

Funded Projects

Translational Research Program Funded Projects 2003 – Current (completed grants are in gray)

MDA Venture Philanthropy, Corporate, and IND-Planning Grants

| Start and End Date | Company/Institution Investigator | Disease | Amount | Time | Title |
|--------------------------------------|--|--|-------------|---------|--|
| 4/1/2011 – 3/31/2014 | Wake Forest University, Martin Childers, Ph.D. | X-linked myotubular myopathy | \$369,365 3 | years | Gene therapy in canine myotubular myopathy |
| 3/11/2011 – 3/15/2012 | ALS Biopharma, Alan Reitz | Amyotrophic Lateral Sclerosis | \$250,000 1 | year | Development of Heat Shock Protein 70-Based Therapy for Amyotrophic Lateral Sclerosis |
| 3/3/2011 – 12/1/2013 | University of Illinois, Matthew Meriggioli, M.D. | Myasthenia Gravis | \$530,480 | 3 years | GM-CSF in the Therapy of Autoimmune Myasthenia Gravis |
| 2/15/2011 – 11/15/2011 | Glialogix, Mark Moore, Ph.D. | Amyotrophic Lateral Sclerosis | \$268,000 1 | year | Investigation of GLX1112, a Potential Pharmacological Agent for the Treatment of Amyotrophic Lateral Sclerosis, Using the SOD1G93A Mouse, a Standard Transgenic Mouse Model of ALS |
| 1/1/2011- 12/31/2011 | ALS-TDI, Steve Perrin, Ph.D. | Amyotrophic Lateral Sclerosis <i>Through Augie's Quest Initiative</i> | \$2M 1 | year | Development of Therapeutic Interventions for Amyotrophic Lateral Sclerosis |
| 12/22/2010 – 1/15/13 canceled due | Acceleron Pharma, Chris Rovaldi | Duchenne Muscular Dystrophy | \$1.5M | 1 year | A Randomized, Double-Blind, Placebo-Controlled, Multiple |

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| to milestone failure (4/28/11) | | | | | Ascending-Dose Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ACE-031 (ActRIIB-IgG1) in Subjects With Duchenne Muscular Dystrophy |
| 12/1/2010-6/30/2012 | Repligen Corporation James Rusche, Ph.D. | Spinal Muscular Atrophy | \$1,423,247 | 2 years | Development of a DcpS inhibitor for SMA |
| 8/1/2010 – 9/30/2013 | University of California, Los Angeles, Carmen Bertoni, Ph.D. | Duchenne muscular dystrophy | \$476,465.3 | years | Preclinical Investigation of RTC#13 for the Treatment of DMD |
| 4/1/2010 – 3/31/2012 | 4s3 Biosciences Dustin Armstrong, Ph.D. | Myotubular Myopathy | \$260,291 | 2 years | Muscle Targeted Myotubularin 1 for Treatment of Congenital Myotubular Myopathy |
| 2/10/2010 – 6/30/2012 | Nationwide Children's Hospital Jerry Mendell, M.D. | Limb Girdle Muscular Dystrophy | \$458,814 | 2 years | Limb Strength by Vascular Delivery of the Alpha-Sarcoglycan Gene in LGMD2D |
| 1/12/2010 – 11/28/11 cancelled due to milestone failure (3/31/10) | PTC Therapeutics Langdon Miller, M.D. | Duchenne Muscular Dystrophy | \$1M | 1 year | Phase 2a Trial of Ataluren in Nonambulatory Boys with Nonsense Mutation DMD/BMD |
| 1/12/2010 – 12/1/2010 | ReveraGen BioPharma Erica Reeves, Ph.D. | Duchenne Muscular Dystrophy | \$360,000 | 1 year | Preclinical Validation of a Non-hormonal Steroid for Duchenne Muscular Dystrophy |
| 1/1/2010 – 12/31/2010 | ALS TDI Steve Perrin, Ph.D. | ALS Through Augie's Quest Initiative | \$2.5M | 1 year | Development of Therapeutic Interventions for Amyotrophic Lateral |

| | | | | | Sclerosis |
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| 12/15/2010 – 9/30/2010 | Catabasis Pharmaceuticals Michael Jirousek, Ph.D. | Duchenne Muscular Dystrophy | \$150,000 | 1 year | Preclinical Validation of a Small Molecular Anti-inflammatory Compound for Duchenne Muscular Dystrophy |
| 11/1/2009 – 6/20/2010 | Repligen Corporation James Rusche, Ph.D. | Friedreich's Ataxia | \$731,534 | 1 year | Clinical Development of a HDAC Inhibitor for Friedreich's Ataxia |
| 7/1/08 – 6/30/09 | California Stem Cell Chris Airress, Ph.D. | Amyotrophic Lateral Sclerosis | \$200,000 | 1 year | Cell Transplantation Strategy For the Treatment of Amyotrophic Lateral Sclerosis |
| 12/1/2007 – 11/30/2009 | Insmad Melvin Sharoky, Ph.D. | Myotonic Muscular Dystrophy | \$2,087,325 | 2 years | Phase II Clinical Study of rhIGF-I/rhIGFBP-3 in Myotonic Dystrophy Type 1 |
| 12/1/2007 – 11/30/2009 | Repligen Corporation James Rusche, Ph.D. | Friedreich's Ataxia | \$731,534 | 1 year | Clinical Development of a HDAC Inhibitor for Friedreich's Ataxia |
| 11/1/2006 – 10/31/2008 | Askepios Biopharmaceutical, Inc. Jude Samulski, Ph.D. | Duchenne Muscular Dystrophy | \$2,403,370 | 3 years | Phase Ib/II Study of Mini-Dystrophin Gene in AAV Vector |
| 3/1/2007 – 2/28/2010 | ALS TDI Steve Perrin, Ph.D. | Amyotrophic Lateral Sclerosis Through Augie's Quest Initiative | \$18M | 3 years | Identification and validation of therapeutic targets for ALS clinical development |
| 4/1/2007 – 3/31/2011 | Washington University Timothy Miller, Ph.D. | Amyotrophic Lateral Sclerosis | \$1,031,122 | 3 years | Toxicology and phase I studies of antisense ISIS 333611 in familial ALS |
| 3/31/2006 – 4/30/2007 | Translational Genomics Research Institute | Amyotrophic Lateral Sclerosis Through Augie's | \$652,056 | 1 year | Resequencing the Variable Human Genome in Sporadic |

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| | Dietrich Stephan, Ph.D. | Quest Initiative | | | ALS |
| 1/1/2005 – 3/31/2006 | PTC Therapeutics Langdon Miller, M.D. | Duchenne Muscular Dystrophy | \$1.5M | 2 years | PTC124 treatment for Duchenne muscular dystrophy |
| 3/1/2006 – 11/30/2006 | Harvard Medical School Richard Mulligan, Ph.D. | Limb Girdle Muscular Dystrophy | \$300,000 | 1 year | Clinical manufacturing of AAV1 vector in support of a phase I/II gene transfer study in limb-girdle |
| 4/1/2004 – 3/31/2006 | Asklepios Biopharmaceutical, Inc. Jude Samulski, Ph.D. | Duchenne Muscular Dystrophy | \$1,621,172 | 2 years | Phase I/II Study of Mini-Dystrophin Gene in AAV Vector |
| 10/1/2004 – 9/30/2007 | Nationwide Children's Hospital Jerry Mendell, M.D. | Limb Girdle Muscular Dystrophy | \$180,000 | 3 years | Transfer of alpha-sarcoglycan gene to LGMD2D patients |
| 1/3/2003 – 6/30/2006 | University of Pennsylvania H. Lee Sweeney | Duchenne Muscular Dystrophy & Limb Girdle Muscular Dystrophy | \$674,560 | 3 years | Preclinical studies to support clinical trials for muscular dystrophy |
| 1/1/2003 – 12/31/2003 | Duke University Priya Kishnani, | Acid Maltase Deficiency | \$50,000 | 1 year | AGLU01702 & AGLU01602 - Pompe's Trial |
| 1/1/2003 – 12/31/2003 | Cincinnati Children's Hospital Nancy Leslie, M.D. | Acid Maltase Deficiency | \$50,000 | 1 year | Pompe's trial - AGLU01702 and AGLU01602 |
| 1/1/2003 – 12/31/2003 | University of Florida Carolyn Spencer, | Acid Maltase Deficiency | \$50,000 | 1 year | Pompe's trial - AGLU01702 and AGLU01602 |

Translational Research Infrastructure Grants

| Start and End Date | Company/Institution Investigator | Disease | Amount | Time | Title |
|-----------------------|--|-----------------------------------|-----------|---------|--|
| 10/1/2010 – 9/30/2013 | Keele University Glen Morris, Ph.D. | Spinal Muscular Atrophy, Duchenne | \$375,425 | 3 years | The MDA Monoclonal Antibody Resource for Neuromuscular Disease |

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| | | Muscular Dystrophy, Myotonic Dystrophy, & Emery-Dreifuss Muscular Dystrophy | | | |
| 6/3/2010 – 9/31/2011 | Lewin Group Paul Hogan | Spinal Muscular Atrophy, Amyotrophic Lateral Sclerosis, Duchenne Muscular Dystrophy | \$261,050 | 1 year | Burden of Disease Study |
| 6/1/2010 – 5/31/11 | Children's Hospital of Philadelphia David Lynch, Ph.D. | Friedreich's Ataxia | \$332,613 | 1 year | Clinical Research Network for Friedreich's Ataxia |
| 4/1/2010 – 3/31/2011 | Wayne State University Michael Shy, Ph.D. | Charcot Marie Tooth Disease | \$242,877 | 1 year | North American CMT Network |
| 7/1/2009 – 6/30/2011 | Emory University Hadhuri Hegde, Ph.D. | Congenital Muscular Dystrophy, Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Emery-Dreifuss Muscular Dystrophy, & Limb Girdle Muscular Dystrophy | \$444,000 | 2 years | Comprehensive high throughput mutation detection in genes associated with NMD |
| 1/1/2009 – 12/31/2011 | University of North Carolina at Chapel Hill Joe Kornegay, D.V.M., | Duchenne Muscular Dystrophy | \$884,864 | 3 years | Natural History and Immunological Parameters in the GSHPMD Dog |

| | Ph.D. | | | | |
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| 1/1/2009 – 12/31/2009 | Wayne State University Michael Shy, Ph.D. | Charcot Marie Tooth Disease | \$275,000 | 1 year | North American CMT Network |
| 9/1/2008 – 8/31/2009 | Emory University Hadhuri Hegde, Ph.D. | Congenital Muscular Dystrophy, Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Emery-Dreifuss Muscular Dystrophy, & Limb Girdle Muscular Dystrophy | \$200,000 | 1 year | Microarray based mutation detection in genes associated with inherited NMDs |
| 7/1/2008 – 6/30/2011 | Indiana University Indianapolis Tatiana Foroud, Ph.D. | Spinal Muscular Atrophy | \$ 102,346 | 3 years | International Spinal Muscular Atrophy Patient Registry |
| 4/1/2008 – 3/30/2011 | University of Rochester Chad Heatwole, Ph.D. | Myotonic Dystrophies, Distal Muscular Dystrophy, Facioscapulohum eral Muscular Dystrophy | \$ 218,685 | 3 years | The Development and Use of Disease-Specific Instruments for Muscular Dystrophies |
| 10/1/2007 – 9/30/2010 | Robert Jones & Agnes Hunt Hospital Glen Morris, Ph.D. | Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Myotonic Dystrophy, & Emery-Dreifuss Muscular | \$366,064 | 3 years | The MDA monoclonal antibody resource |

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| | | Dystrophy | | | |
| 8/1/2007 – 2/1/2008 | University of North Carolina at Chapel Hill Joe Kornegay, D.V.M., Ph.D. | Duchenne Muscular Dystrophy | \$170,000 | 1 year | MDA Bridging Funds to Support Dog Studies at UNC |
| 3/1/2007 – 5/31/2010 | Children's Hospital of Philadelphia David Lynch, Ph.D. | Friedreich's Ataxia | \$619,662 | 3 years | Clinical Research Network for Friedreich's Ataxia |
| 8/1/2006 – 1/31/2010 | California Pacific Medical Center Robert Miller, M.D. | Amyotrophic Lateral Sclerosis | \$425,005 | 3 years | MDA/ALS web-based database |
| 3/1/2006 – 11/30/2006 | ALS Therapy Development Foundation Daniel Benjamin, Ph.D. | Amyotrophic Lateral Sclerosis | \$165,447 | 1 year | Pharmacokinetic analysis of putative ALS drugs: do they reach the spinal cord? |
| 1/1/2005 – 12/31/2007 | Wayne State University Michael Shy, Ph.D. | Charcot Marie Tooth Disease | \$255,000 | 3 years | CMT North American Database |
| 10/1/2004 – 9/30/2007 | Robert Jones & Agnes Hunt Hospital Glen Morris, Ph.D. | Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Myotonic Dystrophy, & Emery-Dreifuss Muscular Dystrophy | \$321,445 | 3 years | A monoclonal antibody resource for genetic neuromuscular diseases |
| 8/1/2003 – 7/31/2006 | California Pacific Medical Center Robert Miller, M.D. | Amyotrophic Lateral Sclerosis | \$466,322 | 3 years | MDA/ALS Web-based Database |

Clinical Research Training Grants

| Start and End Date | Company/Institution Investigator | Disease | Amount | Time | Title |
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| 7/1/2011 - 6/30/2013 | Massachusetts General Hospital James Berry, M.D. | Amyotrophic Lateral Sclerosis | \$180,000 | 2 years | A Phase I/II Study of a Continuous Infusion of ISIS 333611 in SOD1 Familial ALS |
| 7/1/2011 - 6/30/2013 | University of Rochester Araya Puwanant, M.D. | Myotonic Dystrophy | \$173,400 | 2 years | Disease Progression and Physiological Changes in Myotonic Dystrophy |
| 7/1/2010 - 6/30/2012 | Massachusetts General Hospital Nazem Atassi, M.D. | Amyotrophic Lateral Sclerosis | \$180,000 | 2 years | Comparative effectiveness in ALS symptomatic management |
| 7/1/2009 - 6/30/2011 | University of Utah Russell Butterfield, M.D. | Congenital Muscular Dystrophy & Collagen Disorders | \$ 173,500 | 2 years | Natural history and genetic epidemiology of collagen VI related myopathies |
| 7/1/2009 - 6/30/2011 | Massachusetts General Hospital Anne-Marie Wills, M.D. | Amyotrophic Lateral Sclerosis | \$180,000 | 2 years | Trial of high fat/high calorie diet versus optimal calorie replacement in ALS |
| 7/1/2008 - 6/30/2010 | Stanford University Hans Katzberg, M.D. | Amyotrophic Lateral Sclerosis | \$180,000 | 2 years | Effect of Diaphragm Pacing Stimulation on Sleep Quality in Patients with ALS |
| 7/1/2007 - 6/30/2009 | Columbia University Medical Center Jinsy Andrews, M.D. | Amyotrophic Lateral Sclerosis | \$180,000 | 2 years | David A. Gardner Neuromuscular Research Fellow |
| 7/1/2007 - 6/30/2009 | University of North Carolina Zheng Fan, M.D. | Duchenne Muscular Dystrophy | \$ 177,479 | 2 years | Clinical Research Training Grant |
| 7/1/2006 - 6/30/2008 | Wake Forest University Michael Cartwright, M.D. | Duchenne Muscular Dystrophy & Charcot Marie Tooth Disease | \$180,000 | 2 years | William E. Winter Clinical Research Training Fellowship |
| 7/1/2006 - | University of Minnesota | Carnitine | \$ 131,329 | 2 years | Clinical Research Training |

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| 6/30/2008 | Peter Karachunski, M.D. | Palmitoyltransferase Deficiency | | | Grant |
| 7/1/2005 - 6/30/2007 | The University of Western Ontario Christen Shoesmith, M.D. | Amyotrophic Lateral Sclerosis | \$ 144,110 | 2 years | Clinical Research Training Grant |
| 7/1/2005 - 6/30/2007 | Brigham and Women's Hospital Ronan Walsh, M.D. | Polymyositis, Myasthenia Gravis, Inclusion Body Myositis, & Dermatomyositis | \$ 177,500 | 2 years | Clinical Research Training Grant |