Finding Treatments for Muscular Dystrophy

Daniel Miller

Associate Profesor, Pediatrics Clinical Geneticist, Genetics Adjunct Associate Professor, Genome Sciences

CURRENT RESEARCH

Using high throughput screening and state-of-the-art biotechnology to identify drug targets for muscular dystrophy

A slowly progressive condition, FSHD is a debilitating late childhood-onset disease with initial symptoms occurring primarily in the muscles of the face, upper arm, and scapula; frequently the symptoms progress to the point of confining people to wheelchairs or even forcing them to become bedridden. Dr. Daniel Miller, Associate Professor of the University of Washington's Department of Pediatrics is researching the causes and effects of this disease in the hopes of restoring a normal quality of life to those suffering from it. Dr. Miller is taking a two pronged approach, studying first why the rogue DUX4 gene that causes FSHD is being expressed, and secondly what intervention methods may be possible to prevent DUX4 expression and help ailing patients. This disease is often diagnosed in a patient's early-to-mid teenage years during a developmental stage when most teenagers feel invincible. The news of a diagnosis can be devastating. With his research Dr. Miller is offering to return to patients the control of their own bodies. He is working to restore a healthy lifestyle to individuals who should never hear that they may be confined to a wheelchair for the remainder of their lives. Understanding the basics of how and why this disease attacks muscles the way that it does will lead to effective treatments that will stop disease progression, and potentially reverse its effects.

Facioscapulohumeral muscular dystrophy (FSHD) is one of, if not the most prevalent variation of muscular dystrophy diseases, though its slowly progressive nature has allowed it to thrive under the medical news radar. It is a unique variation because it is caused by the inappropriate expression of the DUX4 gene resulting from incorrect...

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AFFILIATION

University of Washington

EDUCATION

- M.D., in Medicine/Pathology, 1992 , University of Washington
- Ph.D., in Medicine/Pathology, 1996 , University of Washington
- Residency, in Pediatrics, 1999 , Seattle Children's Hospital
- Fellowship, in Medical Genetics, 2002 , University of Washington

RESEARCH AREAS

Life Science, Diagnostics, Genomics / Congenital, Pediatric

FUNDING REQUEST

As FSHD occurs in late childhood, and progresses slowly, we have the opportunity to identify people with the disease and stop its progression before it has done significant muscle damage. Your contributions will bolster these efforts and allow therapies to treat muscular dystrophy to be discovered. There is an urgent need for funding of basic science and laboratory equipment to gain a better fundamental understanding of this disease, and to test potential therapies.