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MUSCULAR DYSTROPHY ASSOCIATION INVESTS MORE THAN \$2.5 MILLION ON NINE NEW ALS PROJECTS

TUCSON, Ariz., Aug. 23, 2011 — The Muscular Dystrophy Association (MDA) has awarded research grants totaling more than \$2.5 million over three years to nine researchers studying <u>ALS (amyotrophic lateral sclerosis, or Lou Gehrig's disease)</u>. Together, these new ALS projects represent nearly one-fourth of the <u>40 new research awards</u> recently approved by the MDA Board of Directors.

Over the years the Association has invested more than \$290 million in the fight against ALS, an often rapidly progressive disease that attacks nerve cells and ultimately paralyzes voluntary muscles, including those used for walking, breathing and swallowing. Average life expectancy for people with the disease is three to five years after diagnosis.

Continuing its long tradition of being the largest nongovernmental source of neuromuscular disease research, MDA's latest round of grant investment exceeds \$13.7 million. Many of the peer-reviewed initiatives will run through July 2014.

"Among the nine new ALS research grants are three notable projects to investigators being funded by MDA for the first time," said Valerie Cwik, M.D., MDA's executive vice president for research and medical director. "We're delighted that new MDA-funded research teams in Houston, Montreal and San Diego have joined our fight against ALS."

Raymond Grill, a researcher at University of Texas Health Science Center in Houston, has been awarded \$202,508 for research to determine if the drug Licofelone could enhance the action of riluzole, the first drug approved as an ALS treatment by the U.S. Food and Drug Administration (FDA).

Grill, whose background is in spinal cord injury, is looking to see if a combination drug treatment works better than one drug alone in ALS. Working with mouse models, Grill's team will test Licofelone to see if it helps riluzole better penetrate the nervous system. Grill calls his study a "novel and combinatorial therapy" that also could help with other drugs.

Jasna Kriz, an associate professor at Laval University in Quebec City, Canada, was awarded \$445,086 for research into the "pre-onset ALS." Kriz's lab recently developed and validated a series of mouse models with bioluminescent and fluorescent signals allowing time-lapse imaging of microglial activation, astrogliosis and neuronal damage. Kriz is looking for the earliest visible signs of ALS in part because "not much is known about pre-onset ALS."

"My goal is to visualize early changes before the onset of ALS," said Kriz. "With this imaging mouse model — using the firefly gene — I can see the activation of the genes that involve inflammation and neuronal stress." Kriz added that an advantage of the study is she "can follow the same animal through all stages of the disease."

<u>Clotilde Lagier-Tourenne</u>, a postdoctoral fellow from the University of California, San Diego at the Ludwig Institute for Cancer Research, was awarded \$180,000 for her research in the study of TDP43 and FUS/TLS. Recent identification of ALS-causing mutations in genes encoding for TDP43 and FUS/TLS has sparked a paradigm shift relating to the understanding of ALS pathogenesis.

"What we have decided to do is identify which RNAs these ALS mutations bind to in order to really understand normal function and how RNA is going to be disturbed," she said. "We are using next generation gene-sequencing technology and have identified many targets where TDP43 binds RNA. We will do the same for FUS/TLS to see if the targets are crucial in the ALS disease process."

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 some 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 on the Association's research and other programs, visit mda.org.