



FSH Watch

A publication of the Facioscapulohumeral Muscular Dystrophy Society
Connecting the community of patients, families, clinicians and investigators

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

First Wellstone center in New England and first center in the world to focus exclusively on facioscapulohumeral muscular dystrophy

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 – the new Wellstone Center might not have become a reality. According to the two scientists, 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.

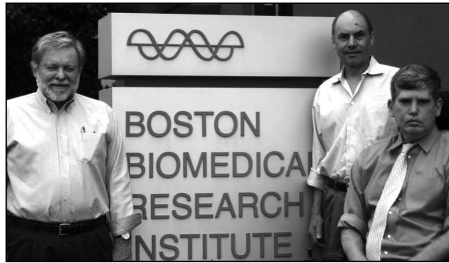
In a congratulatory letter of support for the Wellstone Center, Senator Edward Kennedy wrote, “Beyond research, this new center will provide future medical health pioneers insight and knowledge into muscular disease treatment as well as promote national cooperation among top leading research centers.”

The Wellstone center partners will identify biomarkers to monitor the effectiveness of therapeutics during clinical trials. The center will establish a repository of FSHD-diseased and normal muscle stem cells, tissues and animal models to provide the international community with the resources critically needed to

develop and test new drug and cell-based therapeutics. A key component of the Wellstone Center will be to educate and train the next generation of research and clinical

scientists in an outstanding environment for muscle disease research.

Like the five other Wellstone centers established since 2002, the Boston center is the legacy of Senator Paul Wellstone, who was the Congressional champion of muscular dystrophy. Perez and the Senator worked together on the original MD Care Act 2001 legislation before the Senator’s untimely



Charles Emerson, Ph.D., Boston Biomedical Research Institute; Louis Kunkel, Ph.D., Children’s Hospital Boston and Harvard Medical School, and Daniel Paul Perez, FSH Society

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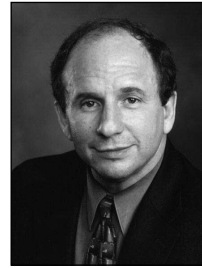
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Wellstone Research Center, continued

death in a plane crash in 2002.

Facioscapulohumeral muscular dystrophy is the second most common adult-onset muscular dystrophy and currently has no treatment or cure. Perez notes that the prevalence of FSHD "is probably three times higher than previously thought given our increased experience with the disease and improved genetic testing for FSHD."



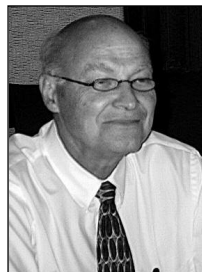
Paul David Wellstone
 1944 – 2002
 United States Senator,
 Minnesota 1991-2002

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 Paulo, 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.



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

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 Scien-

tific 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.