

Convention update and MDA news



Asst. to the President for the Contract Administration Unit Geneva Kubal



Thanks to all the delegates who stopped by the MDA booth. We were able to answer some of your questions, update our branch MDA coordinators, and raise awareness and funds for NALC's only national charity. Several branches and the NALC Auxiliary donated proceeds from the sales of items, and Los Angeles Branch 24 had an evening event to benefit MDA. I would like to thank them for their efforts.

Congratulations go to the winners of the travel vouchers. Marion Kearney of Branch 176, Michelle Luke of Branch 860, Gilles Canuel of Branch 51, Verna Hernandez of Branch 737

and D.J. Williams of Branch 2550 each secured one of five travel vouchers raffled during this national convention.

Thanks to all of you, who did your part to make this fundraiser a huge success. Proceeds will be sent to MDA to help those with MD "Live Unlimited" while MDA continues to provide care, find a cure and champion the cause to live with strength, independence and life.

What has MDA achieved with help from NALC members?

With our partnership, MDA supports nearly 350 physicians and scientists each year with a research commitment to about 40 different neuromuscular diseases. Through our fundraising efforts, the NALC has assisted MDA with contributions to almost every development in muscle biology in the last 50 years.

MDA-sponsored research has resulted in breakthroughs in treating diseases. For example, funded research helped Genzyme develop a therapy for Pompe disease called Myozyme, which has saved the lives of many patients with this devastating disease. Even where no cure is available yet, research has resulted in better treatments that result in increased survival and better quality of life. In the past, boys with Duchenne muscular dystrophy (DMD) died in their teens, but there are now some 40-year-old men living with the disease.

MDA has been involved in research into basic muscle and nerve biology since MDA's inception, when virtually nothing was known about how muscles were formed or functioned, even in non-diseased tissues. Since then, thousands of scientific papers have been published explaining how the tissues work and what goes wrong in neuromuscular disease. MDA funding supported the research that led to the discovery of the genetic causes of dozens of diseases, starting with the discovery of the dystrophin gene in 1986. Without this knowledge, there

was no hope for curing diseases. While continuing to make new discoveries in this area, this research is also paying dividends as new therapies move into clinical testing and to market.

Because MDA's research program has been so successful over the years, MDA started the translational research program in 2004 to help accelerate therapy development based on these results. This translational research is exclusively focused on the funding and commercialization of treatments and cures for neuromuscular diseases, and it hopes to increase the speed of moving drugs through clinical development.

Research into treatments for neuromuscular diseases has progressed enormously in recent years. For many of our diseases, potential treatments are now under investigation either in laboratories or in the clinic. MDA-sponsored research has either directly or indirectly resulted in most of the potential treatments that are now in development.

Even studies that are not currently being funded by MDA are based on basic research into muscle and nerve biology, the causes of the diseases and basic technologies that have been funded by MDA for decades.

Cost of illness for neuromuscular diseases in the United States

A cost study has been completed for three common neuromuscular disorders. This is the first comprehensive study of this kind to be published in the United States, establishing the direct medical and nonmedical costs, as well as the loss of income, associated with these diseases. This data may affect drug development as well as social and economic policies.

A cost-of-illness study establishes the costs associated with having a specific condition, both on an individual annual basis and to society as a whole. MDA's study was a comprehensive study, including both medical and nonmedical costs, and loss of income to families due to caregiving, loss of opportunities for education, etc.

MDA's study was designed to study amyotrophic lateral sclerosis (ALS), Duchenne muscular dystrophy (DMD), myotonic muscular dystrophy (MMD, or DM) and early- and late-onset spinal muscular atrophy (SMA). These diseases were selected for a number of reasons:

1. Therapy development is advanced in some of these fields.
2. Population sizes are large enough and uniform enough for a statistically significant number to be calculated.
3. The conditions could be separated well enough by medical codes to get useful values for medical costs.

I will expand on this topic more in a future issue of *The Postal Record*.