looking forward to listening, learning and sharing to better serve the muscular dystrophy communities"

Nitza Thomasson, Executive Director, Global Head of Research and Development Neurology, Servier

"Great meeting and excellent talks. Perfect opportunity for networking with the experts in the drug delivery field. It was a great opportunity to learn from other groups, to be up to date on the current activities regarding for drug delivery, to connect and network"

5th Annual ALS Drug Development Summit Past Attendee, Servier

Conference Day Two
Thursday, January 28

8.00 **Check-In & Light Breakfast**

8.50 **Chair's Opening Remarks**



Sweta Girgenrath
Vice President, Therapeutic Research & External Innovation Lead
Entrada Therapeutics

Evaluating Preclinical Models to Better Predict Clinical Outcomes in Muscular Disorders

9.00 **Discussing the Appropriateness of 2D, 3D, & *in vivo* Models to Build a Robust Preclinical Data Package in Rare Disease Programs**

- Exploring the use of animal models within a program's means to ensure preclinical work that is predictive into the clinic
- Comparing the advantages and disadvantages of 2D and 3D models for long term rare disease studies in terms of dosing and translatability
- Establishing the importance of a robust preclinical data package and how to achieve this in early strategic development



Sweta Girgenrath
Vice President, Therapeutic Research & External Innovation Lead
Entrada Therapeutics

10.00 **Mitigating Clinical Risk with Strategic Preclinical Design for DMD Exon Skipping Therapeutics**

- Applying AOC platform for exon skipping in DMD as therapeutic approach
- Creating successful and translatable preclinical package for Duchenne's



Barbora Malecova
Director of Biology
Avidity Biosciences

10.30 **Muscular Innovation Exchange**
Join the discussion most relevant to your work and gain insights from experts facing shared translational and clinical challenges. Move between tables split by therapeutic area throughout the session to connect with peers, and gain insight through meaningful industry connections.

MODALITY-BASED NETWORKING SESSION

11.00 **Morning Break**

Comparing Therapeutic Modalities to Develop Meaningful Patient Outcomes & Better Address Muscle Disease Pathology

11.30 **Targeting Muscle Function in DM1 With Small Molecules to Expand Treatment Options Beyond Gene Therapy**

- Highlighting advances in RNA targeting small molecules to demonstrate the benefit of functional improvements achieved through non-genetic mechanisms
- Exploring emerging small molecules designed to enhance therapeutic benefit and broaden therapeutic development options



Domi Stickens
Chief Scientific Officer
Arrakis
BIOTECH INNOVATOR

12.00 **Comparing Antibodies & Small Molecules to Best Address Immunogenicity, Biodistribution & Efficacy in Drug Development**

- Comparing the molecular and pharmacological properties of antibodies and small molecules to understand how size, affinity, valency and molecular architecture influence target engagement and therapeutic performance
- Exploring how modality choice impacts biodistribution, tissue penetration, dosing and productive intracellular delivery, while considering modality-specific challenges including immunogenicity and off-target exposure
- Using preclinical and clinical examples to define where antibodies and small molecules offer distinct advantages, providing a practical framework for selecting the right targeting modality for the target, tissue and therapeutic payload



Jimmy Weterings
Vice President, Head Oligonucleotide Therapeutics
Bonito Biosciences

12.30 **Lunch Break & Networking**

Conference Day Two
Thursday, January 28

Tailoring Drug Development to Prioritize Symptomatic Burden & Deliver Meaningful Benefits for Patients

1.30 **From Dystrophin to Function: A Patient-Centered Approach to Drug Development in Becker Muscular Dystrophy**

- Moving beyond protein restoration towards meaningful functional improvement for patients
- Understanding why muscle function is what matters most to patients
- Exploring the clinical development of Sevasemten to reveal the lessons learned for future development of other muscular dystrophies
- Establishing the role of small molecules within an increasingly diversified treatment landscape



Nitza Thomasson
Executive Director, Global Head of Research and Development Neurology
Servier
IN-TRIAL INSIGHTS

2.00 **Roundtable Discussion: Considering Non-Muscular Products to Uncover Novel Targets & Address Previously Unmet Patient Needs**

- Discussing lesser addressed symptoms for muscular disorders to improve patient quality of life beyond enhancing muscle function
- Examining how biomarkers used in cardiovascular, inflammatory, GI and neurodegenerative diseases can be adapted for muscular disorders to broaden biomarker discovery opportunities and tailor patient characterization
- Investigating successful target identification strategies from other therapeutic areas to uncover shared pathways and reveal new intervention points for muscular disorders

2.30 **Afternoon Break**

Decoding Patient Heterogeneity in Muscular Dystrophy: Biomarkers, Disease Progression, & Clinical Outcomes

3.15 **Refining Patient Pool Selection to Overcome Patient Heterogeneity Challenges & Improve Clinical Success**

- Leveraging genetic, molecular and disease-specific biomarkers to distinguish patient subgroups within Duchenne, Becker, FSHD, DM1 and LGMD and strengthen the interpretation of clinical outcomes
- Characterizing differences in ambulatory status, muscle degeneration patterns and rates of disease progression across patients



Jiyeon Denninger
Medical Director, Clinical Development
Sarepta
BIOTECH INNOVATOR

3.45 **Anticipating Heterogeneity in Rate of Dystrophy to Prepare for Endpoint Variation & Optimize Trial Design**

- Characterizing differences in disease progression trajectories across patient populations to improve endpoint selection
- Designing adaptive and risk-adjusted clinical trial strategies that account for heterogeneous decline patterns and maximize the likelihood of detecting treatment effects



Jim Breitmeyer
Chief Executive Officer
Altay Therapeutics

4.15 **Chair's Closing Remarks**



Ricardo Mondragon Gonzalez
Principal Scientist, Muscle Biology & Rare Diseases
Sanofi

4.25 **End of Muscular Disorders Drug Development Summit**

Partner With Us

Connect with a Global Network of Leading Myopathy Drug Developers, Build Strategic Partnerships & Drive Success

More companies are moving into the muscular drug development space and pharma investment accelerates, evidenced by multi-billion dollar spends from the likes of Servier and Novartis. In turn, the demand for complex preclinical models, digital endpoint support and muscle-specific imaging and biomarker services is growing.

The challenges for rare disease trial design and drug development are unique, with decision makers looking for innovative 2D and 3D preclinical models with high translatability and tools to accurately measure and quantify muscle function. With limited patient pools and biopsy samples available, pharma and biotech are seeking precision and specialist expertise throughout every phase of the drug development process.

That's where we need you.

Our decision makers from pharma and biotech are searching for long-term partners to support them in:

Preclinical CROs & Disease Models

Supporting biopharma to evaluate the safety, efficacy, biodistribution and pharmacokinetics of novel therapeutics before clinical development. As gene-therapies, RNA-targeting approaches, and next-generation modalities advance across muscular dystrophies, developers are seeking CROs with specialized neuromuscular expertise and translational disease models. From animals to human-relevant systems such as muscle-on-a-chip, sponsors need partners who can generate predictive data, improve clinical translation and help de-risk development decisions.

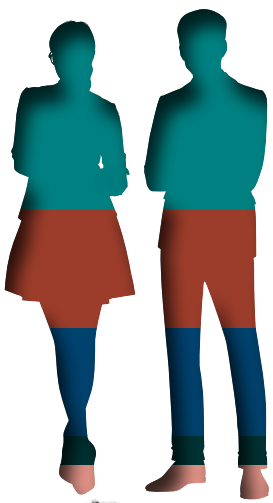
Clinical CROs

Designing, implementing and executing clinical trials across rare muscular dystrophies while navigating the complexities of small patient populations and evolving regulatory expectations. Developers are looking for CROs with expertise in neuromuscular diseases, global trial operations, patient recruitment and natural history-informed study design. Partners who can work seamlessly across functions and manage compliance requirements are critical to accelerating development and working towards clinical success.

Digital & Functional Biomarker Services

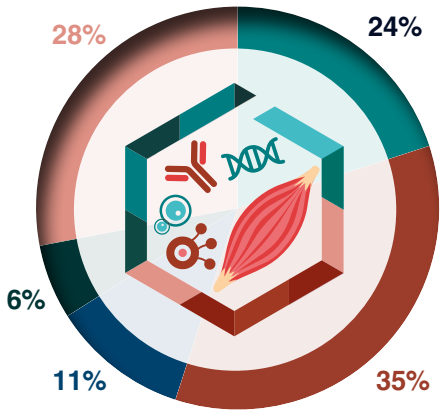
Providing innovative tools and technologies to measure disease progression, treatment response and muscle function across muscular dystrophies. As sponsors look beyond traditional clinical endpoints, demand is growing for providers who can measure digital biomarkers with wearable devices, and remote monitoring solutions alongside established functional assessments.

Seniority of Attendees



- C-Level/VP 41%
- Director & Head Of 24%
- Manager & Group Leads 22%
- Scientist 6%
- Other 7%

Type of Companies Attending



- Pharma
- Biotech
- Academic, NPO & Investor
- Patient, Advocates & Caregivers
- Service Providers

*Statistics taken from the 5th Annual ALS Drug Development Summit



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DISCOVER how leading companies are making drug development decisions in practice, from selecting preclinical tools to measuring functional endpoints

BUILD your knowledge of the current challenges, strategies and solutions to strengthen translational animal models, quantify endpoints and optimize trial design for rare genetic diseases

CONNECT with the Muscular Disorder community, showcase your solutions to developers facing shared R&D challenges, and build lasting relationships that support long term success

Drug Developer Pricing*	Register & Pay By Friday, October 30	On the Door
Full Access Pass	$3,297 (Save $600)	$3,857
Conference Only	$2,399 (Save $400)	$2,799
Academic Pricing**	**Register & Pay By Friday, October 30**	**On the Door**
Full Access Pass	$2,697 (Save $600)	$3,257
Conference Only	$1,999 (Save $400)	$2,399
Service & Solution Provider Pricing	**Register & Pay By Friday, October 30**	**On the Door**
Full Access Pass	$4,497 (Save $400)	$4,897
Conference Only	$3,199 (Save $400)	$3,599

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Do you work for a Not-for-Profit organization? Email us at info@hansonwade.com to inquire about attending

Team Discounts***

- 10% discount – 3 Attendees
- 15% discount – 4 Attendees
- 20% discount – 5+ Attendees

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Contact: register@hansonwade.com

VENUE:

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For further information or assistance, please visit:

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