



## Parent Project Muscular Dystrophy Awards Two End Duchenne Grants

**Researchers at University of Florida -- Gainesville, University of California -- Los Angeles to Receive \$250,000 For Duchenne Muscular Dystrophy Research**

**MIDDLETOWN, Ohio, May 5 /PRNewswire-USNewswire/ --** Patricia A. Furlong, Founding President and CEO of Parent Project Muscular Dystrophy (PPMD), the largest non-profit organization in the United States focused on finding a cure for Duchenne muscular dystrophy (Duchenne), announced today two recipients of the newly launched End Duchenne Grant Award Program. **Krista Vandenborne, Ph.D.**, Chair of the Physical Therapy Department at the University of Florida will receive \$200,000 to continue her work with MRI and MRS technology as a tool to monitor the disease progression of Duchenne. Also receiving a grant is Melissa Spencer, Ph.D., Associate Professor of Neurology at UCLA, who will receive \$50,000 to continue her work with Osteopontin as a therapeutic target for Duchenne.

The End Duchenne Grant Award Program was created by PPMD in partnership with the National Institutes of Health (NIH) in an effort to ensure continuation of promising Duchenne research and translation to human studies. The End Duchenne Grant Award Program is a bridge grant provided by PPMD to selected research projects that receive scores beyond the current funding paylines of the NIH Institutes and Centers supporting Duchenne research.

Explains Ms. Furlong, "With its research budget growing tighter each year, the NIH can fund only a fraction of the many promising applications it receives. Currently, only those grants which score in the top percentiles are able to be funded."

The End Duchenne Grant Award Program represents the first Duchenne specific bridging program and the first ongoing bridge funding program to be presented in the rare disease category. As a collaborative effort with the NIH, the selection strategy behind this award is very different than any other private research investment in Duchenne, yet the goal of this award remains consistent with all of PPMD's research initiatives: to advance promising research which will impact this generation affected by Duchenne.

The recipient of an End Duchenne Grant must be focused on translational research (the process of applying ideas, insights and discoveries generated through basic scientific inquiry to the treatment or prevention of disease or injury). The NIH will notify investigators whose translation grant applications score well, but not within the NIH funding levels, to submit their applications and score sheets to PPMD. Ms. Furlong continues, "PPMD's Scientific Review Board will re-review these applications seeking to identify those with the greatest potential to ultimately help all boys with Duchenne."

Earlier this year, PPMD announced that Brown University would receive the first End Duchenne Grant Award. Since awarding that grant to Brown and Dr. Justin Fallon, Ms. Furlong said that interest in the program is quickly growing and the quality of the applications is exceptional. "For a grant program in its infancy to receive such impressive and promising applications is thrilling. It is our great privilege to support Dr. Vandeborne and Dr. Spencer in their endeavors. The University of Florida and UCLA represent the commitment of leading educational institutions to developing treatments that will help not only the Duchenne community, but the entire medical community on an international level. Their work is the kind of forward-thinking, cutting-edge research that PPMD focuses on and is the reason we created the End Duchenne Grant Award Program. If we can help both Dr. Vandeborne and Dr. Spencer maintain the momentum of these important avenues in treatment, so that they can receive funding from the NIH in the near future, then we will consider this grant program a huge success."

Dr. Vandeborne is delighted that the University of Florida -- Gainesville will receive the support of PPMD and said, "It is an honor to receive this grant and to know that the PPMD review committee feels as strongly about the promise of MRI and MRS as a non-invasive outcome measure for Duchenne. If we can replace biopsies as a tool to monitor efficacy in clinical trials for Duchenne with these methods, I think we will be able to greatly accelerate the translation from pre-clinical animals studies to clinical trials."

UCLA's Melissa Spencer is also excited about the potential this grant gives her work. "Ms. Furlong and the grant committee understand the potential of Osteopontin to help reduce inflammation and fibrosis in Duchenne, and they are willing to show their support financially. I can think of no greater compliment than to have an organization, as conscientious and dedicated to ending Duchenne as PPMD, support our research at UCLA."

The End Duchenne Grant Award Program was designed to enable investigators to continue their projects and generate additional data for a successful re-application within a 12 to 24 month period, thus leveraging additional Duchenne-specific research dollars.

Duchenne, the most common form of childhood muscular dystrophy, is a progressive and fatal muscle disorder affecting boys and young men that causes the loss of muscle function, wheelchair dependency and a decline in respiratory and cardiac function.

An applicant seeking support from the End Duchenne Grant Award Program must be employed at a for-profit or non-profit organization or institution and have submitted and received a complete review of an application to the NIH directed toward translation of research into human clinical studies specific for Duchenne muscular dystrophy. The applicant

must have the resources to conduct the proposed research project and the organization/institution must have appropriate grant administrative capacities for the handling and disbursing of research funds. For more information or to apply, please visit [www.parentprojectmd.org/EndDuchenneGrant](http://www.parentprojectmd.org/EndDuchenneGrant).

#### **About PPMD**

Parent Project Muscular Dystrophy (PPMD) is a national not-for-profit organization founded in 1994 by parents of children with Duchenne and Becker muscular dystrophy. The organization's mission is to improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy through research, advocacy, education and compassion. PPMD is headquartered in Middletown, Ohio with offices in Fort Lee, New Jersey. For more information, visit [www.parentprojectmd.org](http://www.parentprojectmd.org).