



‘Fill the Satchel’ coming in August

In late February, President Rolando and I visited the national headquarters of the Muscular Dystrophy Association in Tucson to meet with the leaders of our national charity. We were impressed, as always, with the caliber of these men and women who devote so much of their lives to helping those afflicted with the diseases covered by MDA research.

While there, we met with researchers and staff to discuss what was happening with MDA and to learn about potential breakthroughs in the area of medical research. We were heartened to see that real advances are being made, and every day, new and exciting breakthroughs are coming. We live in times where technology is increasing before our very eyes—but it takes hard work and money to keep up the good work.

That’s where we come in. President Rolando is very much a part of our planning and we have decided to roll out an additional way for branches to help fight MD. We will hold the first ever “Fill the Satchel” event on Aug. 14. As many of you know, firefighters have been using a similar technique successfully for many years. While our brothers and sisters collect all year long, we will kick off our first effort with a one-day event on a Sunday afternoon.

NALC, working with MDA, will provide all the necessary materials, including posters, in the coming weeks and months. What can you do now to prepare?

Contact your local MDA coordinator and have your branch representative work closely with them. While this is solely an NALC initiative, postal uniforms may be worn for this one-day event. Check with local authorities about collecting at certain locations, including malls or busy intersections. Place the pickup points in safe locations and begin to recruit members to staff the collection points. Also, deal with the accounting needs to make sure all money collected gets to MDA with credit to your branch. More information will be forthcoming.

Here are some examples of information President Rolando and I were briefed on while at MDA headquarters:

Research shows help is on the way—Even in tough economic times, MDA’s worldwide research program continues to search for ways to treat or cure muscle-damaging genetic diseases such as Becker (BMD) and Duchenne (DMD) muscular dystrophies.

These two diseases, caused by a deficiency or complete

lack of the muscle protein dystrophin, result in life-threatening weakness in skeletal muscles (including those used for respiration) and the heart. Researchers are making great headway in these two diseases, as these findings show.

“Exon-skipping” drug shows safety and benefit in DMD—Biotechnology company AVI BioPharma, headquartered in Bothell, WA, reported in October that its exon-skipping compound, AVI4658, proved safe and well-tolerated in a 26-week study and that it increased production of the needed dystrophin protein in participants with DMD.

AVI4658, which is based in part on MDA-supported basic science, is designed to coax cells to “skip over” the flawed part of the dystrophin gene. In the study, the investigators noted that participants’ cardiac, pulmonary and skeletal muscle function remained stable. In addition, they observed a reduction in biological markers indicative of harmful inflammation, and did not see an immune response to the newly produced protein.

AVI will proceed with plans to move to a Phase 2 trial for AVI4658, using higher doses of the investigational drug. Exon-skipping remains one of the most promising treatment strategies for DMD.

Low-dose Ataluren shows benefit in DMD and BMD—Analysis of a 78-person, 48-week trial of Ataluren in participants with DMD and BMD indicated that a low-dose regimen of the drug slows down the rate of decline in the ability to walk.

Ataluren (formerly called PTC124) is designed to coax muscle fibers to read through (ignore) errant “stop signs” called “nonsense” mutations in the gene-carrying instructions for the muscle protein dystrophin.

At the end of the trial, all participants had lost some walking ability, as measured by the distance they could cover in six minutes. However, after about a year, those in the low-dose Ataluren group were able to walk an average of 29.7 meters (about 97 feet) farther than those in both the placebo and high-dose groups.

We can be sure our efforts to raise funds are being used in the fight against muscular dystrophies. The future looks to be bright. After all these years, it appears that we will see treatments for MD in our lifetimes. While the fight isn’t over by any means, we plan on keeping our commitment to be there until a cure is found! ☒