MDA Conference Makes an Impact

In March, more than 2,050 people attended MDA's 2023 Clinical & Scientific Conference at the Hilton Anatole in Dallas.

The four-day event brought together scientists, clinicians, and professionals in neuromuscular disease to present their research, form collaborations, and learn the latest developments in the field. With 1,492 in-person and 567 virtual attendees from at least 33 countries, this was the world's biggest, most comprehensive conference of its kind.

A positive start

MDA President and CEO Donald S. Wood, PhD, kicked off the conference on March 20 and introduced keynote speaker Peter Marks, MD, PhD, Director of the Center for Biologics Evaluation and Research (CBER) at the US Food and Drug Administration (FDA).

Dr. Marks' address, titled "Realizing the Promise of Gene Therapy," detailed how the FDA is implementing several strategies — from international collaboration to accelerated approval

— to help researchers and pharmaceutical companies address the challenges of developing gene therapies for rare diseases and speed the review process.

“We're very committed to advancing the time and availability of gene therapies for all sorts of disorders,” he said.

Legacy Award

The MDA Legacy Award for Achievement in Clinical Research is an annual recognition for outstanding accomplishments in neuromuscular disease research. This year, Stanley Appel, MD, Director of the Ann Kimball and John W. Johnson Center for Cellular Therapeutics at Houston Methodist, presented the award to his longtime mentee Merit E. Cudkowicz, MD, MSc, Director of the Healey Center for ALS and Chief of Neurology at Massachusetts General Hospital.

Dr. Cudkowicz is one of the founders and past co-chairs of the Northeast ALS Consortium (NEALS), a group of over 134 clinical sites in the United States, Canada, Europe, and the Middle East. She has brought innovations to accelerate the development of treatments for people with ALS, including a senior role in the first antisense oligonucleotide treatment for a neurological disorder (SOD1 ALS), adaptive trial designs, and a single IRB approach. Dr. Cudkowicz is the Principal Investigator of the Clinical Coordination Center for the National Institute of Neurological Disorders and Stroke's Neurology Network of Excellence in Clinical Trials (NeuroNEXT). She also launched the first platform trial initiative in ALS, the HEALEY ALS Platform Trial, which is expected to accelerate therapy development in ALS.

“We are at a transformational time in ALS, as well as all the illnesses … in the neuromuscular world,” Dr. Cudkowicz said after accepting the award. “It is so nice to be able to tell [our patients] that we do have options for them today that are on the market, as well many trial options for them.”

Session highlights

This year, the conference’s 30 sessions were organized in seven tracks, giving attendees the opportunity for in-depth learning in topic areas, including ultra-rare diseases, technology, digital outcomes and big data, and gene therapy.

A few notable sessions include:

Practical Considerations in Gene Therapy Track

Updates on Gene Therapy in NMD: Current & Emerging Therapies

Natalie Goedeker, MSN, CPNP (chair)

Emma Ciafaloni, MD, FAAN

Richard Finkel, MD

Alex Murphy, BBM, CRT

Kevin Flanigan, MD

After Dr. Ciafaloni and Dr. Finkel covered current and emerging gene therapies in Duchenne muscular dystrophy (DMD) and spinal muscular atrophy (SMA), respectively, Dr. Murphy addressed pressing questions in gene therapy, including how patients with neutralizing antibodies can be safely and effectively treated with gene therapy and innovative approaches to gene therapy beyond gene replacement. Dr. Flanigan discussed the mechanisms underlying adverse events that have occurred in gene therapy trials and potential interventions to prevent them.

NMD Care Delivery: Care Throughout the Patient Journey Track

Supporting the Patient Throughout Their Journey

Ericka P. Greene, MD, MACM, FAAN (chair)

Rebecca Axline, LCSW-S, CSM, APHSW-C

Keelie Denson, MD

Timothy Lotze, MD

Dr. Lotze addressed barriers and solutions to successful transitions between pediatric and adult care. Dr. Denson, a Neurology resident, spoke about her experience being a caregiver for her father, who had ALS, the importance of seeing patients in the context of their lives, and how multidisciplinary care can address the entire patient journey. Axline spoke about preparing for conversations on palliative and hospice care and helping families understand and make decisions about this care.

DMD Beyond Muscle: A Holistic Approach Track

Making Visible the Invisible: Cognitive and Behavioral Health in Muscular Dystrophy

Mathula Thangarajh, MD (chair)

Natalie Truba, PhD

V. Preethish Kumar, MBBS, MRCP, MRCPE, PhD

Alisha Pollastri, PhD

This session addressed how brain involvement affects systemic outcomes in Duchenne muscular dystrophy (DMD) and provided practical tools for working with DMD patients. Dr. Kumar reviewed neuroimaging studies that can help clinicians understand the role of brain dystrophin isoforms. Dr. Truba talked about emotion and behavior management in boys with DMD and Becker muscular dystrophy (BMD). Dr. Pollastri discussed approaches to behavioral issues, such as collaborative problem-solving, and provided resources to share with parents.

Ultra-Rare Track

MDA Kickstart Program for Ultra-Rare Gene Therapy Development

Sharon Hesterlee, PhD (chair)

Maryna Kolochavina, PharmD, PhD, PMP

Ricardo Maseli, MD

Panelists: Alan Beggs, PhD; Michio Hirano, MD, PhD; Peter Kang, MD; Michael Shy, MD, PhD

In this session, Dr. Kolochavina gave an overview of barriers to developing therapies for ultra-rare neuromuscular diseases, one of which is the lack of agreement on the definition of an ultra-rare disease among key stakeholders. Dr. Hesterlee introduced MDA's new Kickstart Program, designed to identify an ultra-rare neuromuscular disease that the MDA's research team can impact by providing the drug developer with funding and expertise to help it move to the next step. The first disease selected for MDA Kickstart is congenital myasthenic syndrome with episodic apnoea (CMS-EA), which Dr. Maseli presented on. These talks were followed by a panel discussion covering the roadblocks to developing ultra-rare disease treatments, looking for opportunities to develop therapies that will have big impacts when dealing with small patient numbers, and repurposing drugs as an efficient way to develop new therapies for some diseases.

Abstracts and posters

The conference concluded with a full day of abstract presentations, featuring more than 50 oral presentations in the following categories: Insights Into Neuromuscular Disease Mechanisms, Improving Diagnostics and Variant Interpretation, Novel Therapeutic Strategies, Patient Registries and Databases, and Clinical Trial Updates.

In addition, 265 posters were presented during poster sessions in the exhibit hall, and 236 virtual posters were presented online.

CALL TO ACTION

If you missed the 2023 MDA Clinical & Scientific Conference, register to watch it on-demand and browse the abstract library at mdaconference.org. Look for information to come about the 2024 MDA Clinical & Scientific Conference in Orlando.