



## HIGHLIGHTS FROM

## THE 2021 MDA

## VIRTUAL CLINICAL &

## SCIENTIFIC CONFERENCE

The [2021 MDA Virtual Clinical & Scientific Conference](#), held March 15-18, featured more than 140 presenters on the latest clinical care and research advances for more than 40 neuromuscular diseases (NMDs), making it the most comprehensive NMD meeting in the country. More than 1,200 physicians, researchers, and allied health professionals attended.

Livestreamed educational sessions covered timely topics, such as telemedicine, COVID-19, and gene editing, as well as emerging therapies for many different NMDs. Attendees also enjoyed virtual networking and exhibits, poster sessions, and oral presentations from abstract submissions.

*"We are so pleased at the response and participation from our attendees. There was a high level of engagement across the sessions, poster hall, exhibit hall and other activities throughout the week. We are already starting to plan a hybrid in-person/virtual approach for 2022 and look forward to an even more dynamic conference next year."* Meredith Wilson, VP, Professional & Community Education at Muscular Dystrophy Association.

This year's online format allowed participants from all over the United States and beyond to exchange knowledge and share data and best practices in a comfortable, safe environment. Here are some highlights from the Clinical & Scientific Conference's many significant moments.

## Watch Conference Sessions Now ... or Later

Recorded sessions from the 2021 MDA Virtual Clinical & Scientific Conference will be available for on-demand viewing for one year. To access them, visit the [portal](#) here and log in using the email you used to register. If you didn't register previously, you may [register](#) here and have access to the content right away.

### Clinical care-related sessions

#### Barriers to Clinical Trial Participation

*John Day, MD, PhD (Chair)*  
*Katy Eichinger, PT, PhD*  
*Catherine Siener, PT, MHS*  
*Colleen Labbadia, parent*

This session provided an overview of the barriers to clinical trial participation from both the provider and family perspectives. Barriers include the costs of participating — in terms of both time and money — as well as emotional barriers, including fear of experimental therapies, denial of the progression of a disease, and misunderstanding the risks and rewards of clinical trials.

#### Impact of COVID on Neuromuscular Disease Care

*Nicholas Johnson, MD, MS-CI (Chair)*  
*Katherine Mathews, MD*  
*Diana Mnatsakanova, MD*

Presenters shared the challenges of providing good clinical care during a global pandemic, as well as how they found innovative and creative solutions to continue to meet patient and family needs using telemedicine.

#### MOVR & Real-World Evidence

*Rayne Rodgers, MPH (Chair)*  
*Susan Apkon, MD*  
*DeWayne Townsend, DVM, PhD*

As of 2020, the MOVR Data Hub is collecting data from 3,559 participants across seven NMDs. This session covered the first presented use cases of analyzed MOVR data from clinical and preclinical perspectives.

#### Reproductive Health, Family Planning & Parenting

*Elicia Estrella, MS LCGC (Chair)*  
*Judith Jackson, MS CGC*  
*Sharyn Lincoln, MS, CGC*  
*Deodonne Bhattarai, parent*  
*Alexandra Lisi, graduate student*

This session detailed the impacts that neuromuscular disease has on the family unit, including family planning, parenting, and caring for a loved throughout their journey.

### Research-related sessions

#### Ethical Considerations in Gene Therapy Trials

*Kevin Flanigan, MD (Chair)*  
*Diana Bharucha-Goebel, MD*  
*Robert Brown, MD, DPhil*  
*Barry Byrne, MD, PhD*  
*Matthew Kendall, parent*

This session addressed ethical considerations related to gene therapy trials, molecular medicine using AAV gene therapy, and biological therapies in ALS, as well as considerations in children with advanced or severe NMD and a parent perspective.

#### Lab-to-Life: Mitochondrial Diseases Including Friedreich's Ataxia

*Carlos Moraes, PhD (Chair)*  
*Gino Cortopassi, PhD*  
*Anu Soumalainen, MD, PhD*

This session covered how fumarates alleviate lethality and increase mitochondrial functions in animal models of Friedreich's ataxia and Leigh's syndrome, gene therapy for mitochondrial DNA diseases, and the UK perspective.

#### New Biomarkers in Neuromuscular Disease Research

*Elizabeth McNally, MD, PhD (Chair)*  
*Annemieke Aartsma-Rus*  
*Glenn Walter, PhD*  
*Thurman Wheeler, MD*

This session covered biomarker research for DMD, new biomarkers in myotonic dystrophy research, and magnetic resonance biomarkers.

#### Non-viral Delivery in Neuromuscular Disease

*Stanley C. Froehner, PhD (Chair)*  
*Oxana Beskrovnyaya, PhD*  
*James Dahlman, PhD*  
*Melissa Spencer, PhD*  
*Nicholas Whitehead, PhD*

Presenters detailed three non-viral gene delivery systems being developed: delivering RNA to new cells by testing thousands of nanoparticles in vivo, a novel platform for non-viral targeted gene delivery to muscle, and the FORCE platform for delivery of therapeutic oligonucleotides for muscle diseases.