

Disease Code	Number of Grants	Total Award
03.0 DUCHENNE/BECKER MUSCULAR DYSTROPHY	88	\$30,147,138.60
Alexander	1	\$180,000.00
Role of miR-486 in the pathogenesis of Duchenne Muscular Dystrophy	1	\$180,000.00
Asakura	1	\$378,531.00
Angiogenesis-based therapy for muscular dystrophy	1	\$378,531.00
Baum	1	\$405,000.00
The human skeletal muscle cell glycome - structures and functions	1	\$405,000.00
Beavo	1	\$412,500.00
Proposal: Mechanism of sildenafil action in muscular dystrophy	1	\$412,500.00
Bertoni	2	\$813,665.00
Gene Editing of Dystrophin for the Treatment of Duchenne Muscular Dystrophy	1	\$300,000.00
Preclinical Investigation of RTC#13 for the Treatment of DMD	1	\$513,665.00
Blonar	1	\$999,588.00
Small molecule Ryanodine Receptor Modulator for the Treatment of Muscular Dystrophies	1	\$999,588.00
Brack	1	\$353,259.00
Maintenance of the satellite cell pool in murine dystrophic muscle	1	\$353,259.00
Braunstein	1	\$1,000,000.00
Recombinant biglycan for the treatment of Duchenne muscular dystrophy and Becker muscular dystrophy	1	\$1,000,000.00
Burkin	1	\$308,028.00
Laminin-111 protein therapy for Duchenne Muscular Dystrophy	1	\$308,028.00
Chamberlain	1	\$328,628.00
AAV vectors for gene therapy of DMD	1	\$328,628.00
Chaudhuri	1	\$180,000.00
Matrix Conditioning of Mesenchymal Stem Cells to Rescue Muscular Dystrophies	1	\$180,000.00
Chen	1	\$321,489.00
Functional analysis of the small GTPase Rho1 in myoblast fusion in vivo	1	\$321,489.00
Childers	1	\$480,000.00
Dystrophin-deficient cardiomyocytes for high-thruput drug screening	1	\$480,000.00
Choi	1	\$120,000.00
HDAC4 is involved in muscle regeneration; New therapeutic avenue for DMD disease	1	\$120,000.00
Connolly	2	\$994,823.60
Clinical Outcome validation in Non-ambulatory and young Boys/Men with DMD	1	\$651,037.00
Phase 2 Historically Controlled Trial of Corticosteroids in Young Boys with DMD	1	\$343,786.60
Crosbie-Watson	1	\$300,000.00
Evaluation of sarcospan treatment in muscular dystrophy	1	\$300,000.00
Currie	1	\$375,000.00
Small molecule screening in a zebrafish model of Duchenne Muscular Dystrophy	1	\$375,000.00
Darabi	1	\$380,049.00
Optimization of Human ES/iPS based cell therapy for muscular dystrophies	1	\$380,049.00
Davies	2	\$467,566.00

Screening for drugs to increase utrophin levels in DMD	1	\$260,000.00
Utrophin upregulation for treatment of DMD	1	\$207,566.00
Duan	1	\$527,670.00
Improving AAV potency for DMD gene therapy	1	\$527,670.00
Engler	1	\$390,000.00
Mechanically programmed adipose-derived stem cells to treat muscular dystrophy	1	\$390,000.00
Ervasti	1	\$390,000.00
Biophysical Optimization of Therapeutic Dystrophin Constructs	1	\$390,000.00
Fraidenraich	1	\$375,000.00
Pluripotent stem cell-induced corrections in muscle and fat of mdx mice	1	\$375,000.00
Gersbach	1	\$300,000.00
Genetic Correction of Duchenne Muscular Dystrophy with Engineered Nucleases	1	\$300,000.00
Gokhin	1	\$180,000.00
Structure, Regulation, and Function of Gamma-Actin in the Sarcoplasmic Reticulum	1	\$180,000.00
Goldberg	1	\$410,777.00
Protein breakdown in muscle in normal and disease states	1	\$410,777.00
Goldhamer	1	\$375,000.00
Regulation of satellite cell lineage commitment in regeneration and disease	1	\$375,000.00
Griggs	1	\$10,000.00
Novel Molecular Mechanisms of Neuromuscular Disease: Implications for Therapy	1	\$10,000.00
Gussoni	1	\$384,066.00
Melanoma cell adhesion molecule (MCAM) in human myogenic cells	1	\$384,066.00
Hegde	2	\$347,852.00
A comprehensive approach to identifying novel genes associated with NMDs	1	\$262,928.00
A cost effective approach for newborn screening for DMD	1	\$84,924.00
Hinton	1	\$397,596.00
Executive Functions in Boys with Dystrophinopathy	1	\$397,596.00
Ho	1	\$180,000.00
Treatment of mdx/mTR Model of DMD with Human Muscle Stem Cells	1	\$180,000.00
Hoffman	1	\$321,659.00
Asynchronous remodeling: A force driving failed regeneration in DMD.	1	\$321,659.00
Hoshijima	1	\$367,386.00
Genetic treatment of cardio-respiratory failure in muscular dystrophy	1	\$367,386.00
Iwamoto	1	\$405,000.00
Intervention of muscular dystrophy by selective RARgamma agonist	1	\$405,000.00
Khurana	1	\$379,500.00
Utrophin upregulation via microRNA repression as a therapy for DMD	1	\$379,500.00
Kim	1	\$390,000.00
Functional Restoration of Dystrophic Muscle using Bioengineered Cell Patches	1	\$390,000.00
Korneluk	1	\$425,952.00
Role of cIAP1 and cIAP2 in myogenesis and muscular dystrophy	1	\$425,952.00
Liu	1	\$179,912.00

Targeting Smad mediated signaling of TGFbeta family for stem cell therapy of DMD	1	\$179,912.00
Ljubicic	1	\$180,000.00
Dissecting the mechanisms underlying the benefits of novel therapeutics for DMD	1	\$180,000.00
Lynch	1	\$405,000.00
Therapeutic potential of heat shock protein 72 induction in muscular dystrophy	1	\$405,000.00
Martin	1	\$396,000.00
Protein-based GALGT2 therapies for Duchenne muscular dystrophy	1	\$396,000.00
Megoney	1	\$300,000.00
Caspase 3 Limits the Renewal of Activated Satellite Cells	1	\$300,000.00
Mendell	2	\$1,028,000.00
MDA Clinical Network	1	\$918,000.00
Supplemental Support for AVI-4658 Phase II Clinical Trial	1	\$110,000.00
Menhart	1	\$265,251.00
Biophysics of Exon Skipped Dystrophin Rods	1	\$265,251.00
Metzger	1	\$237,868.00
Development and testing of membrane sealants for muscular dystrophy	1	\$237,868.00
Michele	1	\$364,965.00
Reversing nitric oxide synthase dysfunction in muscular dystrophy	1	\$364,965.00
Millay	1	\$180,000.00
Molecular control of mammalian myoblast fusion	1	\$180,000.00
Morris	1	\$375,425.00
The MDA Monoclonal Antibody Resource for Neuromuscular Disease	1	\$375,425.00
Munoz-Canoves	1	\$390,000.00
CELLULAR MECHANISMS OF FIBROSIS DEVELOPMENT IN MUSCULAR DYSTROPHIES	1	\$390,000.00
Nagaraju	1	\$300,000.00
Murine Preclinical Center for Neuromuscular Diseases (MPCNMD)	1	\$300,000.00
Noordermeer	1	\$278,570.00
Elucidating the Synaptic Roles of Dystrophin	1	\$278,570.00
Oishi	1	\$540,000.00
Analysis of human muscle stem cells: Toward therapy for muscular dystrophies	1	\$540,000.00
Olwin	1	\$369,165.00
Identification and Characterization of Satellite Stem Cells	1	\$369,165.00
Partridge	2	\$675,000.00
Measuring the dynamics of muscle growth and disease in the mouse model of DMD	1	\$375,000.00
Role of satellite cells and pericytes in maintenance of dystrophic muscle	1	\$300,000.00
Pavlath	1	\$295,269.00
Mechanisms of Myofiber Branching	1	\$295,269.00
Penton	1	\$180,000.00
Identification of Therapeutics that Improve Skeletal Muscle Regeneration and Ameliorate Skeletal Muscle Atrophy	1	\$180,000.00
Perlingeiro	1	\$390,000.00
DMD IPS CELLS: GENETIC CORRECTION AND MUSCLE REGENERATION	1	\$390,000.00
Puri	1	\$309,336.00

Signal-dependent control of gene expression in satellite cells	1	\$309,336.00
Rafael-Fortney	1	\$380,769.00
Investigation of a new treatment target for heart failure in muscular dystrophy	1	\$380,769.00
Rando	1	\$375,000.00
Mechanisms of Fibrosis in Muscular Dystrophies	1	\$375,000.00
Reyes	1	\$300,000.00
Role of PDGF Receptor alpha signaling in DMD cardiac fibrosis	1	\$300,000.00
Rodgers	1	\$275,482.00
Washington Center for Muscle Biology, Exercise Physiology Phenotyping Core	1	\$275,482.00
Rudnicki	1	\$375,000.00
Molecular Regulation of Satellite Cell Function	1	\$375,000.00
Saba	1	\$392,467.00
Sphingosine-1-phosphate signaling in muscle regeneration and homeostasis	1	\$392,467.00
Sacco	1	\$446,780.00
In utero and neonatal stem cell therapy for Duchenne Muscular Dystrophy	1	\$446,780.00
Shelton	1	\$10,000.00
18th International Congress of the World Muscle Society	1	\$10,000.00
Shi	1	\$180,000.00
Role of MKP-5 in Duchenne Muscular Dystrophy	1	\$180,000.00
Skerjanc	1	\$280,487.00
Enhanced muscle repair with human embryonic stem cells	1	\$280,487.00
Spencer	1	\$375,000.00
Investigation of osteopontin and inflammatory processes in mdx mice	1	\$375,000.00
Stedman	1	\$300,000.00
Pattern Recognition Receptors in Muscular Dystrophy Pathogenesis and Therapy	1	\$300,000.00
Steinman	1	\$308,061.00
Immune Tolerance to AAV and Dystrophin for Gene Therapy	1	\$308,061.00
Sweeney	1	\$278,286.00
Modulation of calcium handling in mouse models of muscular dystrophy	1	\$278,286.00
Taylor	1	\$396,000.00
Muscle development and repair mediated by the BAR-containing Rho GAP, GRAF	1	\$396,000.00
Thomas	1	\$390,000.00
Muscular dystrophy therapy based on small-molecule activators of Ca ²⁺ transport	1	\$390,000.00
Thompson	1	\$330,000.00
Structural studies of myostatin inhibitors	1	\$330,000.00
Wagner	1	\$352,539.00
Myostatin Regulates Fate of Satellite Cells in Dystrophic Muscle	1	\$352,539.00
Wang	1	\$300,000.00
Gene therapy for treating cardiomyopathy in a dog model of DMD	1	\$300,000.00
Wehrens	1	\$313,500.00
Mechanisms underlying arrhythmias and heart failure in Muscular Dystrophy	1	\$313,500.00
Wilton	1	\$300,000.00
Oligomer design & validation for DMD: quantum improvements in exon skipping	1	\$300,000.00

Wiper-Bergeron	1	\$312,422.00
Improving myoblast transplantation outcomes by modulating C/EBPbeta expression.	1	\$312,422.00
Wuebbles	1	\$180,000.00
Laminin-alpha1 fragment and peptide therapy for Duchenne Muscular Dystrophy	1	\$180,000.00