



Highlights From The

MDA 2018 Clinical Conference

MARCH 11-14, 2018 | ARLINGTON, VA.

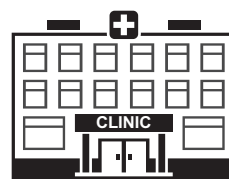
Unprecedented Times The Changing Landscape In Neuromuscular Disease



Participants representing:



academic institutions



MDA Care Centers



biotech



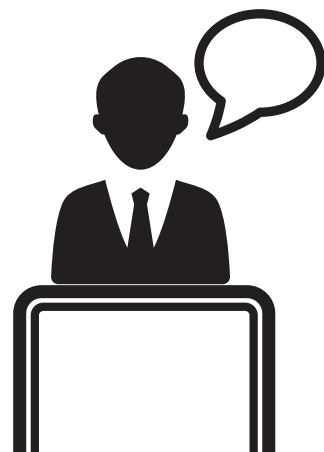
pharmaceutical companies

2018 Clinical Conference by the Numbers

750+
ATTENDEES



FIFTY+
Platform
Presentations



Fourteen
Unique
Sessions



85+
Poster
Presentations



**Four
Days**
collaboration
networking
& exchange



60+
SPEAKERS &
PANELISTS



Up to 17.AMA PRA Category 1 Credits offered

MDA's 2018 Clinical Conference Is In The Books!

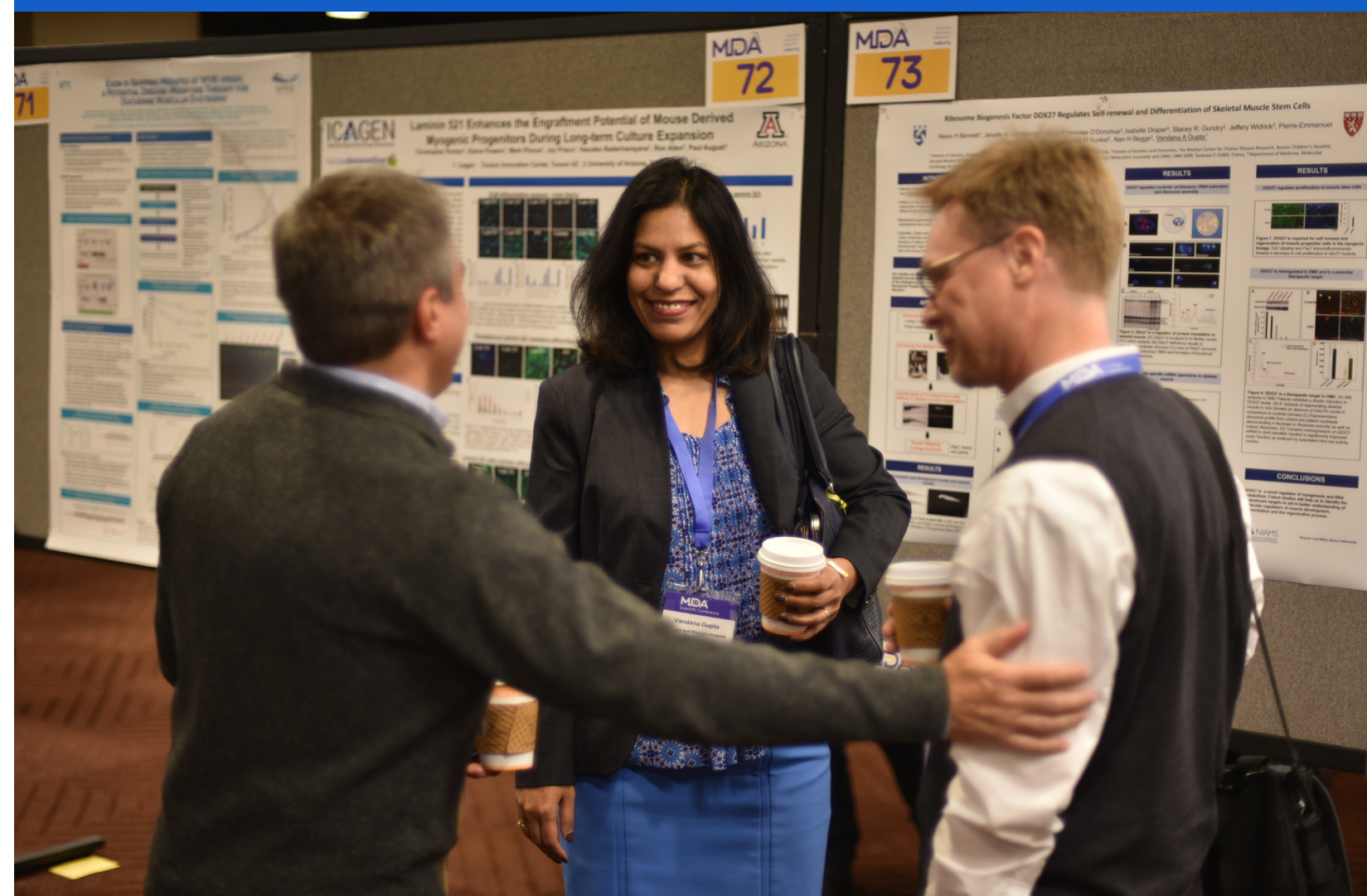
It was a record-setting year for MDA's annual conference series, with more than 700 attendees including MDA Care Center multidisciplinary care providers, as well as medical and scientific experts from academia, private practice, government and industry, gathering in the nation's Capital to share best practices and learn about and discuss the latest scientific and medical advances in neuromuscular disease research and care.

Working together we'll find treatments and cures

At MDA we know that progress doesn't happen in isolation; nor does it move in a straight line. That's why we're committed to bringing together all the key stakeholders in the neuromuscular disease space at events like this. When the world's top neuromuscular experts share and compare information, tools, resources and results, everyone benefits — their teams, their practices, their labs and their research and, most importantly, individuals and families living with neuromuscular disease.

If you joined us for those four jam-packed days in March, we hope you'll enjoy this highlights report. If you weren't able to make it this year, check out some of the key takeaways and see what you can look forward to in 2019!

Communication • Collaboration • Networking



MDA[®]



MDA is founded by a group of interested parents and relatives of children with muscular dystrophy as a dedicated partnership between scientists and concerned citizens aimed at conquering neuromuscular disease

MDA awards its first research grant, for \$1,500, to neuromuscular disease researcher Ade T. Milhorat, M.D.



MDA's first summer camp is conducted in Sussex, N.J., as an experiment to allow an opportunity for kids with neuromuscular disease to enjoy an outdoor camp experience

1955

1950

Understanding basic muscle and nerve b

Scientific and Clinical Milestones



Ade T. Milhorat, M.D., a pioneer in neuromuscular disease research, presents the first report on the molecular basis for DMD¹

To Our Strength for Life Supporters...

Thank You!

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COMMITMENT



An Inspiring Keynote

"With clinical translation, you can achieve heights that you didn't believe were achievable," said Jerry Mendell, M.D. "By and large, what we've learned from one trial, we've translated to another."

Keynote Speaker Jerry Mendell, M.D., founder of the Clinical Translational Gene Therapy Program at the Research Institute at Nationwide Children's Hospital, opened the conference with an inspiring talk about incredible progress underway in the development of gene therapy for neuromuscular diseases.

Mendell was the principal investigator on a November 2017 article on an SMA gene therapy trial in which the experimental SMA gene therapy AVXS-101 showed unequivocal efficacy. Of the 15 infants who participated in the trial, all have achieved event-free survival for more than 20 months and many have achieved motor milestones that they otherwise wouldn't have been expected to achieve. Babies who would have been expected to die before reaching 2 years old are now holding up their heads. Sitting up. Breathing with minimal or no assistance. Even crawling and, in some cases, walking.

"Our experience in SMA has gone a long way," Mendell said, emphasizing that learnings about gene delivery in SMA have direct application now to DMD and perhaps, in the future, other forms of muscular dystrophy.

WHAT'S NEW



After the official welcome and opening remarks, monday’s morning sessions offered a roundup of the latest news and advances in neuromuscular disease — including als (amyotrophic lateral sclerosis), duchenne muscular dystrophy, facioscapulohumeral muscular dystrophy (fshd), spinal muscular atrophy (sma), myotonic dystrophy (dm) and myasthenia gravis (mg).



Amanda Haidet-Phillips

“It’s been so exciting to see at this conference the results of these therapies that have been developed through basic research,” MDA Scientific Officer Amanda Haidet-Phillips said. “It’s been a long road working to understand, to find the causes, the genetic causes of disease, understand how those genes lead to the development of pathology, understand how they’re spliced. We have spent years trying to figure out what’s the best way to develop and to deliver genes using viruses, what’s the best virus to use and how to deliver it. To see it all coming together, all of these years of research, at a clinical conference and seeing these therapies being tested now in patients is really incredible.”

Charles Thornton

DM

Charles Thornton, M.D., noted, “We are making rapid headway” in DM research and care.



Kathryn Wagner

DMD

Kathryn Wagner, M.D., Ph.D., noted: “We are potentially looking at some game-changing strategies for Duchenne.”

Therapeutic strategies currently approved or under development for DMD include immune modulators, anti-myostatin drugs, antisense oligonucleotides and gene therapy approaches.

James D. Berry

ALS

“The really profound answer to what’s new in ALS is, everything is new in ALS,” James D. Berry, M.D., M.P.H., said.

Promising therapeutic strategies under development include antisense oligonucleotides to degrade SOD1 RNA and knock

James Howard

MG

“This is a great time to be involved in neuromuscular disease,” said James F. Howard Jr.

James F. Howard Jr., M.D., FAAN, noted that over the lifetime course of the MG, we are now able to reduce incidence of early death and improve quality of life.

down SOD1 protein expression in the brain and cerebrospinal fluid; AAV-delivered SOD1 gene therapy to knockdown SOD1 protein; the mesenchymal stem cells secreting neurotrophic factors (MSC-NTF) cell therapy; immunomodulation; and combination therapy.

PERSONALIZED MEDICINE



Monday’s afternoon sessions focused on advances in personalized medicine, including the latest in precision therapies, genomics, digital health, and MDA’s MOVR initiative.



Grace Pavlath

MDA MOVR DATA HUB

“MOVR is designed to be the most comprehensive registry in the neuromuscular disease space,” said MDA senior vice president and scientific director Grace Pavlath, Ph.D. “This is going to have a big impact on our ability to transform healthcare and also to lead to more drugs being tested in individuals.”

In 2013, MDA launched the U.S. Neuromuscular Disease Registry to better understand how neuromuscular diseases develop and progress, and to identify which treatments lead to the best outcomes in ALS, BMD, DMD and SMA. Now, MDA is expanding the reach of the registry to 50 sites and seven diseases. It has partnered with IQVIA to expand and enhance what is now the MOVR (NeuroMuscular ObserVational Research) initiative with a new infrastructure, electronic medical record data capture, a digital health module, a user-friendly interface for sites to access their own data and state-of-the-art security protection of data.

[Learn more: mda.org/services/neuromuscular-disease-registry](https://mda.org/services/neuromuscular-disease-registry)

Murray Aitken

DIGITAL HEALTH

Murray Aitken, MBA, said it’s anticipated that digital health tools, including mobile health apps and wearable sensors, will accelerate progress for neuromuscular diseases — including in the areas of wellness and prevention, symptom onset and seeking care, diagnosis, condition monitoring and treatment.



Dennis Matthews

“The importance of accurate diagnosis is even more important now because with new treatments that are becoming available, it’s vital that we know specifically what the gene defect is, what the particular form of gene defect is, so that we can personalize and tailor the medical treatments,” Dennis Matthews, M.D., said. “Personalized medicine is actually what’s coming in to the forefront where your specific diagnosis — your specific gene defect — is the one that we’re going to be able to treat.”

Leonela Amoasii

GENE EDITING IN DMD

Leonela Amoasii, Ph.D., discussed a vision for gene editing in DMD.

Eric Olson’s lab is working to permanently correct the most common DMD mutations by using CRISPR/Cas9 to induce exon skipping, in an approach dubbed myoediting. The team has demonstrated correction of a number of DMD mutations in muscle cells derived from patient iPS cells. It now plans to test the strategy in mutation-specific mouse models, followed by preclinical studies in large animal models and clinical testing in DMD patients.

BEST PRACTICES



On Tuesday morning the focus was on best practices in cardiac care, as well as physical therapy, bone health and nutrition as key components of the multidisciplinary approach to treatment of neuromuscular disease.



Elizabeth McNally

CARDIAC CARE

“I can’t state this enough. The cardiologist and the pulmonologist have to work hand-in-hand in treating the neuromuscular disease patient, and that’s across all the muscular dystrophies and especially those that can affect the heart,” Elizabeth McNally, M.D., Ph.D., said. “That’s because fundamentally what we’re dealing with is a shortness of breath situation. If you’re short of breath, that can come from the lungs, that can come from the heart, and it can come from both. It doesn’t have to just be one or the other. You really need your cardiologist and pulmonologist working hand-in-hand.”

NEWBORN SCREENING

Newborn screening (NBS) was explored through a half-day workshop led by MDA Chairman of the Board and international expert in newborn screening R. Rodney Howell, M.D. It included disease-specific presentations as well as an in-depth panel discussion about the current and future state of NBS.

RISE OF TECHNOLOGY AND BIG DATA

An area of intense interest was technology, with presentations in cognitive computing to help researchers pinpoint links between genes, diseases and drugs, and remote delivery of health-related services and information via telecommunications technologies (telemedicine).

Tuesday afternoon the focus shifted to best practices in newborn screening. Augmentative communication, pediatric-to-adult transition, respiratory health and care coordination also were covered in parallel sessions.

TRANSITIONING FROM PEDIATRIC TO ADULT-BASED CARE

Improvements in supportive — and now disease-modifying — treatments are helping people with neuromuscular disease live longer. Now we must consider: How can we help individuals shift from pediatric care to adult care? Conference conversations centered around how to help ensure individuals and families make a smooth transition.



Lauren Elman

“One of the things that can be very hard for a young adult coming into an adult doctor is that they’re leaving behind something that was very comforting, and very helpful and was present for, maybe all of their life that they can remember. Coming into a doctor who knows their former doctor, and their former team, sort of helps make that transition much smoother,” said Lauren Elman, M.D. “I really try to make it clear that I know their old doctor and their old team and that I’ve talked to their doctor about them, and that this is just the next natural step.”



Robert Griggs

“...As we come to the stage where we have treatments for disease, and some evidence that the sooner you start a treatment the more likely the patients are to do well, then it becomes incumbent upon us to find a diagnosis as early as possible and introduce the treatment before the disease starts,” said Robert Griggs, M.D., FAAN”

PROGRESS



INSPIRATION AND INSIGHT



Wednesday's sessions focused on clinical trial updates, with presenters reporting encouraging results for a number of specific current, ongoing or completed studies including trials to test:

- Autologous infusion of expanded Tregs to suppress neuroinflammation in ALS
- AVXS-101 gene replacement therapy in SMA1
- Domagrozumab, a myostatin inhibitor, in DMD
- ATB200, a next-generation rhGAA enzyme replacement therapy, co-administered with the oral pharmacological chaperone AT2221, in Pompe disease
- Spinraza in presymptomatic SMA
- Golodirsen, a phosphorodiamidate morpholino oligomer (PMO), in DMD
- Omigapil in LAMA2 and COL6-related dystrophy
- The utility of glutamate dehydrogenase (GLDH) as a liver-specific biomarker in DMD
- AMO-02 (tigeglusib) in DM1



Barry Byrne

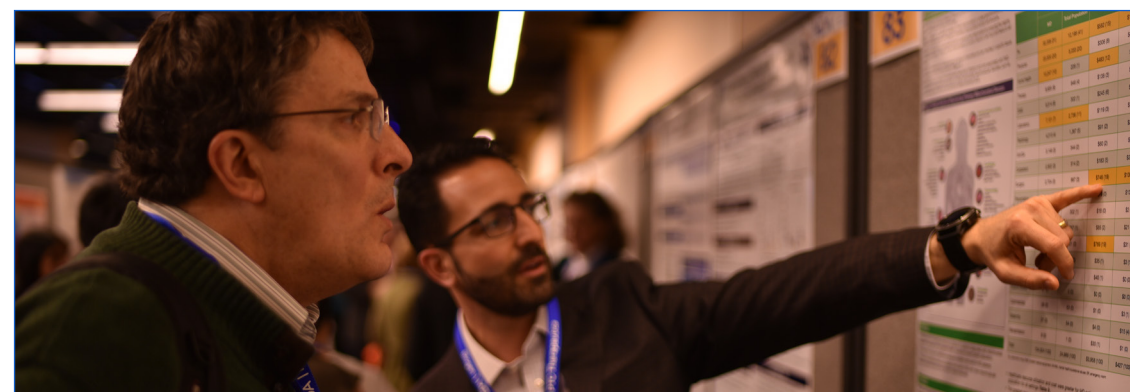
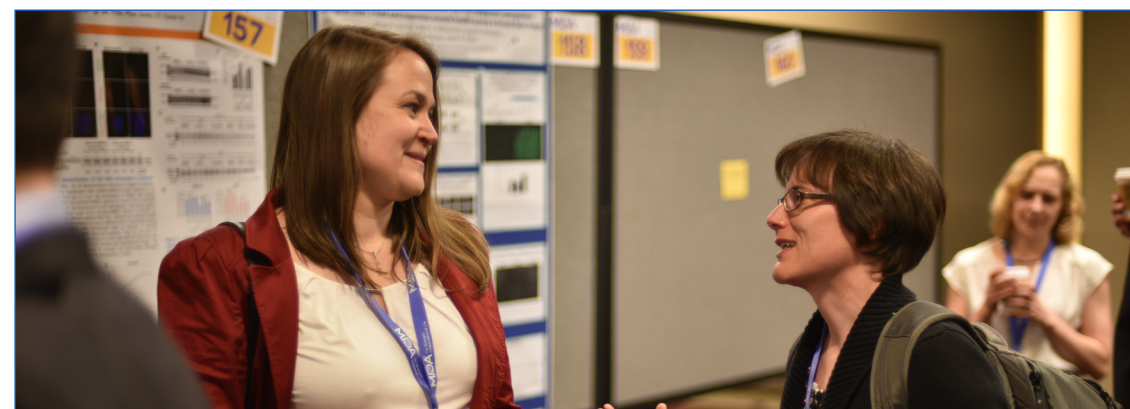
"I think it's an important evolution of the MDA care centers to include not only expertise in clinical care, but full integration with research opportunities for patients," said Barry Byrne, M.D. "The usual clinical care activities have to be excellent, but we also have to offer to patients the opportunity to participate in cutting edge research studies that will really change the trajectory of their disease. So, I think the care center is now the nucleus of that activity and that's why I'm excited that we have such an active group in both expert clinical care and research."



Laura Hagerty

"There are a lot of new technologies that are being described at this conference, including new platform technologies for making drugs, new ways for collecting and storing data, and a new push towards doing genetic testing in patients," said Laura Hagerty, Ph.D., MDA scientific program officer. "And I think this is really exciting because the convergence of those, the maturation of those three different things, really means that we're going to be able to push forward with precision medicines in a way that we haven't before."

Networking, collaboration, and conversations around sharing ideas and brainstorming ways to address new opportunities and challenges were the essence of the conference.



SAVE THE DATE

We're looking forward to next year and hope you'll plan to join us for the 2019 MDA Scientific/Clinical Conference!



2019 MDA Scientific/Clinical Conference

April 13-17, 2019

Hyatt Regency Orlando
9801 International Drive
Orlando, Fla. 32819

Please email Pam Bittner at pbittner@mdausa.org
for any questions related to this event

More information will be posted within the next few weeks. Stay tuned!



2018 CLINICAL CONFERENCE SUPPORTERS

The Muscular Dystrophy Association is grateful to the following companies for supporting the 2018 Clinical Conference and continuing medical education program aimed at optimizing care and helping kids and adults with muscular dystrophy, ALS and related life-threatening diseases live longer and grow stronger. Together with our supporters, MDA is fighting to save and improve the lives of families living with neuromuscular disease.

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MDA is leading the fight to free individuals — and the families who love them — from the harm of muscular dystrophy, ALS and related muscle-debilitating diseases that take away physical strength, independence and life. We use our collective strength to help kids and adults live longer and grow stronger by finding research breakthroughs across diseases; caring for individuals from day one; and empowering families with services and support in hometowns across America.

MDA[®]