NEWS from MDA

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MDA AWARDS UNIVERSITY OF MINNESOTA RESEARCHER $375,000 TO STUDY FACIOSCAPULOHUMERAL MUSCULAR DYSTROPHY

TUCSON, Ariz., Aug. 23, 2011 — The Muscular Dystrophy Association has awarded a $375,000 research grant to Michael Kyba, Ph.D., associate professor in the department of pediatrics at the University of Minnesota.

Kyba's innovative project is one of 40 new multiyear research awards recently approved by the MDA Board of Directors. 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.

Kyba's new study on the DUX4 gene and its impact on facioscapulohumeral muscular dystrophy (FSHD) is now under way and focused on identifying inhibitors of DUX4 as lead candidate drugs. The initiative — one of three new FSHD projects (see Wagner and Rahimov) contributing to MDA's total current FSHD research funding of $3.2 million — also will test promising compounds on DUX4-transgenic mice that model the disease.

According to MDA Executive Vice President for Research and Medical Director Valerie Cwik, M.D., "Years of elegant FSHD work created a natural foundation for this exciting new project at the University of Minnesota. We're delighted that Dr. Kyba is now part of the family of MDA-funded investigators working to speed treatments for this puzzling muscle disease."

"We've been chasing this hypothesis that DUX4 is responsible for FSHD," Kyba explained. "There is accumulating evidence from genetic studies to show that DUX4 is an important factor in FSHD — perhaps the cause itself."

"We think the DUX4 gene interferes, not with the muscle cells themselves, but the muscle stem cells so the repair of normal damage and wear and tear is ineffective in these individuals."

This is Kyba's first MDA grant. He has been studying FHSD for more than four years.

"We are excited that the MDA has helped us continue this work," Kyba said.

About FSHD

FSHD is one of nine types of muscular dystrophy, a group of genetic, degenerative diseases primarily affecting voluntary muscles. It is usually inherited in an autosomal dominant pattern, meaning it is passed from generation to generation. The primary symptoms of FSHD include weakness of the muscles around the eyes, mouth, shoulders and upper arms. Disease severity ranges widely from individual to individual. The disease typically progresses slowly although there may periods of rapid deterioration.

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

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