Report From the Chairman of the Board

The year 2007 has been an exciting one in pursuing MDA's research mission.

In genetic muscle disease, particularly Duchenne muscular dystrophy, several research strategies have borne fruit.

The long-awaited gene therapy trial in boys with DMD passed its first test — safety — with flying colors.

In addition, the drug PTC124, developed by the biotech firm PTC Therapeutics with significant MDA support, showed it could restore production of the necessary muscle protein dystrophin in children with DMD. The mechanism by which it does this — ignoring incorrect genetic instructions — could not even have been imagined when I began working with the Association some 25 years ago.

Just as amazing is the development (with MDA support) of exon skipping, in which cells are coaxed to "skip over" defective parts of a gene and splice together the useful sections. This technique is showing very positive results in boys with DMD.

MDA scientists also have made great strides in understanding the mechanisms of myotonic dystrophy, and the biotech company Insmed is reporting promising results from a trial of a therapeutic substance called IGF1.

Among the greatest triumphs in neuromuscular disease in recent years is the FDA's approval of Myozyme, an enzyme developed in part with help from MDA basic research, and tested with MDA support. Myozyme literally saves the lives of babies born with acid maltase deficiency, or Pompe disease. In 2007, Myozyme showed another wonderful benefit: It increases the strength and endurance of children and adults who develop Pompe disease later in life.

In ALS, the collaboration between MDA and the ALS Therapy Development Institute is proceeding apace, with the facility conducting ALS experiments with unprecedented speed and accuracy, on a scale never before seen in the field.

MDA researchers also are busy looking for the best possible stem cells to try as experimental treatments for muscle and nerve diseases. In 2007, attention focused on the mesoangioblast, derived from nonembryonic stem cell sources, which had been identified by an MDA-supported group in 2006 as an exciting possibility in this cutting-edge field.

Truly, we live in remarkable times, in which new technologies and strategies are turning the once-unthinkable into wide-eyed reality. MDA is maximizing these opportunities by collaborating with other coalitions dedicated to similar ends, such as the Duchenne Research Collaborative International and the Spinal Muscular Atrophy International Coordinating Committee.

These developments, and more, open a clear path from the laboratory to children and adults awaiting safe and effective treatments for neuromuscular diseases.

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