

## Report From the Chairman of the Board

Momentum continued to build on the MDA [research](#) front in 2008, as Association-supported scientists made advances against multiple [diseases](#) in MDA's program.

MDA supported more than 330 scientists and research teams around the world in 2008, and organized a new clinical research network that's speeding research in [Duchenne muscular dystrophy \(DMD\)](#) and [ALS \(amyotrophic lateral sclerosis\)](#). The Association continued relationships with biotechnology companies and nonprofit organizations with which it shares common goals. As you'll read in this report, these collaborations led to a number of exciting advances over the course of the year.

It's often the case that research progress in one disease — for example ALS or DMD — also spells progress for other diseases under MDA's umbrella, such as [spinal muscular atrophy](#), [Friedreich's ataxia](#), [Charcot-Marie-Tooth disease](#), [myotonic](#) and [limb-girdle](#) muscular dystrophies, and more.

For example, MDA's support of the [ALS Therapy Development Institute](#), made possible through MDA's [Augie's Quest](#) research initiative, has resulted in state-of-the-art, painstakingly controlled trials of experimental treatments in mice with a disease that closely resembles a human form of ALS.

This unique approach to ALS mouse trials — in which treated and untreated mice are carefully matched on all characteristics — has raised the bar on laboratory research standards throughout the world, allowing investigators to make bet-


ter informed decisions about which treatments show promise and deserve to be moved into human trials, and which do not.

Similarly, vital information about how the body responds to gene therapy, gained in MDA's Duchenne muscular dystrophy clinical trial of the strategy, will no doubt be important to the field of gene therapy in general and to gene therapy for muscular dystrophies in particular.

If strategies like exon skipping and stop codon read-through, which coax cells to reprocess erroneous genetic information, are successful, they could have implications for all the genetic diseases MDA covers and more.

And development of stem cell-based treatments, which MDA is avidly pursuing, has the potential to change the lives of patients with degenerative diseases of all types.

It's clear from these advances and the many others detailed in this report that, in spite of the troubled global economy, MDA remains a vibrant, flexible and results-oriented organization that is closing in on treatments for diseases once thought to be forever beyond the reach of human intervention.



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