2022 MDA Clinical & Scientific Conference Highlights

An engaged audience gathered in person for informative sessions

As the coronavirus recedes from world headlines, organizations are resuming in-person meetings and events — and MDA is no exception. From March 13-16, more than a thousand people gathered in Nashville, Tennessee, for MDA's 2022 Clinical & Scientific Conference.

"It's a delight to meet again in person," said MDA President and CEO Don Wood, PhD, in a welcome address. "For many of you, it's the first time on a plane in two years."

In fact, the four-day event held at Nashville's Gaylord Opryland Resort & Convention Center was the world's largest, most comprehensive meeting of neuromuscular disease researchers, physicians, allied health professionals, investors, and advocates, with 1,029 in-person attendees and an additional 691 virtual participants via livestream, representing 15 countries.

The conference featured 32 sessions with one full day of clinical trial updates, as well as more than 120 speakers, 150 posters, 30 exhibitors, 10 industry forums, 100 virtual posters, and 40 virtual exhibits.

Recognizing progress

Dr. Wood delivered a welcome address on March 14, remarking on the progress that has been made in developing neuromuscular disease therapies and the promise of continuing advances. “This is an exciting time in neuromuscular disease research … a time when we’re developing a whole new approach: genetic medicine,” he said. “Most of the diseases we cover are genetic in origin. And where there were no treatments 15 years ago, we now have 15 or 20 FDA-approved treatments, many of which originated with research funded by the MDA.”

Dr. Wood introduced Amy Shinneman, 47, MDA’s new National Ambassador, who, along with current National Ambassador Ethan LyBrand, 12, will represent families living with neuromuscular diseases.

Amy, who lives with Bethlem myopathy, presented MDA’s first Legacy Award for Achievement in Clinical Research — a new annual recognition for outstanding accomplishments in neuromuscular disease research — to Carsten Bönnemann, MD, of the National Institute of Neurological Disorders and Stroke, a division of the National Institutes of Health (NIH).

Dr. Bönneman’s accomplishments include identifying beta and delta sarcoglycan genes as causes of limb-girdle muscular dystrophies (LGMD); developing preclinical animal models for congenital muscular dystrophies (CMD), which are now used to test exon-skipping therapies; establishing natural history and outcome measures for congenital myopathies towards clinical trials; and conducting the first adeno-associated virus (AAV) gene therapy trial for giant axonal neuropathy in humans.

Notable sessions

This year’s MDA Clinical & Scientific Conference was characterized by significant attendee engagement and poignant moments amid the educational sessions covering timely topics, such as telehealth learnings, new gene discoveries, and personalized medicine, as well as diversity in research and ethics in neuromuscular disease care.

Below are some highlights.

When Gut Feelings Aren’t Helping, Revisited: Navigating Complicated Ethics in Neuromuscular Care in 2022
John Brandsema, MD (Chair)
Bakri Elsheikh, MD
Brian Jackson, MD
Julie Parsons, MD

Clinicians presented six ethically complicated cases from neuromuscular practice, touching on end-of-life issues, pain management, access to critical care resources, and more. The panelists had an open discussion about how to navigate those cases and similar situations.

Hot topics in NMD:

Latest Developments Across the NMD Registry Data Landscape

Rayne Rodgers, MPH (Co-Chair)
Elisabeth Kilroy, PhD (Co-Chair)
Alexandre Bétourné, PhD, PharmD, PMP
Russell Butterfield, MD, PhD
Sarah Emmons
Nicholas Johnson, MD, MS-CI, FAAN
Allison Moore
Paul Strumph, MD

This session provided updates on MDA's MOVR Data Hub (neuroMuscular ObserVational Research), as well as MD STARnet (Muscular Dystrophy Surveillance, Tracking and Research Network), RDCA-DAP (Rare Disease Cures Accelerator — Data and Analytics Platform), the Jain Foundation's Dysferlin Registry, and GRIN (Global Registry for Inherited Neuropathy). Representatives from each organization presented the latest innovations in collecting and analyzing real-world data.

Promoting Diversity in Research

Susan Apkon, MD (Chair)
Jay Griffin
Gisel Lopez
Mark Terrelonge, MD, MPH

Panelists detailed the current state of diversity in clinical research, which is lagging, especially in rare disease clinical trials. They also shared their experiences with outreach efforts and suggestions for improving those efforts. In a powerful moment, Jay Griffin, a Black father of a boy with Duchenne muscular dystrophy (DMD), spoke about his family's experience with access barriers and eventual enrollment in a clinical trial. In conclusion, he told the audience of medical scientists, "We are not asking you to carry our burden … but your voice has to be louder than ours. You have to be the cheerleader, coming to where we are."

Impact of Gene Transfer Therapy on Long-Term Multidisciplinary Care

Emma Ciafaloni, MD (Chair)
John Brandsema, MD
Julie Parsons, MD
Edward Smith, MD

This session addressed how newborn screening and gene therapy are transforming the neuromuscular disease treatment landscape, introducing new opportunities and challenges. In this landscape, specialized neuromuscular clinics, multidisciplinary care, and working toward equitable access to novel gene therapies are more important than ever.

Translational research:

Lab to Life: LGMD
Nicholas Johnson, MD, MS-CI, FAAN (Chair)
Qi Lu, PhD, MD
Melissa Spencer, PhD
Conrad Chris Weihl, MD, PhD

This session addressed how clinicians and geneticists are working together to resolve variants of unknown significance and detailed advances in gene replacement therapy.

Lab to Life: FSHD
Jeffrey Statland, MD (Chair)
Scott Harper, PhD
Peter Jones, PhD
Michelle Mellion, MD
Stephen Tapscott, MD, PhD

This session explored building models of progression to instruct trial design and improve care, developing new animal models, and implementation of targeted therapies in clinical trials.

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Lab to Life: MG
Henry Kaminski, MD (Chair)
Petra Duda, MD, PhD
Sara M Jones, MD
Linda Kusner, PhD
Meridith O'Connor, MSW

This session reviewed the journey of myasthenia gravis treatments from bench to bedside, as well as the journey patients take from diagnosis to treatment, and ongoing challenges to developing treatments and providing access to those treatments.

Translational research (continued):

Lab to Life: Congenital Myopathies/Dystrophies
Chamindra Konersman, MD (Chair)
Alan Beggs, PhD
Russell Butterfield, MD, PhD
Michael W. Lawlor, MD, PhD
Dimah Saade, MD

This session presented an overview of the diagnostic approach to congenital myopathies and muscular dystrophies, case studies, and current clinical trials for these diseases.

View sessions on-demand

Recordings of the 2022 MDA Virtual Clinical & Scientific Conference are available to watch on-demand for those registered for the conference. Visit 2022mdavirtualconference.org to view sessions, the virtual exhibit hall, and the virtual poster hall.

Save the date

The 2023 MDA Clinical & Scientific Conference will be held in Dallas, Texas, March 18-22.