



## IMMEDIATE RELEASE

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# MUSCULAR DYSTROPHY ASSOCIATION TARGETS DUCHENNE MUSCULAR DYSTROPHY WITH \$5.3 MILLION IN NEW DMD RESEARCH GRANTS

TUCSON, Ariz., August 23, 2011 — The Muscular Dystrophy Association today announced it has awarded over \$1.1 million in new research grants to three scientists working to expedite potential treatments for [Duchenne muscular dystrophy \(DMD\)](#), a relentless neuromuscular disease that begins in childhood and affects predominantly young boys.

The grants, to researchers in North Carolina, Pennsylvania and Australia, are among 13 new MDA grants focused on DMD totaling \$5.3 million, and another 25 new research awards for other neuromuscular diseases recently approved by MDA's Board of Directors. Continuing its rich tradition of being the largest nongovernmental source of neuromuscular disease research, MDA's total new grant investment exceeds \$13.7 million.

"These three grants represent the breadth of therapeutic strategies being explored to improve the lives of individuals living with DMD," said Sanjay Bidichandani, M.B.B.S., Ph.D., MDA's vice president for research.

[Martin Childers, Ph.D.](#), professor at the Institute for Regenerative Medicine in Winston-Salem, N.C., will apply his \$480,000 three-year grant to study DMD-related heart disease from a unique perspective.

"We will use a groundbreaking method called 'cellular reprogramming.' This method was first used to make stem cells out of skin cells taken from patients," Childers said. After reprogramming the DMD patient's skin cells to create heart cells, Childers' team will use those cells to test thousands of experimental compounds and drugs already approved by the U. S. Food and Drug Administration (FDA).

“If we find compounds or even older drugs that can improve the function of individual heart cells from DMD patients, and these new drugs get further developed and tested, then it is very possible that discoveries in our assays could lead to prevention or reversal of heart disease in DMD patients in the future,” Childers noted.

[Tejvir Khurana, M.D., Ph.D.](#) at the University of Pennsylvania will apply his new three-year MDA grant of \$379,500 to finding a successful method of delivering utrophin protein to dystrophic muscles. Utrophin is similar in structure and function to the dystrophin protein, that when absent causes DMD. Because of its similarities to dystrophin and its presence in DMD and healthy muscle cells, increasing levels of the utrophin protein may ultimately improve muscle strength and function.

“We have found that utrophin is in a state of repression and that a class of molecules called microRNA’s cause the repression,” Khurana said. “We will develop methods to repress the repressors and hence achieve utrophin upregulation,” he added.

[Margaret Zacharin, F.R.A.C.P.](#), pediatric oncologist at Murdoch Childrens Research Institute at Royal Children’s Hospital in Parkville, Victoria, Australia, is working to prevent extensive bone loss caused by corticosteroids, the only available treatment shown to slow the progression of the disease, but with significant adverse side effects including weight gain and bone thinning. She has been given a \$268,021 two-year grant by MDA to study the use of bisphosphonates in DMD patients.

“Bisphosphonates have been shown to largely prevent corticosteroid-induced osteoporosis in adults,” Zacharin said. “If bisphosphonate use can improve bone quality and reduce fracture risk in children with DMD, this effect will allow the boys to remain in better health for longer,” she added.

### **About Duchenne muscular dystrophy**

In DMD, boys begin to show signs of muscle weakness as early as age 3. The disease gradually weakens the skeletal, or voluntary, muscles in the arms, legs and trunk. By the early teens or even earlier, the boy’s heart and respiratory muscles also may be affected.

### **About MDA**

MDA is the nonprofit health agency dedicated to curing muscular dystrophy, ALS and related diseases by funding worldwide research. The Association also provides comprehensive health care and support services, advocacy and education. See MDA’s award-winning [“Make a Muscle, Make a Difference”® PSA](#).

In addition to funding more than 300 research projects worldwide, MDA maintains a national network of some 200 hospital-affiliated clinics; facilitates hundreds of support groups for families affected by neuromuscular diseases; and provides extraordinary local summer camp opportunities for

thousands of youngsters fighting progressive muscle diseases. The Association is the first nonprofit to receive a Lifetime Achievement Award from the American Medical Association “for significant and lasting contributions to the health and welfare of humanity.”

For more information about the Association and its programs, go to [mda.org](http://mda.org).

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