

Dear Brothers & Sisters,



Thank you for all you do to support MDA research. This newsletter is filled with articles about the ways in which the NALC's support is making a difference in the fight against muscle-wasting diseases. This year, thanks to all of your hard work, we've raised more than \$2 million for MDA.

In this issue, you'll learn about how MDA is investing the dollars raised in some exciting strategies that MDA-funded researchers are using to attack Duchenne and limb-girdle muscular dystrophies. You'll also read about spinal muscular atrophy research that's successfully coaxing an existing gene into compensating for the gene missing in SMA.

Also in these pages is information about MDA-sponsored research into the causes of and treatments for ALS, and a new MDA clinical trial network that greatly enhances and extends this work.

These are just a few examples of how the NALC's commitment to MDA is translating into lifesaving treatments for Jerry's Kids.

Please visit www.mda.org to learn more about the ways MDA is moving us closer to the ultimate goal — an end to muscular dystrophy and related diseases forever.

Longer Survival, New Hope in Spinal Muscular Atrophy

The outlook for children with spinal muscular atrophy ([SMA](#)) is better now than at any time in the past. This is thanks to more aggressive medical management of disease symptoms, and exciting research insights into causes and treatments for the disease.

Since the mid-1990s, [MDA](#) researchers have been refining their knowledge of SMA, a disease in which the nerve cells that activate muscle fibers are lost.

In its most severe form, called type 1, the disease takes the lives of infants and young children. In its least severe form, [type 3](#), patients live into adulthood but often have severe disabilities.

Severely affected children living longer

Late last year, Petra Kaufmann and Darryl De Vivo, co-directors of the MDA Clinic at Columbia University in New York, were part of a research team that found that children born with type 1 SMA between 1995 and 2006 lived significantly longer than those born between 1980 and 1994.

[\(Read more\)](#)

New Strategies in Muscular Dystrophies

In 1986, the identification of the flawed gene that causes [Duchenne muscular dystrophy](#) (and its less severe variant, [Becker muscular dystrophy](#)) inaugurated a new era in muscular dystrophy research.

Today, what was once unthinkable is being tested in children — using genetics-based therapies to correct the muscle damage caused by this flawed gene.

Research milestone

This March, researchers, physicians, families and funding agencies gathered at Children's National Medical Center in Washington to commemorate the identification of the dystrophin gene.

[\(Read more\)](#)

Bill Young
President
NALC

Full-Court Press Against ALS

ALS is a deadly, paralyzing disease that strikes adults in the prime of life and often is fatal within three to five years of diagnosis.

So far, [ALS \(also called Lou Gehrig's disease, or amyotrophic lateral sclerosis\)](#) has resisted all efforts to crack its secrets. But MDA-funded researchers continue to chip away at the mysteries surrounding the disease, and progress is being made.
[\(Read more\)](#)

Lithium may slow disease course

Early this year, researchers in Italy made a surprising announcement: The well-known drug lithium carbonate, used to treat bipolar disorder, appears to significantly slow the progression of ALS symptoms and extend survival.
[\(Read more\)](#)

ALS TDI scientists forge ahead

Progress at the MDA-supported [ALS Therapy Development Institute \(ALS TDI\)](#) in Cambridge, Mass., continues at a rapid clip.
[\(Read more\)](#)

MDA launches new Clinical Research Network

A new Clinical Research Network will speed and support research into two forms of muscle-wasting disease.

[\(Read more\)](#)