

## Report from the Chairman of the Board

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Whether you call it “bench to bedside,” “laboratory to clinic,” or “microscope to marketplace,” a major emphasis of MDA’s research program in 2010 was clear: to move promising treatments through the development pipeline – where possible, all the way to people with neuromuscular diseases.

In amyotrophic lateral sclerosis, that’s meant continuing MDA’s robust partnership with the ALS Therapy Development Institute (ALS TDI) of Cambridge, Mass., where an experimental compound that modifies the immune system’s behavior is a front-runner; continued support of the five-center MDA ALS Clinical Research Network; and funding for Isis Pharmaceuticals in Carlsbad, Calif., for development of an innovative drug to block toxic protein synthesis.

In Duchenne and Becker muscular dystrophies, the MDA DMD Clinical Research Network began studies of heart dysfunction and tests of two heart drugs, as well as studies to fill in the gaps about the natural history of DMD in children younger than 3 and young men who are no longer walking. In addition, researchers gained crucial insights into the immunologic barriers to gene therapy in DMD/BMD.

A drug called ataluren, developed by PTC Therapeutics of South Plainfield, N.J., with MDA support, showed promising results in boys with DMD or BMD caused by a certain type of genetic mutation.

Also in 2010, MDA awarded \$1.4 million to the biopharmaceutical company Repligen of Waltham, Mass., to develop RG3039, an experimental drug designed to raise levels of a protein that’s deficient in spinal muscular atrophy.

MDA funded a number of promising research projects at later stages along the development pipeline while, at the same time, its basic science program continued to feed new ideas into its beginning, ensuring a steady stream of therapeutically focused projects.

Many of MDA’s “beginning of the pipeline” grants in 2010 were to seasoned academic researchers holding prestigious posts at major universities. Others were to new entrants into the field, young people with recently minted doctorates in medicine and/or science who are now committed to MDA’s goals.

Most of the awards made to projects further along the drug development pipeline have been to biotechnology companies, uniquely positioned to bring innovative drugs to market.

The downturn in the U.S. economy did not change much in 2010, but neither did MDA’s unwavering commitment to find treatments for the more than 40 diseases in the Association’s program.

A handwritten signature in black ink that reads "R. Rodney Howell". The signature is written in a cursive, flowing style.

R. Rodney Howell, M.D.