NIH funds new Wellstone Research Center for muscular dystrophy at Boston Biomedical Research Institute

By Patti Jacobs

Boston (Watertown), MA – Today the US National Institutes of Health (NIH) awarded nine million dollars to launch a unique collaboration of researchers, clinicians, patients, government research agencies and pharmaceutical/biomedical companies to study the causes and potential treatments for facioscapulohumeral muscular dystrophy (FSHD), a muscle weakening and disabling disease that affects, at the least, one in 20,000 individuals worldwide. The award will create the first Senator Wellstone Muscular Dystrophy Cooperative Research Center (MD CRC) to focus on FSHD.

Headquartered at the Boston Biomedical Research Institute (BBRI), this center for excellence in muscular dystrophy will be the first ever in New England and the first of these prestigious centers – named for the late Senator Paul Wellstone – to focus on FSHD, the second most prevalent adult muscular dystrophy. The Wellstone MD CRCs themselves represent a paradigm shift in research because of their intensely collaborative nature and their mandate to include the patient advocacy organizations as full partners in the research process.

“We see this as a unique opportunity to tackle a tough disease in the most efficient way to set the stage for development of therapies,” says Dr. Charles Emerson, director of the new center and president of BBRI. “We have recruited the best minds in basic and clinical research and have an extraordinarily committed patient advocacy group to help us focus on this problem.”

Co-director Dr. Louis Kunkel, at Harvard Medical School and Children’s Hospital, agrees. “We believe this research center model will bring discoveries from bench to bedside more rapidly than the traditional model because we already have the full participation of the people with the most at stake in our work – the patients.”

In fact, both Emerson and Kunkel admit that without the advocacy of Daniel Paul Perez – FSHD patient and the founder and CEO of the FSH Society

Perez has been fighting tirelessly behind the scenes and in front of Congress, the NIH and the scientific community for 20 years for funds, increased research and attention for this disease. “This is a victory for all of us,” says Perez. “It represents decades of work by dedicated researchers, Society leaders and patients to find a research home for this disease.” Perez was diagnosed as a toddler and has been the national leader for patients with FSHD since graduating from college.

Another major collaborator will be Acceleron Pharma, a biotech company that will partner with the Wellstone Center scientists and clinicians to determine the safety and effectiveness of a new class of drugs that enhance muscle mass and strength. The hope is that these drugs will help maintain muscle strength and physical function in patients with FSHD and other dystrophies. Additionally, Genzyme Corporation will participate in the development of cell-based therapeutic approaches, which exhibit noteworthy promise.

Perez and the Senator worked together on the original MD Care Act 2001 legislation before the Senator’s untimely
Wellstone Research Center, continued

In addition to Emerson, Kunkel and Perez, the center’s network of collaborators includes Kathryn Wagner, M.D., Ph.D., The Johns Hopkins Hospital; Mayana Zatz, M.Sc., Ph.D., University of Sao Paolo, Brazil; Robert J. Bloch, Ph.D., University of Maryland School of Medicine; Woodring Wright, M.D., Ph.D., University of Texas Southwestern Medical Center; and Jeffery B. Miller, Ph.D., Boston Biomedical Research Institute.

A Letter from the Chairman, Board of Directors, FSH Society

Dear Friends,

My family and I are delighted that at long last a research home will be established by the new FSHD-focused Wellstone Center after decades of work by dedicated researchers, Society leaders, and patients.

Projects in the new center include clinical trials of new compounds along with the development of cell and mouse models and, hopefully, the discovery of new biomarkers that may be important in FSHD disease development, as well as establishing a cell and tissue repository for FSHD researchers worldwide, and a training component that prepares young scientists for careers in skeletal muscle biology and FSHD.

The diverse group of basic scientists and clinical investigators that has been assembled is superb. Center director, Dr. Charles Emerson is internationally recognized as a developmental biologist; he has contributed significantly to studies of skeletal muscle tissue. Dr. Louis Kunkel, co-director, is an internationally distinguished geneticist with decades of experience in muscular dystrophy research. He is also a valuable and dedicated member of the Society’s Board of Directors and Scientific Advisory Board. We are also grateful for the participation of Drs. Kathryn Wagner (also a member of the Society’s Scientific Advisory Board), Mayana Zatz, Robert Bloch, Jeffery Miller, and Woodring Wright.

I know that you will also wish to join me in thanking Daniel Perez for his unwavering leadership of the FSH Society over these many years. His internationally recognized leadership in FSH muscular dystrophy has inspired and catalyzed the award for this center. The Society’s role in the new center is as liaison with the community of patients and researchers. We will receive funds from this award to support patient meetings, research meetings and website development. We will continue fundraising which is vital for component projects that need additional funds such as building a mouse core similar to the cell and tissue repository, and expanding all research projects that are in this program. Furthermore, your dollars enable us to continue our longstanding support of multifaceted FSHD research not included in the Wellstone program.

The new center brings us closer than ever to the development of a treatment for FSHD. You and your fellow FSH Society members have contributed to this progress to date. I hope you will continue to support these advances and give another gift at this time.

Thank you very much.

Sincerely,

William R. Lewis, Sr., M.D.