Scientific Conference
March 19–22, 2017 • Arlington, VA

Advancing Scientific Breakthroughs To Save and Improve Lives

mda.org
#MDASciCon
Muscular Dystrophy Association
Conference Information

Disclosure Statement

It is MDA's policy to ensure objectivity and scientific rigor in all of its educational activities. All participating speakers are required to disclose at the beginning of their presentation any financial relationships related to the subject matter to be discussed. Any such financial relationships of spouse/partner should also be disclosed.

Poster Sessions

Poster judging for trainees takes place on Monday, March 20 (6:45-8:45 p.m.). All trainees should be by their posters during this time. Awardees will be notified via email no later than Tuesday morning, March 21. Poster awards will be announced to the general audience on Wednesday, March 20, 8:30-9 a.m. Poster awardees will each give a three-minute oral presentation during this time, using a maximum of two slides.

Non-trainees should stand by their posters as follows:

Odd numbers: Monday, March 20 (6:45-8:45 p.m.)

Even numbers: Tuesday, March 21 (6:30-8:30 p.m.).

Please make sure to remove your poster from the exhibit hall by 8 a.m. on Wednesday, March 22. MDA will not be responsible for any posters remaining after this time.

Wireless Login Instructions:

1. Please select Wi-Fi Internet Access: ACCELERON
2. Once ACCELERON is selected, it will route you to the HYATT splash page and will request your access code.
3. Please enter: mda2017 (this code allows you to access Wi-Fi in all meeting spaces).
4. Press enter.
5. You will see the Hyatt Crystal City home page, which indicates that you have gained access to the Wi-Fi.

Conference Evaluation:

Please complete the survey at: surveymonkey.com/r/2017_mda_scientific_conference
Welcome to the 2017 MDA Scientific Conference

Thank you for joining us at the 2017 MDA Scientific Conference. We're excited to have you with us as we work collectively toward advancing scientific breakthroughs to save and improve lives.

We're gathering at a special time in the history of MDA and the neuromuscular disease field, as we've recently seen three drugs, all developed and tested with critical support from MDA, be granted FDA approval to treat diseases in our program. Three drugs in six months!

Following these successes, our sense of urgency to find treatments for all the diseases under our umbrella has only been heightened, our efforts redoubled. We are certain that these approvals will provide inspiration and motivation for academics, biotechs, pharmaceutical companies and other key industry stakeholders to galvanize their efforts and spur advances in drug development across the board. Perhaps even more importantly, these new treatment options have brought an even greater sense of hope to the individuals and families we serve, those who are counting on us — on all of us — to drive the development of therapies and cures.

Your participation over the next three days is proof of your commitment to working together toward our common goals, not only to keep up this newfound momentum but to accelerate progress toward the solutions so many children and adults are waiting for.

Our 2017 conference is focused on increasing knowledge of disease causes, identifying new therapeutic targets and innovative technologies, and discussing new advances in preclinical and clinical research — all aimed at accelerating drug development and targeted treatments for neuromuscular diseases. We plan to cover a broad range of topics, with ample opportunity for dialogue and exchange of ideas.

Because of you and the work you are doing throughout the United States and around the world, individuals with neuromuscular diseases are living longer and growing stronger. We truly appreciate your passion and partnership as we work together for strength, independence and life. Thank you for all you do every day to help accelerate progress in our fight to free individuals — and the families who love them — from the harmful effects of neuromuscular diseases.
Matthew Porteus, M.D., Ph.D.
Stanford University
Genome Editing for Genetic Diseases: Challenges and Opportunities

Matthew Porteus is an associate professor at Stanford. His research program has made important discoveries in advancing the field of genome editing, including the first use of genome editing using engineered nucleases in human cells and optimizing the use of the CRISPR/Cas9 system in primary human stem cells. As an attending physician, Porteus cares for children undergoing bone marrow transplantation. His goal is to combine his research and clinical interests to bring innovative curative therapies to patients.

Mark Schnitzer, Ph.D.
Stanford University
In Vivo Imaging of Human Sarcomere Twitch Dynamics in Individual Motor Units

Mark Schnitzer is an associate professor at Stanford University and an investigator of the Howard Hughes Medical Institute. His research concerns the innovation of novel optical imaging technologies, and their use in the pursuit of understanding neural circuits and muscle microstructure and contractile dynamics. His approaches are being used clinically to better visualize how muscle structure and function is compromised in the context of neuromuscular diseases.

Enabling Technology Speaker

Sandeep Robert Datta, M.D., Ph.D.
Harvard Medical School
Deep Phenotyping Using Motion Sequencing

Sandeep Robert Datta is an assistant professor at Harvard Medical School. Datta’s lab focuses on understanding how the brain transforms information about sensory cues into patterns of motivated action. This work involves studying genes involved in detecting odors, revealing patterns of neural activity that encode sensory maps of the outside world, and probing the fundamental statistical structure of behavior itself. His innovative approach for studying the modular structure of behavior can be used as a sensitive read-out of the effects of disease mutations and potential therapeutics.
Sunday, March 19, 2017

3 – 6:30 p.m. Registration ............................................................... Regency Foyer

Keynote Speakers ................................................................. Regency CD Ctr EF

5 – 6:30 p.m. Genome Editing for Genetic Diseases: Challenges and Opportunities
Matthew Porteus, M.D., Ph.D., Stanford University

In Vivo Imaging of Human Sarcomere Twitch Dynamics in Individual Motor Units
Mark Schnitzer, Ph.D., Stanford University

6:30 – 8:30 p.m. Champions Reception ............................................. Independence Center A

Monday, March 20, 2017

6 – 6:45 a.m. MDA Team Momentum Morning Fun Run (optional) .............. Lobby

7 – 8 a.m. Breakfast ................................................................. Independence Center B

Opening Session ................................................................. Regency CD Ctr EF

8 – 8:30 a.m. Welcome and Opening Remarks

Session 1: Genetics/Epigenetics/Gene Discovery ..................................... Regency CD Ctr EF

Session Chairs: Robert Brown and Monkol Lek

8:30 – 9 a.m. Systematic Identification of Causal Mutations in Severe Muscle Diseases Using Various Genomic Technologies
Monkol Lek, Ph.D., Massachusetts General Hospital

9 – 9:30 a.m. Collagen VI-Related Dystrophy — Mutation-Specific Approaches to Dominant Mutations
Carsten Bönnemann, M.D., National Institutes of Health

9:30 – 9:45 a.m. The Role of Harakiri, a Mitochondrial Apoptosis Mediator, in Maintaining Membrane Stability of Myositis Muscle
Jessica Boehler, Children’s National Health System

9:45 - 10 a.m. Epigenetic Silencing in Friedreich's Ataxia is Caused by Hypermethylation of the FXN CpG Island Shore
Sanjay Bidichandani, MBBS, Ph.D., University of Oklahoma Health Sciences Center

10 – 10:30 a.m. Coffee Break ...................................................... Independence Center A

10:30 – 11 a.m. TBD
Robert Brown, M.D., Ph.D., University of Massachusetts Medical School

11 – 11:15 a.m. Targeting the Long Non-Coding RNA SMN-AS1 as a Treatment for Spinal Muscular Atrophy
Constantin d’Ydewalle, Ph.D., Johns Hopkins School of Medicine

11:15 – 11:30 a.m. Mutations in INPP5K Cause a Novel Syndrome with Congenital Muscular Dystrophy, Intellectual Disability and Cataracts
M. Chiara Manzini, Ph.D., George Washington University
Facioscapulohumeral Muscular Dystrophy is a Model Epigenetic Disease
Peter Jones, Ph.D., University of Nevada

Enabling Technology
Deep Phenotyping Using Motion Sequencing
Sandeep Robert Datta, M.D., Ph.D., Harvard Medical School

Session 2: Precision Medicine Approaches
Session Chairs: Adrian Krainer and Matthew Wood

Targeted Antisense Therapy for Spinal Muscular Atrophy
Adrian Krainer, Ph.D., Cold Spring Harbor Laboratory

Translating DUX4-Targeted RNAi Therapy for Facioscapulohumeral Muscular Dystrophy
Lindsay Wallace, Ph.D., Nationwide Children’s Hospital

Dystrophin Induction Following Muscle-Specific AAV-CRISPR/Cas9-Mediated Editing of the Murine Dystrophin Gene
Jeffrey Chamberlain, Ph.D., University of Washington

Advanced Oligonucleotide Therapeutics for Neuromuscular Disease
Matthew Wood, Ph.D., University of Oxford

IMPEDE: Inhibition of Microsatellite Promoted Expression of Deleterious Expansions in Myotonic Dystrophy
Andrew Berglund, Ph.D., University of Florida

New Insights on Exercise-Triggered Weakness in Hypokalemic Periodic Paralysis
Steve Cannon, M.D., Ph.D., University of California, Los Angeles

FG Nucleoporin Dysfunction in Amyotrophic Lateral Sclerosis (ALS)
Christopher Donnelly, Ph.D., University of Pittsburgh

Glutamate Anaplerosis as a Mechanism of Metabolic Adaptation in Mitochondrial Diseases
Giovanni Manfredi, M.D., Ph.D., Weill Medical College
## Monday, March 20, 2017

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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<tbody>
<tr>
<td>5:30 – 6 p.m.</td>
<td>Early Cl⁻ and K⁺ Channel Defects in Huntington's Disease Skeletal Muscle Reveal Disrupted Maturation  &lt;br&gt; Andrew Voss, Ph.D., Wright State University</td>
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<tr>
<td>6 – 6:30 p.m.</td>
<td>Discovery of a Persistent Inward Sodium Current in Skeletal Muscle That Contributes to Myotonic Action Potentials  &lt;br&gt; Mark Rich, M.D., Ph.D., Wright State University</td>
</tr>
<tr>
<td>5:15 – 5:30 p.m.</td>
<td>Disruption of Nucleocytoplasmic and Chemokine Signaling and Proteostasis by Loss of the Nucleoporin, Ranbp2, in Motoneurons Causes Amyotrophic Lateral Sclerosis (ALS)-Like Syndromes  &lt;br&gt; Paulo Ferreira, Ph.D., Duke University Medical Center</td>
</tr>
<tr>
<td>5:30 – 5:45 p.m.</td>
<td>Nuclear Envelope Mutations Associated with Emery-Dreifuss Muscular Dystrophy Cause Softer and More Fragile Nuclei  &lt;br&gt; Gregory Fedorchak, Cornell University</td>
</tr>
<tr>
<td>5:45 – 6 p.m.</td>
<td>Regulation of Nucleus-Nucleus Interactions During Myonuclear Movement  &lt;br&gt; Eric Folker, Ph.D., Boston College</td>
</tr>
<tr>
<td>6 – 6:15 p.m.</td>
<td>Rescue of Emerin-Null Myogenic Progenitor Differentiation by Inhibition of Multiple MAP Kinases or Activation of HDAC3  &lt;br&gt; Joseph Ellis, University of the Sciences</td>
</tr>
<tr>
<td>6:15 – 6:30 p.m.</td>
<td>Loss of Proteostasis and Redox Homeostasis in Nuclear Envelope Muscular Dystrophy  &lt;br&gt; Lori Wallrath, Ph.D., University of Iowa</td>
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</tbody>
</table>

6:45 – 8:45 p.m. Poster Session and Reception .................................... Independence Center A  <br> Poster Judging - Trainees  <br> Posters - Non-Trainees (Odd numbers)  

— Dinner On Your Own —
Session 4A: Dystrophin Glycoprotein Complex Diseases
Regency C & D

Session Chairs: Aaron Beedle and Elizabeth McNally

8 – 8:30 a.m.
Evidence of mTORC1 Activity in the Pathogenesis of Dystroglycanopathy-Type Muscular Dystrophy
Aaron Beedle, Ph.D., Binghamton University

8:30 – 8:45 a.m.
Intracellular Signaling Responses that Mediate Membrane Repair in Striated Muscle Can Compensate for Membrane Fragility in Muscular Dystrophy
Noah Weisleder, Ph.D., Ohio State University

8:45 – 9 a.m.
Vamorolone Improves Inflammation Via the GR in Addition to Heart Health Via the MR in mdx Mice
Christopher Heier, Ph.D., Children's National Medical Center

9 – 9:30 a.m.
Molecular Regulation of Muscle Stem Cell Asymmetric Division
Michael Rudnicki, Ph.D., Ottawa Hospital Research Institute

9:30 – 10 a.m. Coffee Break........................................................ Independence A

Session 4B: Motor and Peripheral Neuron Diseases
Regency E & F

Session Chairs: Tania Gendron and Angelo Lepore

8 – 8:30 a.m.
Poly(GP) Dipeptide Repeat Proteins as a Pharmacodynamics Biomarker for the Development of C9ALS Therapies
Tania Gendron, Ph.D., Mayo Clinic - Jacksonville

8:30 – 9 a.m.
MFN1 Augmentation to Suppress Toxicity in a Novel Mouse Model of CMT2A
Robert Baloh, M.D., Ph.D., Cedars-Sinai Medical Center

9 – 9:30 a.m.
Dissecting DNA Methylation Dynamics During the Development and Function of Human Motor Neurons
Evangelos Kiskinis, Ph.D., Northwestern University

10 – 10:30 a.m.
Modifiers of Muscular Dystrophy
Elizabeth McNally, M.D., Ph.D., Northwestern University

10 – 10:30 a.m.
Nuclear Transport and Pores in Neurodegenerative Diseases
Jeffrey Rothstein, M.D., Ph.D., Johns Hopkins University
Tuesday, March 21, 2017

10:30 – 11 a.m.
Role of Osteopontin in Promotion of DMD Pathogenesis
Melissa Spencer, Ph.D., University of California - Los Angeles

11 – 11:15 a.m.
Exon 51 Skipping Mediated by WVE-210201, a Potential Disease-Modifying Therapy for Duchenne Muscular Dystrophy
Kirsten Gruis, M.D., WAVE Life Sciences

11:15 – 11:30 a.m.
miR-146a Inhibits Dystrophin Production and Promotes Inflammation in Multiple Muscle Disorders
Alyson Fiorillo, Ph.D., Children’s National Medical Center

10:30 – 10:45 a.m.
TMEM184b Controls Axon Degeneration and Neuromuscular Junction Structure and Modulates Autophagy
Martha Bhattacharya, Ph.D., University of Arizona

10:45 – 11 a.m.
Development of a Pharmacologically-Induced Intermediate Mouse Model to Identify Protective Genetic Modifiers of Spinal Muscular Atrophy
Kevin Kaifer, University of Missouri

11 – 11:30 a.m.
Toward Therapeutic Intervention in ALS: Role of Ephrin Signaling in Astrocytes
Angelo Lepore, Ph.D., Thomas Jefferson University

11:30 a.m. – 1 p.m. Lunch ....................................................... Independence Center B

Session 5A: Repeat Expansion Diseases
Regency C & D
Session Chairs: Laura Ranum and Eric Wang

1 – 1:30 p.m.
Regulation of RNA Processing in Myotonic Dystrophy
Eric Wang, Ph.D., University of Florida

1:30 – 2 p.m.
Facioscapulohumeral Dystrophy: Molecular Mechanisms and Therapeutic Opportunities
Stephen Tapscott, M.D., Ph.D., Fred Hutchinson Cancer Research Center

2 – 2:30 p.m.
Inducible and Reversible Phenotypes in a Novel Mouse Model of Friedreich’s Ataxia
Vijayendran Chandran, Ph.D., University of Florida

Session 5B: Protein Misfolding and Turnover
Regency E & F
Session Chairs: Neil Cashman and Rudolph Kley

1 – 1:30 p.m.
Initiation, Propagation and Inhibition of SOD1 Misfolding in Amyotrophic Lateral Sclerosis
Neil Cashman, M.D., University of British Columbia

1:30 – 2 p.m.
Functional Analysis of the PABPN1 Protein Provides Insight into the Mechanisms Underlying Oculopharyngeal Muscular Dystrophy
Anita Corbett, Ph.D., Emory University

2 – 2:30 p.m.
Defining the Role of Skeletal Muscle in X-Linked Spinal and Bulbar Muscular Atrophy: Implications for Therapy Development in Motor Neuron Disease
Albert LaSpada, M.D., Ph.D., University of California, San Diego

2:30 – 3 p.m. Coffee Break ....................................................... Independence A
### Session 5A: Repeat Expansion Diseases
Regency C & D

<table>
<thead>
<tr>
<th>Time</th>
<th>Topic</th>
<th>Speaker</th>
<th>Institution</th>
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<tbody>
<tr>
<td>3 – 3:30 p.m.</td>
<td>RAN Translation Regulated by Muscleblind Proteins in Myotonic Dystrophy Type 2</td>
<td>Laura Ranum, Ph.D.</td>
<td>University of Florida</td>
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<tr>
<td>3:30 – 4 p.m.</td>
<td>Building a Biomarker for Myotonic Dystrophy Type 1 (DM1)</td>
<td>Charles Thornton, M.D.</td>
<td>University of Rochester Medical Center</td>
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### Session 5B: Protein Misfolding and Turnover
Regency E & F

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<tr>
<td>3 – 3:30 p.m.</td>
<td>Regulation of the Ubiquitin-Proteasome Pathway in Normal and Atrophying Muscles and with Exercise</td>
<td>Alfred Goldberg, Ph.D.</td>
<td>Harvard Medical School</td>
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<tr>
<td>3:30 – 4 p.m.</td>
<td>Protein Aggregation in Myofibrillar Myopathies</td>
<td>Rudolf Kley, M.D.</td>
<td>Ruhr-Universität Bochum</td>
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### Workshop 1A: Scientific Careers Outside of Academia
Regency C & D

<table>
<thead>
<tr>
<th>Time</th>
<th>Topic</th>
<th>Moderator</th>
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<tbody>
<tr>
<td>4:30 – 6 p.m.</td>
<td>Scientific Careers Outside of Academia</td>
<td>Amanda Haidet-Phillips</td>
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</tbody>
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### Workshop 1B: How to Move Forward with your Drug Idea: Strategies and Resources for Academics
Regency E & F

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<tr>
<td>4:30 – 6 p.m.</td>
<td>How to Move Forward with your Drug Idea: Strategies and Resources for Academics</td>
<td>Laura Hagerty and Lianna Orlando</td>
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### Poster Session and Reception
Independence Center A

<table>
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<tr>
<td>6:30 – 8:30 p.m.</td>
<td>Poster Session and Reception</td>
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— Dinner On Your Own —
Wednesday, March 22, 2017

7 – 8 a.m. Breakfast ..................................................... Independence Center B

Opening Session. ............................................................. Regency CD Ctr EF

8 – 8:30 am  Advocacy and its Importance on Advancing Neuromuscular Disease Research
Kristin Stephenson, M.H.A, J.D.

8:30 – 9 a.m. Poster Award Announcements and Trainee Presentations

Session 6: Clinical Trials ......................................................... Regency CD Ctr EF
Session Chairs: Laura Hagerty and Lianna Orlando

9 – 9:30 a.m. A Novel Therapeutic Approach in Spinal Muscular Atrophy (SMA): The Clinical Development of Nusinersen
Wildon Farwell, M.D., Biogen

9:30 – 9:45 a.m. Phase 1/2 Clinical Trial of Clenbuterol in Pompe Disease Patients Stably Treated with ERT
Edward Smith, M.D., Duke University Hospital

9:45 – 10 a.m. A Phase 1b/2a Trial of Ibudilast (MN-166-ALS-1201), a Phosphodiesterase Type 4 Inhibitor for Amyotrophic Lateral Sclerosis (ALS)
Benjamin Brooks, M.D., Carolinas Health Care System Neurosciences Institute

10 – 10:30 a.m. Coffee Break  ..................................................... Regency E & F Foyer

10:30 – 10:45 a.m. Trehalose Treatment For Oculopharyngeal Muscular Dystrophy (OPMD): Safety, Pharmacokinetics and Efficacy Signals in an Open Label Phase 2 Trial
Zohar Argov, M.D., Hadassah-Hebrew University Medical School

10:45 – 11 a.m. Effects of Long-Term Treatment With Eteplirsen on Cardiac Function: Left Ventricular Ejection Fraction in Eteplirsen-Treated Patients vs Disease Natural History
Petra Duda, M.D., Ph.D., Sarepta Therapeutics

11 – 11:15 a.m. Preliminary Pharmacokinetic and Safety Data in Patients With Pompe Disease in First-In-Human Study Receiving ATB200/AT2221
Swati Sathe, M.D., Amicus Therapeutics

11:15 – 11:45 a.m. MoveDMD:  Phase 1/2 Trial of Edasalonexent, an NF-κB Inhibitor, in 4- to 7-Year-Old Patients with Duchenne Muscular Dystrophy
Joanne Donovan, M.D., Ph.D., Catabasis Pharmaceuticals

— Conference Concludes —
2017 Scientific Conference Sponsors

The Muscular Dystrophy Association is grateful to the following companies for sponsoring the 2017 Scientific Conference. Together with our sponsors, MDA is advancing the scientific breakthroughs that will save and improve lives.

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MDA Venture Philanthropy is the Muscular Dystrophy Association’s drug development program, exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular diseases.

Who can apply?
Biotechnology, pharmaceutical or other companies are eligible for MVP funding. MVP also may choose to invest in academic projects with a clear path toward clinical development.

What kinds of projects are funded?
MVP funds projects from proof-of-principle studies through phase 2 trials. Projects may include optimization, toxicology, manufacturing and scale-up, pre-IND, phase 1 or early phase 2 trials. For-profit entities are required to contribute funds that match or exceed MDA’s contribution. MDA will negotiate return on investment. Projects are milestone-driven, with funds released according to the completion of pre-agreed-upon sections of the project.

Development Grants are awarded to researchers on the brink of becoming independent investigators, and are intended as seed money to help launch the scientific programs of promising new neuromuscular disease researchers. Development grants total $60,000 per year, for one to three years.

Who can apply?
The applicant should hold a Ph.D., M.D. or other advanced degree, and must be a member of a research team operating under the guidance of a principal investigator working on one of the diseases in MDA’s program. Applicants must have a strong record of achievement with their postdoctoral mentor and be at least 18 months, but no more than 60 months, from receiving their most advanced degree (Ph.D. or M.D.).

What kinds of projects are funded?
Projects should be hypothesis-driven and used to investigate disease mechanism, potential therapeutic targets, animal models, drug screens, etc., for a disease in MDA’s program.

Research Grants are awarded to established investigators. These grants total $100,000 per year, for one to three years. In a very limited number of cases, awards will exceed $100,000/year but pre-approval is required before submission.

Who can apply?
Independent investigators from academic or corporate research institutions are eligible to apply.

Human Clinical Trial Grants are designed to support both clinical trials of compounds already on the market and clinical studies performed at academic medical centers. (Phase 1 and phase 2a trials of novel therapeutics should be submitted as MDA Venture Philanthropy projects.) HCTG grants may last up to three years and are milestone-driven, with funds released according to the completion of pre-agreed-upon sections of the project.

Who can apply?
The applicant should hold a Ph.D., M.D. or other equivalent advanced degree and be an independent investigator.

What kinds of projects are funded?
Human Clinical Trial Grants support clinical trials of repurposed drugs, non-drug interventions, natural history studies and clinical studies performed at academic medical centers. All trials and studies must be of relevance to MDA-covered diseases, FDA-approved (if appropriate), and approved through an Institutional Review Board.
Research Infrastructure Grant

Research Infrastructure Grants fund the development of infrastructure — tools, techniques or services — that will be of use to the neuromuscular disease research community for the purpose of therapy development.

Who can apply?
Independent investigators from academic, non-profit, or other research institutions may apply.

What kinds of projects are funded?
Any research designed to support the development of tools, techniques and services of need to the neuromuscular research community may be funded through this grant type. Recipients of an MDA Infrastructure Grant must develop a plan to make this MDA-funded resource available to the research community, and have a plan for sustaining the infrastructure beyond the period of MDA support. Letters of support from the community are an essential part of the application.

Clinical Research Training Scholarship

MDA has partnered with the American Academy of Neurology and the American Brain Foundation to offer training scholarships to early-stage investigators entering the field of academic clinical neuromuscular research. Awards total $55,000 per year for two years, plus a $10,000-per-year stipend to support education and research-related costs.

Who can apply?
Applicants must have an M.D. and must have completed residency within the past five years of the start date of the award (July 1).

What kinds of projects are funded?
Projects should relate to patient-oriented research or translational research designed to develop treatments or enhance diagnosis for diseases covered under the MDA umbrella.

Conference Grant

MDA supports conferences, meetings, and workshops that facilitate the exchange of scientific ideas and crucial information relevant to diseases in MDA’s program. Awards total up to $10,000.

Who can apply?
Independent investigators from academic, nonprofit, or other research institutions may apply.

What kinds of projects are funded?
Conferences should relate to diseases covered in MDA’s program and can include research conferences, symposiums focused on new techniques or therapeutic strategies, workshops to update clinical care guidelines, or meetings that promote collaboration across diseases.

Grant Cycle Information: RG, DG

Spring Review Cycle

Letter of Intent Due: Dec. 15
Grant Application Due: Jan. 31
Funding Decisions: May/June
Funding Start Date: Aug. 1

Fall Review Cycle

Letter of Intent Due: June 15
Grant Application Due: July 15
Funding Decisions: Nov./Dec.
Funding Start Date: Feb. 1

MVP Letter of Intent due dates are: March 1, June 1, Sept. 1, Dec. 1.

HCTG, RIG and CG operate on rolling deadlines.

CRTS applications are due Oct. 1. Visit www.AAN.com/view/ResearchProgram to apply for these awards.

For general inquiries, please contact grants@mdausa.org.

Muscular Dystrophy Association • mda.org/researchgrants
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.

Joe Akmakjian
2017 MDA National Ambassador