2023 MDA Clinical & Scientific Conference
Dallas, TX
3/19/2023 – 3/22/2023

Activity Overview

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

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

Session: Making visible the invisible: Cognitive and Behavioral Health in Muscular Dystrophy

- Highlight recent advances in our understanding of brain health in muscular dystrophy. Participants will also learn pragmatic approaches and available resources to help families navigate challenges in this domain.
- Explore the potential of MRI and ultrasound imaging measures as biomarkers of disease progression and treatment response in certain dystrophies.
- Discuss how operant approaches are largely ineffective for addressing chronic behavior challenges.
- Review mental, emotional/behavioral and learning concerns evident in DBMD population.
- Describe basic overview of brain dystrophin.
- Introduce conceptual framework for understanding and managing emotional/behavioral and learning concerns.

Session: Bone Health - Where have we been, where are we now, and where are we going?

- Review where we have been in bone health.
- Examine the view beyond the current guidelines of bone health.
- Discuss current guidelines and management of bone health.

Session: Digestive Health Impacts from Top to Bottom

- Recognize common GI conditions in DMD.
- Describe characteristics of swallowing deficits in DMD.
- Review the distinct nutritional phases of Duchenne Muscular Dystrophy and the challenges of nutritional assessment in this population.

Session: Diagnostic Testing Options

- Discuss the limitations of mutation detection in blood-derived genomic DNA tests.
- Review the need for RNA analysis in a subset of dystrophinopathy patients.
- Explain the molecular mechanisms that result in discordance with clinical testing predictions of the “reading-frame rule”.
- List some of the genetic neuromuscular disorders with specific, diagnostic pathologic features observable in a muscle biopsy.
- Describe the biopsy evaluation methods that provide diagnostic information in patients with genetic neuromuscular disease.
- Recognize some of the classic neuromuscular pathologic features associated with genetic myopathies and neuropathies.

Session: Supporting the Patient Throughout Their Journey

- Discuss the successful transition of the patient across developmental milestones from childhood to adulthood.
- Recognize the indication for palliative care and end of life decision making.
- Identify ways to represent the caregiver and family perspective of everyday life with neuromuscular disease.

Session: Registry Engagement Successes

- Explain the history, status and future direction of the MDA MOVR Data Hub.
- Express to patients and caregivers the numerous facets of the National ALS Registry and the importance of joining this research initiative.
- Identify the challenges in recruiting subjects to registries.
- Review The development of the CF Foundation data registry.
- Describe the possibilities to improve care using data registries.
- List challenges and opportunities that registries can provide.

Session: Leveraging Providers in the Community

- Identify ways to leverage community resources to supplement adherence to published care standards.
- Identify strategies for fostering communication between community-based providers and the specialist care team.
- Identify opportunities to effectively work and communicate with community-based services in public schools and the workplace (ex. IEP and 504 planning, workplace adaptations).
- Identify special issues related to transition to adult roles and adult care. (ex. transition to regional adult providers, occupational/vocational rehabilitation, college student care, etc.).
- Identify community resources who can promote activity to prevent secondary conditions.
- Identify home-based technologies that can supplement clinical follow-up in the community setting.
- Identify strategies for employing telehealth-enabled services and technologies (eg. remote telehealth assessments, pulmonary function monitoring, electronic patient-reported outcomes, oral analytics, sleep studies/overnight capnography).
- Identify areas where new technical tools are being developed to improve community functional ability (eg. augmentative communication tools, “wearable” robotics).

Session: Holistic Approach to Transitioning into Adulthood

- Discuss the importance of effective, multidisciplinary transition.
- Describe the barriers to transition in the neuromuscular population.
- Differentiate between transition and transfer.

Session: Therapy Interventions – Modify Therapy Approaches with Evolving Market Landscape

- Discover the impact of a novel therapeutic modality for optimizing movement in children treated for spinal muscular atrophy.
- Recognize the potential utility of isometric resistance exercise in patients with Duchenne muscular dystrophy.
- Select strategies to enhance the implementation of a remote exercise program.
- List three caregiver-reported benefits of home exercise training using a Therapeutic Play Gym for children with medically complex neuromuscular diagnosis.
- Examine how the CV-19 pandemic is rapidly changing future health delivery models, and how pediatric therapists may implement these changes within their clinical practice.
- Identify what resources may be available or are under development for exercise intervention in patients with disabilities.
- Identify who may be using these exercise resources.
- Identify limitations and strengths of current resources.
- Recognize needs for guidance for patients and other healthcare professionals utilizing resources.
- Describe how wearable technology can provide digital mobility outcomes for individuals with neuromuscular diseases in a disease modifying treatment landscape.
- Identify limitations to current consumer and research grade wearable technology.
- Describe validity and reliability of AI-enabled insole technology in controlled and real-world settings.
- Summarize the use of wearable sensors in quantifying infant movement and their application for infants with neuromuscular disorders.

Session: Future of Gene Therapy in NMD: Considerations for Newborn Screening & Clinical Trial Design

- Describe the process by which a disorder is nominated, approved, and implemented in NBS.
- Identify the very real factors that will make it difficult for NBS to quickly add new disorders, even those with transformative treatments.
- Suggest strategies for successful adoption of screening for neuromuscular disorders when treatments are proven and available.
- Discuss therapeutic options DMD, SMA, and related diseases.
- Explain some of the challenges for conducting ongoing clinical trials in these diseases.
- Review tools and approaches to overcoming clinical trials challenges.

Session: Practical Management of Gene Therapy: Procurement & Administration

- Discuss how to create a gene therapy standard operating procedure (SOP).
- Describe the process for implementing gene therapy in the clinic space, including required staff and parental education.
- Review challenges, including some real-world case examples, and how we can learn from experience to date in this area.
- Discuss a pragmatic approach to deciding which treatment is right for your child.
- Explain the timeline in which you should begin reaching out about trial access.
- Describe the commitment and importance of these trials, and why they matter.
- Recognize the importance of newborn screening and early diagnosis.
- Identify potential for changing disease progression with early treatment.
- Examine ways to leverage medicinal therapies and support services.
- Paraphrase the potential impact of continued research.
- Express the considerations when preparing for the assessment of a patient pursuing gene transfer therapy.
- Review the complexities and options for acquisition of specialty products.
- Discuss payor considerations and utilizing policies to optimize efficiency.
- Describe options for administration.
- Summarize the importance of ongoing coordinated specialty care for the patient with rare disease.

Session: Clinical Challenges in the Gene Therapy Era: Roundtable Discussion of Difficult Cases

- Discuss the difficulties that may be encountered in gene therapy administration.
- Identify options for addressing gene therapy-related challenges using real-world case examples.

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/230003mda

Please claim your credit by 3/31/2023

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 9 Interprofessional Continuing Education (IPCE) credit for learning and change.

Physicians

AKH Inc., Advancing Knowledge in Healthcare designates this live activity for a maximum of 10 *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: 10 ANCC contact hours

Pharmacists
AKH Inc., Advancing Knowledge in Healthcare designates this continuing education activity for 10 contact hours.

Physician Assistant
AKH Inc., Advancing Knowledge in Healthcare has been authorized by the American Academy of PAs (AAPA) to award AAPA Category 1 CME credit for activities planned in accordance with AAPA CME Criteria. This activity is designated for 10 AAPA Category 1 CME credits. PAs should only claim credit commensurate with the extent of their participation.

Social Workers
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Commercial Support
There is no commercial support for this activity.

Name	Relationship	Commercial Interest
Ajay Kaul, MD (faculty)	N/A	Nothing to disclose
Ambereen Mehta,MD, MPH, APHS (faculty)	N/A	Nothing to disclose
Alisha Pollastri, PhD (faculty)	N/A	Nothing to disclose
Ambereen Mehta, MD, MPH, APHS (faculty)	N/A	Nothing to disclose
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Anthony Mozzone, BBM, CRT	Employee	Promptcare Companies
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Craig McDonald, MD (faculty)	N/A	Nothing to disclose
Crystal Proud, MD (faculty)	Advisor Researcher Speaker	Biogen, Sarepta, Novartis Gene Therapies, Genentech/Roche, Scholar Rock Biogen, Sarepta, Novartis Gene Therapies, Scholar Rock, Astellas, CSL Behring, Fibrogen, PTC, Pfizer Biogen
Daragh Heitzman, MD (faculty)	Consultant, Speaker Researcher	Cytokinetics, Amylyx Amylyx Pharmaceuticals, Cytokinetics, Ra Pharmaceuticals, Biohaven Pharmaceuticals, Clene Nanomedicine, Prilenia Therapeutics, Seelos Therapeutics, Anelixis Therapeutics
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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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