Summer 2015 Grants by State

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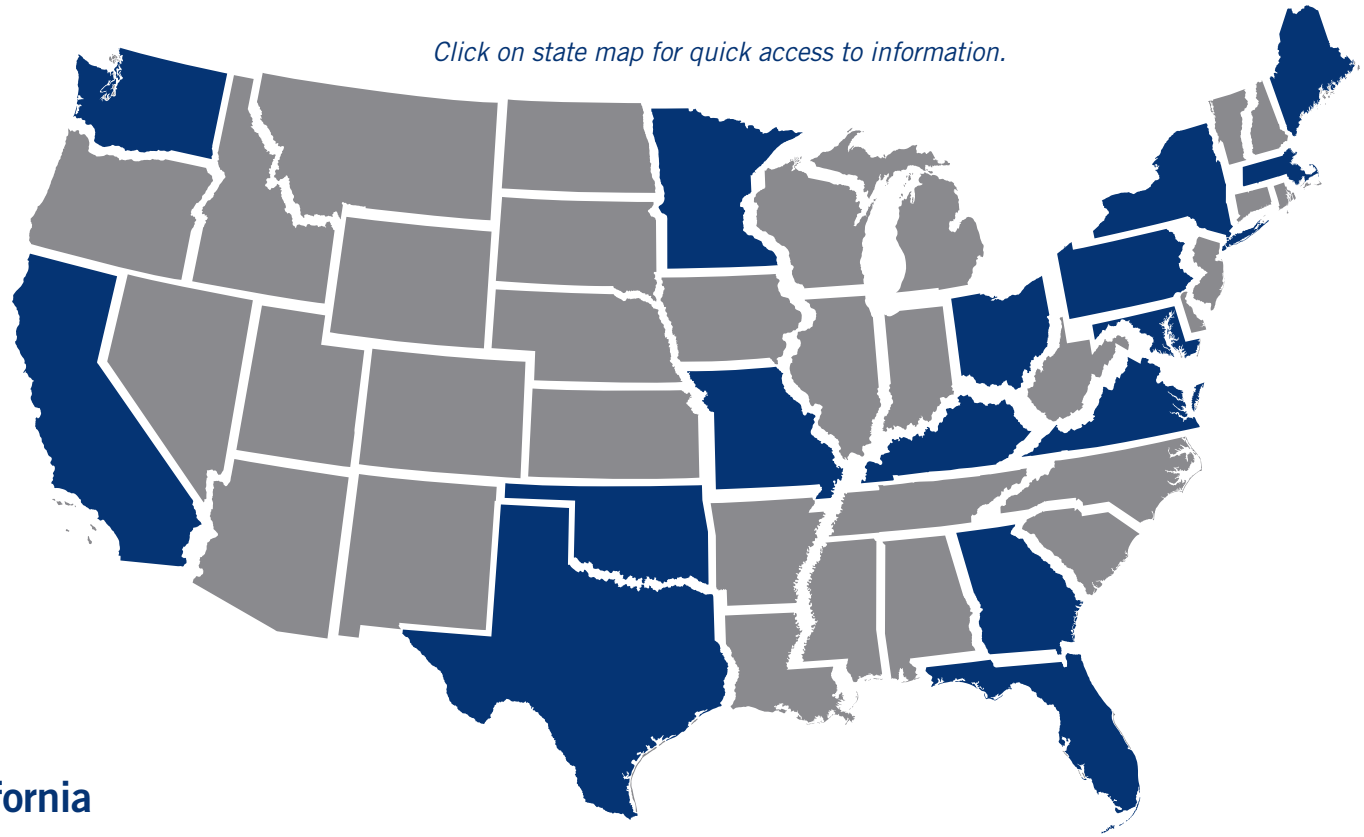

California

- **Robert Baloh**, associate professor-in-residence in the department of neurology at the University of California, Los Angeles, was awarded an MDA research grant totaling $300,000 over three years to study the molecular mechanism of type 2A **Charcot-Marie-Tooth disease (CMT)** due to mutations in the Mitofusin 2 (MFN2) gene.

- **Don Cleveland,** distinguished professor and chair in the department of cellular and molecular medicine at the University of California, San Diego, in La Jolla, was awarded an MDA research grant totaling $300,000 over three years to elucidate the mechanisms of **ALS (amyotrophic lateral sclerosis)** caused by mutations in the Fused in Sarcoma (FUS) gene.

- **Justin Ichida**, assistant professor in the department of stem cell biology and regenerative medicine at the University of Southern California in Los Angeles, was awarded an MDA research grant to elucidate the mechanisms underlying **ALS (amyotrophic lateral sclerosis)** caused by mutations in the C9ORF72 gene.

- **Ricardo Maselli,** a professor in the neurology department at the University of California Davis was awarded an MDA research grant totaling $300,000 over three years to test whether transplantation of stem cells engineered to secrete a needed protein could be a beneficial treatment strategy in **congenital myasthenic syndromes (CMS)**. If studies in a mouse model are successful, testing could advance to patients. Promising results could steer the field to a new therapeutic strategy for CMS.

- **Antoine de Morrée**, a postdoctoral scholar at Palo Alto Veterans Institute for Research and Stanford University in Palo Alto, Calif., was awarded an MDA development grant totaling $180,000 over three years to test a way to stop muscles from making toxic DUX4 protein as a possible treatment for **facioscapulohumeral muscular dystrophy (FSHD).**

- **Thomas Rando,** at Palo Alto Veterans Institute for Research and Stanford University in California, was awarded an MDA research grant totaling $300,000 over three years to develop a mouse model — a so-called "reporter mouse" — that will reflect and quantify degeneration of skeletal muscles in **Duchenne muscular dystrophy (DMD)** and other muscular dystrophies.

Florida

- **Rebecca Willcocks**, an adjunct research assistant scientist in the department of physical therapy at the University of Florida in Gainesville, has been awarded an MDA development grant totaling $171,422 over three years to study magnetic resonance biomarkers in **Duchenne muscular dystrophy (DMD)**.

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Georgia

- **Criss Hartzell**, professor of cell biology at Emory University School of Medicine in Atlanta, Ga., was awarded an MDA research grant to elucidate the mechanisms underlying type 2L **limb-girdle muscular dystrophy (LGMD)**, caused by mutations in the ANO5 gene.

Kentucky

- **Haining Zhu**, a professor in the department of molecular and cellular biochemistry at the University of Kentucky in Lexington, was awarded an MDA research grant totaling $300,000 over three years to study the underlying mechanisms of **ALS (amyotrophic lateral sclerosis)** caused by mutations in the Fused in Sarcoma (FUS) gene.

Maine

- **Robert Burgess**, a professor at The Jackson Laboratory in Bar Harbor, Maine, has been awarded an MDA research grant totaling $300,000 over three years. Burgess and co-investigator Scott Harper, associate professor at Nationwide Children's Hospital Center for Gene Therapy in Columbus, Ohio, will test an AAV gene therapy approach to specifically block the altered form of the GARS gene in a newly developed mouse model for type 2D **Charcot-Marie-Tooth disease (CMT)**.

Maryland

- **Aikaterini Kontrogianni-Konstantopoulos**, an associate professor in the department of biochemistry and molecular biology at the University of Maryland School of Medicine in Baltimore, was awarded an MDA research grant totaling $300,000 over three years to study how the Myosin Binding Protein-C family of proteins may regulate contractile function of skeletal muscle in **distal muscular dystrophy (DD)**.

- **Constantin d'Ydewalle**, a postdoctoral fellow at Johns Hopkins School of Medicine in Baltimore was awarded an MDA development grant totaling $180,000 over three years to test a gene therapy designed to increase levels of SMN protein in **spinal muscular atrophy (SMA)**.

- **Jeffrey Rothstein**, professor of neurology and neuroscience at Johns Hopkins University School of Medicine in Baltimore, was awarded an MDA research grant totaling $300,000 over three years to elucidate the mechanisms underlying **ALS (amyotrophic lateral sclerosis)** caused by mutations in the C9ORF72 gene.

- **Rita Sattler**, associate professor of neurobiology at Barrow Neurological Institute, Dignity Health – St. Joseph's Hospital in Phoenix, was awarded an MDA research grant totaling $300,000 over three years to elucidate the mechanisms underlying **ALS (amyotrophic lateral sclerosis)** caused by mutations in the C9ORF72 gene.

Massachusetts

- **Shinichi Takayama**, a research associate professor at Boston University School of Medicine, was awarded an MDA grant totaling $300,000 over three years to identify potential therapeutic targets in **limb-girdle muscular dystrophy (LGMD)**. He will study the scaffolding protein BAG3 and its interactions with proteins implicated in myofibrillar myopathies and LGMD.

Minnesota

- **James Ervasti**, professor in the department of biochemistry, molecular biology & biophysics at the University of Minnesota in Minneapolis, was awarded an MDA research grant totaling $300,000 over three years to develop methods of identifying non-invasive biomarkers that can be measured in all patients across the entire spectrum of **Duchenne muscular dystrophy (DMD)** disease severity.

- **Michael Kyba**, at the University of Minnesota in Minneapolis, was awarded an MDA research grant totaling $300,000 over three years to study the role of satellite cells in skeletal muscle regeneration.

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Minnesota (cont.)

- **DeWayne Townsend,** assistant professor at the University of Minnesota in Minneapolis, was awarded an MDA research grant totaling $300,000 over three years to study the role of oxygen in the **Duchenne muscular dystrophy (DMD)**-affected heart.

Missouri

- Alan A. and Edith L. Wolff Professor of Developmental Biology **Aaron DiAntonio** at Washington University school of Medicine in St. Louis was awarded an MDA research grant totaling $300,000 over three years to identify novel targets to block nerve degeneration in **Charcot-Marie-Tooth disease (CMT)**.
- **Daniel Summers**, a postdoctoral research scholar in the department of genetics at Washington University School of Medicine in St. Louis, was awarded an MDA development grant totaling $180,000 over three years to investigate how activation of a protein called SARM leads to the loss of metabolites that are essential for the health of peripheral nerves in **Charcot-Marie-Tooth disease (CMT)**.

New York

- **Liza Pon**, professor of pathology and cell biology at Columbia University in New York was awarded an MDA research grant totaling $300,000 over three years to study the underlying mechanisms at work in CHKB **congenital muscular dystrophy (CMD)**.

Ohio

- **Kevin Foust**, assistant professor in the department of neuroscience at Ohio State University in Columbus, was awarded an MDA research grant totaling $293,378 over three years to investigate disruption of gut bacteria in **spinal muscular atrophy (SMA)**.
- **Arthur Burghes**, professor of biological chemistry and pharmacology, molecular genetics, and neurology at The Ohio State University Wexner Medical Center in Columbus, was awarded an MDA research grant totaling $188,613 over two years to refine how a genotype can be used to predict the severity of **spinal muscular atrophy (SMA)**.

Oklahoma

- CMRI Claire Gordon Duncan Chair in Genetics and Professor of Pediatrics **Sanjay Bidichandani**, at University of Oklahoma Health Sciences Center in Oklahoma City, was awarded an MDA research grant totaling $300,000 over three years to address clinically and scientifically important questions regarding the use of existing and novel HDAC inhibitors to increase levels of the frataxin protein in **Friedreich's ataxia (FA)**.

Pennsylvania

- **Angelo Lepore**, assistant professor in the department of neuroscience in Sidney Kimmel Medical College at Thomas Jefferson University in Philadelphia, was awarded an MDA research grant totaling $300,000 over three years to examine a new mechanism by which non-nerve cells called astrocytes contribute to motor neuron death in **ALS (amyotrophic lateral sclerosis)**.
- **Diane Merry,** associate professor at Thomas Jefferson University in Philadelphia, was awarded an MDA research grant totaling $300,000 over three years to identify therapeutic opportunities to promote normal androgen receptor function while preventing the toxic effects of polyglutamine expansion in **spinal-bulbar muscular atrophy (SBMA)**.
- **Davide Trotti**, professor of neuroscience at Thomas Jefferson University in Philadelphia, was awarded an MDA research grant totaling $300,000 over three years to elucidate the mechanisms of **ALS (amyotrophic lateral sclerosis)** caused by mutations in the C9ORF72 gene.

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Texas

- **Ronald Haller**, professor of neurology and neurotherapeutics at University of Texas Southwestern Medical Center in Dallas, was awarded an MDA research grant totaling $300,000 over three years to identify the cause and possible effective treatment for the oxidative defect that accompanies blocked glycogen breakdown in **McArdle disease (phosphorylase deficiency)**.

Virginia

- **Montserrat Samso**, assistant professor in the department of physiology at Virginia Commonwealth University in Richmond, was awarded an MDA research grant totaling $300,000 over three years to generate a crystal structure of the ryanodine receptor (RyR1), an intracellular calcium channel, at high resolution and in different conformational states, with and without disease-causing mutations, to allow a better understanding of its function and role in **central core disease (CCD)**.

Washington

- **Donghoon Lee**, a research associate professor in the department of radiology at the University of Washington in Seattle, was awarded an MDA research grant totaling $300,000 over three years to develop imaging biomarkers for **Duchenne muscular dystrophy (DMD)**.

Washington, D.C.

- **Yetrib Hathout,** associate professor in the department of integrative systems biology at Children's National Medical Center in Washington, D.C., was awarded an MDA research grant totaling $300,000 over three years to develop a panel of molecular biomarkers, detectable in blood, for **Duchenne muscular dystrophy (DMD)**.

Grants in other countries

- **Gary Armstrong**, a senior post-doctoral researcher at Université de Montréal in Quebec, **Canada**, was awarded a development grant totaling $177,670 over three years to further our understanding of the synaptic defects that occur in **ALS (amyotrophic lateral sclerosis)**.
- **Colin Crist**, an assistant professor in the department of human genetics at McGill University in Québec, **Canada**, was awarded an MDA research grant totaling $300,000 over three years to investigate a biological mechanism that determines whether skeletal muscle stem cells, which play an important role in muscle regeneration, self-renew or differentiate.
- **Peter Currie**, professor of medicine at Monash University in **Australia**, was awarded an MDA research grant totaling $300,000 over three years to test two therapeutic strategies for laminin alpha-2 deficiency in a zebrafish model of **congenital muscular dystrophy (CMD)**.
- **Addolorata Pisconti**, at the University of Liverpool, **United Kingdom**, was awarded an MDA research grant totaling $300,000 over three years to study how the environment established by fibrosis and inflammation affects the maintenance and regenerative properties of muscle stem cells in **Duchenne muscular dystrophy (DMD)**.
- **Natassia Vieira**, an investigator at Biosciences Institute, University of São Paulo - São Paulo, **Brazil**, was awarded an MDA development grant totaling $180,000 to study a genetic mechanism that appears to compensate for mutations in the gene that causes **Duchenne muscular dystrophy (DMD)**. Vieira will study the mechanism by which overexpression of a protein called Jagged1 protects dogs and fish with a DMD-causing mutation from exhibiting the typical symptoms and progression of the disease and aim to validate it as a new and potentially very exciting new drug target for DMD.