



## MDA Marks 75th Anniversary at 2025 Clinical & Scientific Conference

MDA's 75th anniversary legacy, impact, and momentum were brought to life at the 2025 Clinical & Scientific Conference. In March, more than 2,000 neuromuscular researchers, clinicians, allied healthcare professionals, industry leaders, and advocacy organizations joined us in Dallas, with nearly 500 more participating virtually.

### Keynote speaker and panel discussion

Former FDA Commissioner Robert Califf, MD, MACC, opened the 2025 Conference with the keynote address. A renowned cardiologist and clinical trial expert who led the FDA from 2016-2017 and again from 2022-2025, he currently serves as an Instructor in Medicine at Duke University School of Medicine.

Dr. Califf's keynote address set the tone for the opening day. He offered a candid look at the current landscape of biomedical research and healthcare and highlighted the challenges and opportunities ahead.



Among the challenges Dr. Califf pointed to are a fragmented healthcare system increasingly influenced by profit and the lack of a viable financial and organization model for manufacturing and distributing treatments for rare diseases. He also noted that federal support for biomedical research is waning.

Dr. Califf emphasized the critical role of the research and medical communities in rising to meet these challenges. “This is an opportunity, and it’s people like you who can really make a difference, but you’re going to have to get out there and let your voices be heard,” he told the audience.

The keynote session was followed by a panel discussion moderated by Sharon Hesterlee, PhD, MDA Chief Research officer. Panelists included Dr. Califf; Elizabeth McNally, MD, a researcher at Northwestern University specializing in cardiovascular genetics and inherited heart conditions; Barry Byrne, MD, PhD, a physician-scientist at the University of Florida specializing in gene therapy and neuromuscular diseases; David Allison, CEO of TREAT-NM, an international organization dedicated to advancing neuromuscular disease research and care; and Timothy Miller, MD, Vice President of Enterprise Science and Innovation at ThermoFisher Scientific.

The panel expanded on the issues raised by Dr. Califf and offered a hopeful perspective on what’s working in the neuromuscular disease space. Panelists highlighted the deeply collaborative nature of the neuromuscular field, where researchers, industry, and advocacy organizations are aligned in their pursuit of treatments. Dr. McNally underscored the broader impact of this work: “What we’re doing in rare diseases will make a difference in common diseases. Rare diseases lead the way,” she said.

### **Awards for research impact**

MDA’s Legacy Award for Achievement in Clinical Research recognizes outstanding achievement in neuromuscular disease research or care.

The 2025 MDA Legacy Award was given to Katherine Mathews, MD, a leader in genetic medicine and pediatric neurology at the University of Iowa. She is best known for her early work in helping pinpoint the genetic cause of facioscapulohumeral muscular dystrophy (FSHD), as well as her work in documenting the natural history of Duchenne muscular dystrophy (DMD), the dystroglycanopathies, and Friedreich ataxia (FRDA). She has conducted more than 30 industry-sponsored clinical trials focused on neuromuscular diseases, significantly advancing clinical care and scientific understanding. In addition to her research, Dr. Mathews has played a key role in mentoring the next generation of clinical researchers through her leadership as part of the Iowa Wellstone Muscular Dystrophy Specialized Research Center.

Dr. Mathews spoke at the MDA Conference, reflecting on her career at the University of Iowa, where she found outstanding mentors and stimulating research opportunities. She noted MDA's impact throughout her career, from early support of bench research to her involvement in MDA's grant review process.

Dr. Mathews also presented an overview of the dystroglycanopathies and observations from the Iowa Natural History Study.



This year, MDA presented its first-ever Community Impact in Research Award to a community member. Donavon Decker accepted the award on behalf of himself and his family.

Donavon and four of his siblings were diagnosed with limb-girdle muscular dystrophy type 2D (LGMD2D). In 1999, he volunteered for the first gene therapy trial for a muscular dystrophy, led by Jerry Mendell, MD, of The Ohio State University. The therapy was injected into muscles in his feet. Donavon recalled Dr. Mendell explaining that this was a safety trial and the therapy was not expected to alter his LGMD2D.

Donavon shared fond memories of seeing Dr. Mendell daily during his two weeks in the hospital for the trial. Since that experience, Donavon has worked tirelessly to accelerate research efforts and improve the quality of life for individuals living with LGMD. In 2001, he testified before Congress in support of the MD CARE Act. Years later, his sister June Burney became the first person to receive gene therapy for muscular dystrophy via vascular delivery (into the bloodstream). Donavon also went on to cofound Angle Therapeutics, a company focused on developing nonviral gene therapy for LGMD.

Donavon's and his family's dedication have helped drive meaningful progress in our understanding of LGMD, and his advocacy continues to inspire others to engage in and support research across the rare disease community.

## **Educational sessions**

This year's conference featured 39 sessions, 16 of which offered continuing education (CE) credit. Sessions in the Genetic Medicine track, such as "Gene Therapy Updates — Where Are We Today?" and "New Genetic Technologies in Diagnosis and Treatment of Neuromuscular Disease," drew large audiences eager to engage with the latest advancements, funding strategies, and case examples.

The Care Management track featured forward-thinking sessions designed to spark conversation and action. “Changing Policies and Pushing Boundaries” addressed challenges such as high drug costs, cross-state collaboration, and other barriers to delivering optimal patient care. “Preparing for Tomorrow’s Leaders & Fostering Diversity” took an interactive approach, prompting audience discussions around the importance of representation in medicine and strategies to increase diversity in clinical trials and the neuromuscular field. Attendees broke into small groups to discuss their own experiences and insights, followed by expert panel commentary grounded in real-world experience from their practices.

MDA is also advancing the field by building strategic partnerships that drive innovation in care and research. The CE-accredited session “Pompe Disease: Mechanisms, Therapeutic Advances, Advocacy, and Integrated Care Approaches” was presented in Partnership with TREAT-NMD, a global network of experts in the neuromuscular field. This session covered key topics, from historical insights into enzyme replacement therapy (ERT) landscape to the role of biomarkers in disease management and emerging next-generation therapies. Two individuals living with Pompe also shared their diagnostic journeys and perspectives, offering powerful firsthand insight into the patient experience.

### Allied healthcare providers

Allied healthcare providers who attended this year’s conference enjoyed programming tailored to their interests and valuable opportunities to connect and learn from peers. On Sunday, March 16, nearly 50 attendees participated in the Allied Health Workshop, called “Shifting Care for Shifting Needs: Coordinating Care and Increasing Quality of Life via Assistive Devices.”

The Allied Health track also featured sessions on cardiopulmonary care, nutrition, and physical therapy. MDA remains committed to offering robust allied health programming at future conferences.



## View sessions online

[Access to MDA's 2025 Clinical & Scientific Conference program recordings are available for purchase here.](#)

Please note: Access to the platform will be provided within 3-5 business days after purchase.

## 2025 MDA Clinical & Scientific Conference by the Numbers

- 2,509 total attendees
  - 2,012 in person
  - 497 virtual
  - 41 countries represented
- 8 tracks
- 39 sessions
- 207 speakers
- 263 in-person posters
- 379 virtual posters
- 56 oral poster presentations
- 56 exhibitors
- 20 Patient Advocacy Pavilion participants
- 18 industry forums