2024 MDA Clinical and Scientific Conference
Orlando, FL
March 3-6, 2024

Activity Overview
The 2024 MDA Clinical & Scientific Conference will provide a comprehensive exploration of pre-clinical, translational, and clinical research and care for individuals with neuromuscular disease (NMD), with particular attention to some of the changes the field is undergoing in response to the approval of new therapies**.**

Target Audience
This activity is designed for an audience of nurses, nurse practitioners, pharmacists, physicians, physician assistants, and social workers.

Learning Objectives
Upon completion of the educational activity, participants should be able to:

Allied Health Symposium

Navigating Challenges in Care Transitions & Coordination

- Identify challenges to coordinating care and navigating care transitions in NMD.
- Identify care center-specific solutions to care challenges in NMD.
- Tailor best practices in coordinating care from exemplary institutions to institutional needs.

Track: Clinical Considerations

Rapid-Fire Clinical Case Reviews Part 1: *Motor Neuron Disease & NMJ Disorders*

- Identify SOD1 phenotypes, including the most severe and most mild forms of ALS
- Evaluate potential risks of available therapies for specific phenotypes.
- Examine the role of nutritional deficiencies and supplementation strategies in neuromuscular disease.
- Identify ways to accelerate diagnosis and opportunities to increase access to genetically targeted NMD treatments.
- Identify strategies to communicate with families resistant to treatment plans.
- Identify ways to involve external entities when the well-being of a child is at stake.
- Increase vigilance and follow-up testing to avoid potential newborn screening “misses”, even in
- states that have adopted specific NMDs into their statewide screening programs.
- Integrate differential diagnosis of NMJ disorders into care, including assessment of NMJ dysfunction in patients presenting with proximal weakness.

Rapid-Fire Clinical Case Reviews Part 2: Muscle Diseases

- Adopt strategies to identify and manage cardiovascular obstetric complications of pregnancy in people with NMDs.
- Identify diagnostic challenges in pediatric NMD, including limitations of current genetic testing 3. Identify phenotypes among the broad spectrum of titinopathies.
- Adopt strategies to investigate VUS identified by NGS in TTN, such as muscle imaging, family studies, and new in silico tools.
- Recognize when to utilize broad genetic testing, additional phenotyping, or biochemical testing for a specific disorder or complex case.
- Anticipate genetic risks and communication needs with patients regarding family planning 7. Identify examples of diversity in genetic muscle disorders and related opportunities to optimize management for better patient quality of life.

Challenges Facing the Clinician in the Era of Treatment

- Identify corticosteroid regimens used in management of neuromuscular diseases; advantages, disadvantages and potential challenges when evaluating potential corticosteroid regimens.
- Identify key questions to ask when discussing treatment options with families.
- Identify FDA-approved treatments for NMDs and recognize differences in approved therapies based on indication, age range, stage of disease.
- Explore clinical challenges/implications of accelerated vs full approval types (biomarker-based vs demonstrated clinical benefit).
- Explore the utility of biomarkers as a clinical endpoint, especially as it applies to exon skipping and gene-based therapies.

- Consider restrictions to therapy access, including FDA labeling, payer approvals, biases in equitable access, and local barriers to access, including personnel requirements.
- Identify strategies by pharma/ government to alleviate access challenges (eg, sponsored programs, "right to try" legislation).
- Recognize considerations for using combination therapies in DMD and SMA.

Setting Patients up for Success: Life Transitions

- Identify at least 3 types of common transitions experienced by the neuromuscular patient population.
- Identify at least one opportunity for improvement in their own clinics regarding successful transitions.

Overview of Therapeutics for FSHD

- Recognize clinical presentation and disease progression.
- Discuss the current multidisciplinary approach to care for FSHD.
- Recognize the role of aerobic exercise in cardiovascular health for FSHD, and be able to provide guidance for resistance training for FSHD
- Discuss the current treatment landscape, recognizing small molecule therapies targeting DUX4 expression and evaluating the impact of non-DUX4 targeted therapeutics in FSHD.
- Discuss the mechanistic argument for disease targeted therapies, and future approaches for gene therapy.

Track: Clinical Approaches to Streamlining Care

Newborn Screening in NMD

- Recognize newborn screening pilot study design and the importance of early detection.
- Compare benefits and drawbacks of newborn screening for Duchenne muscular dystrophy using creatine kinase -MM and sequencing methods.
- Describe potential algorithms for newborn screening for Duchenne muscular dystrophy.
- Discover Minnesota's experience with NBS in Pompe disease and compare Pompe Disease Screening Protocols Worldwide.
- Compare and contrast the Ethical and Social Implications: What We Know from Minnesota.

Pediatric Neuromuscular Disease

- Recognize ways to collaborate / share in care of pediatric NM patients with their primary care physicians.
- Recognize ways to collaborate / share in care of pediatric NM patients with school based physical therapists.
- Recognize ways to collaborate / share in care with emergency medicine doctors, clear communication of NMD patients risk factors leading to need for emergency medical attention.
- Discuss strategies for successful transition of pediatric patients to an adult setting.
- Compare the variety of strategies used across different regions.

Engaging Primary Care and Non-NMD Experts

- State two ways to engage with the clinical community for NMD patients
- State two strategies to ensure the identification of critical clinical stages in NMD and that appropriate referrals are made
- State two ways to provide educational opportunities to facilitate comprehensive clinical workup, diagnostic testing, and expedited referral to NMD specialists
- Utilize case studies presented to facilitate understanding of engaging primary care of non-NMD providers in the clinical care of NMD patients.

Novel Strategies for Clinical Trial Recruitment & Participation

- Recognize what constitutes a Recruitment Plan.
- Utilize social media platforms to aid in recruiting a diverse audience.
- Identify why diverse representation is crucial for clinical research design.
- Identify strategies designed to include community experts in research design from implementation through retention.
- Implement CARE Research Center-designed solutions within existing clinical trial engagement.

Multidisciplinary Care Panels: Meeting the Needs of the Patient TODAY

- Explore the current data on patient health outcomes for multidisciplinary clinics.

- Recognize possible pitfalls and opportunities of starting a multidisciplinary clinic within the current US healthcare system.
- Discover different models of multidisciplinary care.
- Discover how to improve and optimize multidisciplinary clinic workflow.
- Recognize how to integrate physicians, allied health and patients/parents' perspective for quality improvement.

Underserved Communities & Working with What We've Got: Overcoming Challenges in the Healthcare System

- Learn collaborative strategies to provide complex treatment, especially in underserved communities.
- Recognize how to use outreach clinics to treat and educate patients and how to recruit rural patients for research studies.
- Discover how to navigate limited resources and care for a refugee population through the Polish National Health Service experience.
- Identify special cultural considerations in treating underserved communities through the Native American experience in the Southwest and how these principles can apply to many patients.
- Recognize the role of early diagnosis and treatment initiation in SMA (NBS, newly diagnosed patient's pathway).
- Recognize the need for disease modifying therapies (DMTs) criteria and coordination of care (our Polish SMA experience – a centralized system).
- Explore transitioning patients from pediatric to adult care.

Track: Practical Considerations in Gene Therapy

Where Are We in Gene Therapy in Clinical Practice?

- Identify conditions for which gene therapy is a treatment option.
- Recognize potential risks and benefits of gene therapy across neuromuscular diseases.
- Examine mitigation strategy when adverse events occur as part of gene therapy.
- Explore physiology of the immune response in gene therapy.
- Identify strategies to discuss gene therapy as a treatment option with families with NMD, and anticipate potential questions/ concerns that may be received from families.
- Identify ethical questions that arise with a unique therapy such as gene therapy.
- Recognize how gene therapy might impact families with a child undergoing gene therapy.

Is my Institution Prepared for Gene Transfer Therapies?

- Review considerations for clinical care pre- and post-gene transfer therapy administration.
- Discuss complexities of dosing day, including scheduling, patient readiness, and staff readiness. Explore the need for patient access to subspecialty centers.
- Identify necessary infrastructure components including specialized facilities/equipment, hospital leadership, clinical leadership, supportive personnel, and pharmacy considerations.
- Assess the current state of your institution's infrastructure and resources and develop a roadmap for addressing any infrastructural gaps or strengthening existing capabilities.
- Recognize the logistical considerations involved in sourcing and delivering gene therapies, including storage, transportation, and administration procedures.
- Review the end-to-end process for GRT from a hospital administration perspective.
- Identify necessary steps to prepare the child and family for the gene replacement therapy procedure.
- Develop a differential diagnosis for and management plan for nausea and vomiting occurring in a child who has received gene replacement therapy.

Orchestrating the Team and Planning for the Unexpected

- Identify gene therapy resources, educational opportunities and best practice sharing to support the MDA Care Center Network and the NMD community.
- Discuss the institutional experience for a gene therapy as a non-clinical trial site.
- Recognize potential safety issues that may arise during the monitoring period post viral mediated gene transfer, and the need for a gene therapy team to coordinate and monitor post gene transfer care.
- Explore the ongoing gaps in understanding related to post gene transfer monitoring – including: timing of vaccinations and while on high dose corticosteroids; precautions for patients post gene transfer; use of additional immune modulation beyond steroids; and follow-up safety monitoring labs.

Navigating Difficult Conversations

- Describe the process of determining eligibility for gene therapy.
- Recognize the nuances involved in the decision-making process such as assessing health literacy to guide patients/parents in decision making and helping decision makers through the fear and uncertainty involved.
- Recognize the barriers to access both external (insurance coverage, regional location) and internal (psychological, practical) barriers to access.
- Explore the ethical considerations associated with guiding parents and patients through the process.

Gene Therapy in DMD: Is It Now or Never

- Review current data on available DMD gene therapies.
- Review challenges related to DMD gene therapy and clinician and caregiver decision making.
- Outline case examples to highlight care and issues related to DMD gene therapies.
- Explore the progress made in the development and path to approval of dystrophin-targeted therapies in DMD.
- Recognize the current challenges and limitations for AAV-based gene therapy in human clinical trials for DMD.
- Review future opportunities and possible solutions for next generation gene-based treatments for DMD.

Criteria for Success

There is no fee to participate in this activity. Statements of credit will be awarded based on the participant's attendance. A statement of credit will be available upon completion of an online evaluation/claim credit form available at:

https://akhinc.formstack.com/forms/240021mda

Please claim your credit by 6/6/2024

If you have questions about this CE activity, please contact AKH Inc at wendi@akhcme.com

CE credit provided by AKH Inc., Advancing Knowledge in Healthcare.

In support of improving patient care, this activity has been planned and implemented by AKH Inc., Advancing Knowledge in Healthcare and MDA. AKH Inc., Advancing Knowledge in Healthcare is jointly accredited by the Accreditation Council for Continuing Medical Education (ACCME), the Accreditation Council for Pharmacy Education (ACPE), and the American Nurses Credentialing Center (ANCC), to provide continuing education for the healthcare team.

This activity was planned by and for the healthcare team, and learners will receive 16 Interprofessional Continuing Education (IPCE) credit for learning and change.

Physicians

AKH Inc., Advancing Knowledge in Healthcare designates this live activity for a maximum of 16 *AMA PRA Category 1 Credit(s)*™. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

Nurses

Credit being awarded: 16 ANCC contact hours

Nurse Practitioners

This activity has been planned and implemented in accordance with the Accreditation Standards of the American Association of Nurse Practitioners (AANP) through the joint providership of AKH Inc., Advancing Knowledge in Healthcare and JP. AKH Inc., Advancing Knowledge in Healthcare is accredited by the American Association of Nurse Practitioners as an approved provider of nurse practitioner continuing education. Provider number: 030803. This activity is approved for 16 contact hour(s) (which includes 16 hour(s) of pharmacology).

Pharmacists

AKH Inc., Advancing Knowledge in Healthcare designates this continuing education activity for 16 contact hours.

Physician Assistant

AKH Inc., Advancing Knowledge in Healthcare has been authorized by the American Academy of Physician Associates (AAPA) to award AAPA Category 1 CME credit for activities planned in accordance with AAPA CME Criteria. This activity is designated for 16 AAPA Category 1 CME credits. PAs should only claim credit commensurate with the extent of their participation.

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Commercial Support

There is no commercial support for this activity.

Name	Relationship	Commercial Interest
Omer Abdul-Hamid, MD	N/A	Nothing to disclose
Faria Abdullah, MD, MS	N/A	Nothing to disclose
Amy Bartlett	N/A	Nothing to disclose
Kaitlin Batley, MD	Advisor	Sarepta Therapeutics, PTC Therapeutics Reata Pharmaceuticals, Biogen, UCB, Inc Pfizer, Catalyst Pharmaceuticals, Inc.
Diana Bharucha-Goebel	N/A	Nothing to disclose
Enrico Bugiardini MD, PhD	Advisor Consultant	Avidity Biosciences, Inc Fulcrum
Russell Butterfield, MD, PhD	Advisor Researcher	Sarepta Therapeudics, Pfizer, Aavanti Bio, Scholar Rock, Reata Ionis Pharmeceuticals
Nora Capocci	N/A	Nothing to disclose
Meeta Cardon, MD	Consultant	Sarepta
Elena Caron	N/A	Nothing to disclose
Diana Castro, MD	Advisor Researcher	Sarepta, PTC, Novartis Edgewise
Emma Ciafaloni, MD	Advisor Researcher	Sarepta, Alexion, Argenx Sarepta, Neurogene
Lora Clawson, MSN, CRNP	N/A	Nothing to disclose
Anne Connolly MD	Advisor	Sarepta, Edgewise, Biohaven, Avidity Therapeutics, Octapharma
Thomas Crawford, MD	Advisor	Biogen Idec, Novartis AG, Scholar Rock, Sarepta, Catalyst, Erydel, Genentech

Urvi Desai, MBBS, MD	Advisor Speaker	Takeda, Argenx, Fulcrum, Alnylam, Biogen CSL Behring, Alexion, Argenx
John Day, MD, PhD	Advisor Consultant	Avidity Biosciences; Biogen; Epirium; Kate Therapeutics; Novartis Gene Therapies; PepGen; Roche/Genentech Pharmaceuticals; Sanofi; Sarepta Therapeutics; Scholar Rock; Vertex Pharmaceuticals AMO Pharma; AnnJi Pharmaceutical; Astellas Gene Therapies; Avidity Biosciences; Biogen;Genentech; Ionis Pharmaceuticals; NMD Pharma; Novartis Gene Therapies; Roche Pharmaceuticals; Sanofi-Genzyme; Sarepta Therapeutics; Scholar Rock
Deidre Devier, MSW, PhD	N/A	Nothing to disclose
Bakri Elsheikh, MBBS, FRCP	Consultant	Biogen, Genentech, Argnex, Medscape
Marcia Felker, MD	Advisor Researcher	AveXis/Novartis Novartis, Biohaven
Erika Finanger, MD	Advisor Researcher	Sarepta Therapeutics, Pfizer, PTC Reata Sarepta, Fibrogen, Dyne, Italfarmaco Biogen, Scholar Rock. Novartis
Rich Finkel, MD	Advisor	Biogen, Genentech/Roche, Novartis, Sarepta, Scholar Rock, Dyne, Affinia
Dominic Fullenkamp, MD, PhD	N/A	Nothing to disclose
Natalie Goedeker, DNP, CPNP	Advisor	Sarepta
Helen Hemley, MPA	N/A	Nothing to disclose
Jessica Herron, PT, DPT	N/A	Nothing to disclose
M. Becky Hopfinger, MSN, MBA, FNP-BC	N/A	Nothing to disclose
Candice Johnson, OTD, OTR/L	N/A	Nothing to disclose
Natalie Katz, MD, PhD	N/A	Nothing to disclose
Katerina Kucera, PhD	Researcher	Sarepta Therapeutics Ionis Pharmaceuticals
Nancy Kuntz, MD	Advisor Researcher Speaker	Argenx, Astellas-X, Biogen, Catalyst, Genentech, Novartis, Sarepta Argenx, Astellas-X, Biogen, Catalyst, Genentech, Novartis, Sarepta, Reveragen Sarepta
Anna Kostera-Pruszczyk, MD, PhD	Advisor/Consultant/Speaker	Biogen, Novartis/Avexis, Roche, PTC
Monika Krzesniak-Swinarska MD	N/A	Nothing to disclose
Chamindra Laverty MD	N/A	Nothing to disclose
Kaitlin Lewis, BA, CTRS	N/A	Nothing to disclose
Brian Lindsey, DO	N/A	Nothing to disclose
Katherine Mathews, MD	Researcher	Sarepta, PTC, Fibrogen, Italfarmaco Capricor, Pfizer, Edgewise
Leslie Morrison, MD	Consultant and Speaker	Neurelis
Diane Murrell, MSW, LCSW	N/A	Nothing to disclose
Julie Parsons, MD	Advisors Researcher	Biogen, Novartis Biogen, Novartis, PTC Therapeutics, Scholar Rock, Genentech, Biohaven
Holly Peay, PhD, MS	Researcher	Sarepta Therapeutics
Amanda Peltier, MD	Consultant	Astrazeneca, Alnylam
Nishitha Pillai MD, FAAP, FACMG	Consultant	Sanofi, Amicus, Denali
Crystal Proud MD	Advisor Researcher	Biogen, Sarepta, AveXis/Novartis Gene Therapies, Genentech/Roche and Scholar Rock

	Speaker	AveXis/Novartis Gene Therapies, Astellas, Biogen, CSL Behring, Fibrogen, PTC, Pfizer, Sarepta, Scholar Rock Biogen
Bruce Rabin	N/A	Nothing to disclose
Leigh Ramos-Platt	Advisor	Catalyst
Emelia Rodgers, LICSW, MSW	N/A	Nothing to disclose
Jennifer Roggenbuck, MS, CGC	Consultant	Biogen, Ionis
Jacinda Sampson, MD, PhD	Advisor Speaker	Sanofi, Dyne, Expansion Therapeutics Sarepta, Viking Biogen
Perry Shieh MD PhD	Consultant Speaker	Sarepta, Pfizer, Biogen, Genentech, Novartis, Alexion, argenx, UCB, Catalyst CSL Behring, Grifols, argenx, Alexion, Genentech, Biogen, Catalyst
Rulla Starr MS	Speaker	Biogen
Jeffrey Statland	Consultant	Dyne, Avidity, Roche, Fulcrum, Epic Bio, Kate, Vertex, Armatus, Lupin, Entrada
Norma Tavakoli, PhD	Researcher	Pfizer, Inc., PTC Therapeutics, Revvity, Sarepta Therapeutics, Solid Biosciences, Wave Life Sciences
Rabi Tawil	Consultant	Ionis, Ipsen Pharma, MT Pharma, Fulcrum Therapeutics, miRecule, MT Pharma, Epic Bio., Arrowhead Pharma, Entrada Therapeutics.
Aravindhan Veerapandiyan MD	Advisor	AMO Pharma, AveXis, Biogen, Edgewise Therapeutics, FibroGen, Novartis, Pfizer, PTC Therapeutics, Sarepta Therapeutics, Inc., UCB Pharma, Catalyst, and Scholar Rock
Megan Waldrop MD	Advisor Consultant Researcher	Sarepta Therapeutics, Inc Novartis Gene Therapies Sarepta Therapeutics, Inc Novartis Gene Therapies Sarepta Therapeutics Inc Audentes Therapeutics/Astellas Gene Therapies
Matthew Wicklund MD	Consultant	Amicus Therapeutics, Sarepta Therapeutics, Spark Therapeutics UCB Pharma, Ultragenyx Pharmaceutical
Amanda Witt, MD	N/A	Nothing to disclose
Gil Wolfe, MD	Consultant	Alexion, ArgenX, BPL, UCB, Cartesian, Janssen
AKH Inc Staff and Planners	N/A	Nothing to disclose
MDA Staff and Planners	N/A	Nothing to disclose
All of the relevant financial relationships listed for these individuals have been mitigated.		

Disclosures

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