

High-Dose Vitamin C to be Tested in CMT

An MDA-supported trial will test the hypothesis that high-dose vitamin C may help patients with type 1A Charcot-Marie-Tooth disease, a disorder of peripheral nerves, after studies in mice with a CMT-like disease appear promising.

2007:

300 'Antisense' Compounds Developed for Possible Use in DMD

An MDA-funded team in Australia develops some 300 "antisense" compounds that can coax muscle cells to skip over errors in the dystrophin gene and produce functional dystrophin protein molecules. Dystrophin is needed but missing in DMD. One such compound is already being tested in boys with the disease.

Researchers Release Molecular 'Brake' on Protein that Could Help Treat DMD

MDA-supported researchers identify a molecule called ERF that keeps a potentially therapeutic protein, utrophin, confined to one small area of muscle fibers. Reducing ERF levels appears to release this "brake" on utrophin production, allowing it to be produced all over the fibers and opening up a possible new therapeutic pathway for DMD.

Toxic Neighboring Cells Identified in ALS-Affected Nervous System

MDA-supported researchers find that nervous system cells called glia secrete an unknown toxic compound that kills neighboring motor neurons, the muscle-controlling nerve cells affected in amyotrophic lateral sclerosis. They say transplanting stem cells that become good glia into people with ALS might be beneficial.

Blocking Inflammation Pathway Helps in DMD

MDA-backed researchers confirm that blocking inflammation has significant benefits in Duchenne muscular dystrophy. When they treated DMD-affected mice with an engineered molecule that blocks a specific part of the inflammatory pathway, the animals had more regeneration of muscle tissue

and more effective breathing muscles than untreated mice did. The researchers believe these findings may help unravel some of the underlying mechanisms involved in DMD and improve understanding and use of anti-inflammatory drugs, such as prednisone.

Researchers Identify New Type of Muscle Stem Cell

MDA-supported researchers in Italy announce they've identified a new type of muscle stem cell that they believe is highly promising for treatment of muscular dystrophies. These new stem cells, called "pericyte-derived," are located around small blood vessels in muscle tissue. When injected into mice with Duchenne muscular dystrophy, they matured into muscle fibers and improved the animals' ability to grip a rod and stay on a treadmill.

Two Anti-Scarring Drugs Show Promise in Mice with DMD

An MDA research grantee is among the scientists who announced that two drugs, losartan and pirfenidone, have shown promise in reducing scar formation (fibrosis) in mice affected by Duchenne muscular dystrophy. Scar formation resulting from excess deposits of connective tissue is a major factor in muscle damage in DMD and other muscle diseases.

Largest Ever ALS Drug Search Begins

MDA and the ALS Therapy Development Institute in Cambridge, Mass., launch the largest drug discovery project in amyotrophic lateral sclerosis in history. The three-year, \$36 million endeavor will attempt to identify biochemical targets and find drugs that work on them.

2006:

Lab-Made Enzyme Approved by FDA

Myozyme, a laboratory-engineered enzyme patented by Genzyme and developed in part with basic research funded by MDA, is approved for use in children and adults with acid maltase deficiency (Pompe disease). It replaces the missing enzyme in this metabolic muscle disease.

Gene Therapy Trial for Duchenne Dystrophy Begins

Scientists and physicians launch the first U.S. human gene therapy trial directed at Duchenne muscular dystrophy, with the support of a \$1.6 million grant from MDA. The first of six boys with DMD receives an injection of genes for dystrophin, the missing protein in DMD, in one arm and a placebo in the other. The scientists will later measure dystrophin production and monitor the effects of the gene transfer on the children.

Variants in 'Detox' Genes Found to Raise ALS Risk

MDA-supported investigators identify variations in and around genes known as PONs, whose normal role is to detoxify poisons such as pesticides and nerve gas, as risk factors for developing amyotrophic lateral sclerosis. The finding may help explain why Gulf War veterans have a higher than normal rate of ALS (Lou Gehrig's disease) occasionally have been identified.

2005:

Cardiac Stem Cells ID'd in Lab

MDA research grantees find cardiac muscle stem cells in the hearts of rodents and humans. They say the cells, identified by the presence of the protein islet-1, are likely to help researchers understand human heart muscle disease and may even lead to treatment strategies.

Sodium Phenylbutyrate Trial Begins in ALS

MDA researchers discover that sodium phenylbutyrate appears to interfere with a cell death program and extends the lives of mice with amyotrophic lateral sclerosis (Lou Gehrig's disease). In conjunction with the Veterans Administration, they begin a trial of the drug in people with ALS.

Ceftriaxone Helps Mice with ALS

An MDA-supported research team reports that the drug ceftriaxone extends lives and prolongs strength in mice with ALS. A clinical trial of the drug, which experts believe improves recycling of the potentially toxic chemical glutamate, is approved by the Food and Drug Administration in 2006.

2004:

Three MD Centers of Excellence Result from MDA-NIH Collaboration

Three new "centers of excellence" in muscular dystrophy research are established at the University of Washington in Seattle, the University of Pittsburgh and the University of Rochester (N.Y.), as a result of a collaborative funding arrangement between MDA and the National Institutes of Health.

Key Mechanisms Found in Myotonic MD

MDA-funded groups discover that two types of proteins — transcription factors and muscleblind — are both interfered with in cells affected by myotonic muscular dystrophy. The findings lead to additional investigations at the newly established muscular dystrophy center of excellence at the University of Rochester (N.Y.), co-funded by MDA and the National Institutes of Health.

Gene Found for Rare Form of ALS

MDA-backed researchers find the gene for a rare, juvenile-onset form of ALS. The gene, on chromosome 9, carries instructions for a protein called senataxin. The finding has clear implications for diagnosis of juvenile-onset ALS and may increase understanding of ALS in general.



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