



FSH Watch

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

At Last: Bringing Gene Therapy for FSHD into the Laboratory

By Scott Q. Harper, Ph.D., Assistant Professor, Center for Gene Therapy
 The Research Institute at Nationwide Children's Hospital and Department
 of Pediatrics, The Ohio State University, Columbus, Ohio

In June 2007, I was appointed Assistant Professor at The Ohio State University and Principal Investigator of my own lab in the Center for Gene Therapy at the Research Institute at Nationwide Children's Hospital in Columbus, Ohio. I am combining my two areas of training, RNAi and muscle gene therapy, to develop RNAi-based treatments for facioscapulohumeral muscular dystrophy (FSHD) and other dominant muscular dystrophies. To initially show proof-of-concept, we generated adeno-associated virus (AAV) vectors carrying microRNAs that reduce over-expressed FRG1, a candidate gene for FSHD. We are testing the efficacy of these vectors in mice that produce toxic FRG1 levels in muscle and show FSHD-like symptoms. Since FSHD may be



Dr. Harper and researchers in his lab, Jorge Torres, Jennifer Allen, Lindsay Wallace, Sara Garwick, and Dr. Harper, Center for Gene Therapy, The Research Institute at Nationwide Children's Hospital and Department of Pediatrics, The Ohio State University

caused by mis-expression of other, yet-to-be defined genes, we believe that this strategy will set the stage for future RNAi-based treatment strategies as more genes are identified. Likewise, we are actively searching for other genes that may be involved in FSHD biology and may be candidates for RNAi treatment.

ivated people. My center director, Jerry Mendell, M.D., works tirelessly to make gene therapy treatment for all types of muscular dystrophy, including FSHD and duchenne muscular dystrophy (DMD), a reality. He has initiated numerous clinical trials, and provided the leadership to make the Center for Gene Therapy a world-class institution by, among other things, recruiting outstanding scientists and clinicians and building a clinical-grade AAV vector production facility for use in human trials.

For those readers considering a professional career in biomedical research, my path to becoming a scientist began in public school in Saginaw, Michigan. My non-scientist parents – mom was a homemaker who later became a kindergarten teacher and dad had numerous jobs, including driving a bread truck and selling appliances – enrolled me in an advanced

math/science program at Saginaw's Center for the Arts and Sciences (CAS). I attended the CAS in the mornings and then took a bus to my regular junior high school, to take classes in English, PE, history, etc. The math/science curriculum was split evenly between the two subjects, but only one grade was given. I always aced the science part and essentially ignored my math work. Since the grade was averaged, I ended up getting a lot of C's. Here I was first inspired to pursue a career in science. At the risk

of making a political statement, I am at least one example of how the American public school system, if properly funded and managed, can help mold lives in a positive way. Somewhat contradictory to this is that I was generally bored with school (except science classes) and cannot claim to have excelled.

I graduated at the age of sixteen, took a year off, spent a year at a community

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Dorr Foundation Awards Grant for Teenagers and Young Adults Program

Parents and young people tell us that facioscapulohumeral muscular dystrophy (FSHD) is lonely and isolating, that it affects teenagers and young adults in a way different from other age groups. Parents and their children have the usual issues of adolescence, now compounded by a disease for which there is no treatment and no cure. At a time when this age group is interested in dating, in athletics, in making lifelong friends, and in planning their academic future, the FSHD patient population also experiences or faces the beginning of irreversible muscle loss and the reality of a lifelong disease. Because the disease is genetic, other children in the family may have it, as may one of the parents, perhaps resulting in greater stress in family life.

The Dorr Foundation has made a generous award to the FSH Society to develop a program of education and support for teenagers and young adults with FSHD. Please consider how you might participate and ask young people you know to contact the Society and get involved.

Next Steps

We invite individuals – parents and young people – to form an Advisory

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Dorr Foundation Awards Grant, *continued from front page*

Group. Please let the Society know your interest in joining this group. The Advisory Group will work to help the FSH Society better serve young people. We will also use the International Patient and Researcher Meeting, July 27, Coralville/Iowa City, to launch this program. Two workshops are included that address our young audience: "Taking Care of Ourselves for Teenagers and Young Adults with FSHD: A session for patients to talk with each other and with physicians and researchers about FSHD;" and "Teenagers and Young Adults Networking and Making Plans: A session to share experiences and concerns."

Recently, Mickey Slevin, Program Assistant at the FSH Society, published entries for the Society on MySpace™, www.myspace.com/fshsociety, and on Facebook™, www.facebook.com, where one searches for FSH Society or facioscapulohumeral in the search box, to provide a social networking opportunity for young people and to give the Society a means for communicating with these members. (See related article.) This is also a way to reach young patients who have not found the Society through other means. Both of these sites are moderated for content and members.

How Will You Participate?

- Introduce the Society to the young people in your home and family; and
- Join or share your feelings with the Advisory Group.



It is the editorial policy to report on developments regarding FacioScapuloHumeral Muscular Dystrophy (FSHD), but not to endorse any of the drugs or treatments discussed. We urge you to consult with your own physician about the procedures mentioned.

What Are the Advisory Group's Objectives for the Coming Months?

- Make a plan to identify needs of teenagers and young adults and respond to these needs;
- Create a membership category for these individuals;
- Communicate, communicate, communicate: use *Watch* newsletter and make maximum use of technology and communication vehicles embraced by these individuals; and
- Evaluate progress and participation in early 2009.

The Future

Over the years the Society has been focused primarily on the needs and concerns of adults and families, and on research to find treatments and a cure. We are not wavering from these commitments. However, we recognize that one of the ways to serve more people, to grow the Society and to meet a very important need is to reach out to more teenagers and young adults. At every step we are aware that we must listen to the concerns of young people about coping with the various stages of the disease, and help them to manage their personal, social and academic lives.

A number of inspiring teenagers are already involved with the Society, connecting with each other and expressing their concerns, dreams and fears. They often mention that until the Society introduced them to a peer with FSHD, they had never met another person with this disease. They write for our newsletter, they go to the FSH Society Bulletin Board to chat (moderated), and they have extraordinary insight as they share their feelings and experiences.

We look forward to this program. On behalf of all Society members, we thank the Dorr Foundation and its trustees including Mrs. Virginia Maxwell, grandmother of a young person with FSHD, for this award. ◇

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Bringing Gene Therapy into the Laboratory, continued from front page

college, then joined the U.S. Navy. The Navy was another profound experience for me; sounds like a recruiting commercial, but there I learned the importance of commitment and hard work. After I went from active duty to the Reserves, I returned to college and got my B.S. in biology from Saginaw Valley State University (SVSU). In 1996, I began my Ph.D. studies in cellular and molecular biology at the University of Michigan in Ann Arbor. My first year there was, to say the least, humbling. Classes were taught differently than in college, there was a whole language of molecular biology that I had not learned yet, and, coming from small, obscure SVSU, I was intimidated by my fellow graduate students who came from places like Princeton and Yale.

While at Michigan, I had the great opportunity to join the lab of Jeffrey Chamberlain, Ph.D., who was working on developing gene therapy for DMD. The lab was doing exciting work, and people there were enthusiastic and enjoyed what they did. This was a reflection of Dr. Chamberlain, whose scientific brilliance is equally matched by his quality of character. Dr. Chamberlain continues to be developing a gene therapy method to treat DMD. Children afflicted with this dystrophy are either missing a piece of their X chromosome containing a gene called dystrophin or have a copy of the gene that does not function normally. Before I joined the lab, Dr. Chamberlain used a mouse model for DMD to show that adding back dystrophin in muscles lacking it essentially “cured” the muscular dystrophy in these mice. This study suggested that dystrophin replacement might be a way to treat human DMD, but delivering it to people was a much tougher task. The strategy Dr. Chamberlain began developing in the 1990’s was to deliver the dystrophin gene using viruses that naturally infect muscle. In this approach, genes required for viral replication in humans are removed, so the virus is still capable of infecting cells but cannot reproduce itself. These deleted viral genes are then replaced by inserting human dystrophin. The end result is the virus is “tricked” into delivering

the dystrophin gene to muscle in their place.

Because dystrophin is a huge gene, the only virus capable of carrying it was adenovirus. However, this virus was not long-lasting and it soon became clear that other methods of delivery were necessary. One such alternative was adeno-associated virus (AAV), which was known to efficiently infect muscle and produce long-term gene expression. Importantly, AAV does

not cause any known disease in humans, so it is, in many ways, the ideal vector for muscle gene therapy. The major drawback to AAV is its extremely small size; the dystrophin gene was about three times larger than the carrying capacity of AAV. To use this vector, we had to dramatically reduce the size of dystrophin. This is where my project began. Based on naturally occurring dystrophin deletions found in mildly-affected patients, we made rational deletions in dystrophin to reduce its size. These so-called micro-dystrophins were capable of fitting into AAV, and, when delivered to diseased mouse muscle, improved many of the symptoms associated with DMD. This work is still moving forward in Dr. Chamberlain’s lab, as well as others, and clinical trials are underway.

After receiving my Ph.D. in 2002, I then moved to the University of Iowa to do my post-doctoral research in the laboratory of Beverly Davidson, Ph.D. Again, I found an outstanding mentor and scientist to help guide this stage of my career. Here, I began working on developing a new technology, called RNA interference (RNAi), for gene therapy. RNAi can be used to reduce, rather than replace, expression of a mutated gene. This is important for dominant disorders in which only one bad copy of a gene is sufficient to cause disease, or diseases caused by

toxic over-abundance of normal genes. RNAi is a natural process in which small inhibitory RNAs (called microRNAs) help prevent expression of other, protein-coding RNAs (called messenger RNAs; mRNAs).

This is achieved, in part, through sequence similarity between the inhibitory RNA and mRNA. This similarity allows inhibitory RNAs to guide cellular gene silencing machinery to the proper mRNAs and prevent them from being made into proteins. For gene therapy, we can design artificial microRNAs that are similar to any

gene we are interested in controlling.

Dr. Davidson specializes in brain gene therapy, and shortly after joining her lab, I began working on developing an RNAi-based strategy to reduce expression of the toxic gene causing the brain disorder, Huntington’s disease (HD). To do this, I again used AAV vectors to deliver inhibitory RNAs to mouse brain, since these vectors also efficiently infect mammalian neurons. We were the first to show that RNAi could be used to suppress the mutant HD gene in mice and improve HD-related symptoms. Dr. Davidson is moving this strategy forward with the goal of eventually using it to treat human HD, and we continue to publish in this area.

I hope it is evident that the success of my career so far is owed in large part to several exceptional people. I am happy to find also that the FSH Society has been extremely supportive in my very short career as an independent scientist. Daniel Perez has been instrumental in helping to guide the focus of my lab and putting me in touch with many of the excellent established investigators in the field who have graciously shared their advice and resources, including Rossella Tupler, M.D., Ph.D., Alexandra Belayew, Ph.D., Rabi Tawil, M.D., and Melanie Ehrlich, Ph.D. I thank you for the opportunity to introduce myself and my lab to the FSHD community. ♦

Dr. Harper has support from the FSH Society Landsman Charitable Trust Fellowship.

“ I began working on developing a new technology, called RNA interference (RNAi), for gene therapy. RNAi can be used to reduce, rather than replace, expression of a mutated gene. This is important for dominant disorders in which only one bad copy of a gene is sufficient to cause disease, or diseases caused by toxic over-abundance of normal genes. ”

Perspective on MYO-029 Clinical Trial

The Clinical Viewpoint from the Principal Investigator

By Kathryn Wagner, M.D., Ph.D., Associate Professor of Neurology and Neuroscience; co-director, Johns Hopkins MDAUSA Clinic; and co-director, Johns Hopkins/University of Pennsylvania Senator Paul Wellstone Muscular Dystrophy Cooperative Research Center; The Johns Hopkins Hospital, Baltimore, Maryland

As many readers will know, a clinical trial sponsored by Wyeth Pharmaceuticals was recently completed and published (Ann Neurol. 2008 Mar 11. [Epub ahead of print] PMID: 18335515) of an inhibitor of myostatin in adult muscular dystrophy. One third of the participants had FSHD. Myostatin is a negative regulator of muscle growth and inhibition of myostatin stimulates muscle growth and regeneration. The trial showed that MYO-029 was safe at the doses evaluated and had a suggestion of biological efficacy. Although everyone involved was disappointed that a larger effect was not observed and that MYO-029 will not be further developed by Wyeth, there are many reasons to be pleased with the experience.

First, the trial was extremely successful in evaluating various outcome measures that will be invaluable in designing the next clinical trial in FSHD. Second, the trial brought together several different sites across the United States and in the United Kingdom for similar training of investigators, study coordinators and clinical evaluators. Third, the visibility of the disease and the FSH Society was increased to the larger community. Finally, as you will read below, people with FSHD were empowered by the experience of making a valuable contribution to research in therapeutics.

I have had the opportunity to personally thank the subjects who participated at Johns Hopkins and extend my thanks now to those at other sites. One of the endearing qualities of FSHD participants was their complete enthusiasm for the project. Typically, enrollment for a cohort, or grouping of subjects at a particular dose, would open and close on the same day for

FSHD. Patients at Hopkins arrived at 7:00 a.m. in the morning to make sure they would be enrolled. They made all their appointments and they volunteered for muscle biopsies (a surgical procedure). More important than endearing yourselves forever in my heart, this devotion has left all involved with a desire to work with the FSHD population again in clinical trials. Therefore, through the FSH Society and through the natural enthusiasm of those with FSHD, future clinical trials of myostatin inhibitors or other muscle enhancing drugs are currently being highly considered in FSHD.

... And Patients Share Their Experiences

Richard Holmes
Brewster, Massachusetts



In 2005, my neurologist, Anthony Amato, M.D., Brigham and Women's Hospital, Boston, asked if I might participate in a drug study he was planning for patients with three types of muscular dystrophy – facioscapulohumeral, limb-girdle and Becker. It was to be a safety study of a myostatin inhibitor. There was some evidence of animals and humans with a genetic mutation that naturally suppressed myostatin in their bodies, causing them to develop stronger muscles. Dr. Amato said the drug would attempt to replicate this natural process.

The theory seemed plausible. There was – and is – no existing drug to

counter the chronic muscle-wasting of FSHD. The gene therapy I had read about sounded both risky and far from being developed into a realistic form of treatment. I knew there would be a lot of trips between Boston and my home on the Cape, 85 miles away, and a lot of testing. But the prospect excited me, as it was a way I could do something about my disease, even if it did not directly benefit me.

I did not really expect any positive results from the treatment, but, of course, I hoped for some. I knew that I might be one of the participants who received a placebo, and I also knew that I was in the first group being tested, and would therefore be getting the lowest dosage if I received the drug.

During my visits, there were other study participants getting their intravenous dose as I got mine. We asked each other if we had noticed any effects. Once or twice, someone remarked on a recent feeling of strength and wondered if the drug caused it.

The first cohort of the study ended, and we were told the results would not be forthcoming until after two subsequent higher dosage groups had been completed and the data analyzed. It would be a year or two before the results would be released and a wider study would be needed before the drug hit the market, if it proved safe and successful. We were in information limbo.

I finally learned in March by an e-mail from FSH Society executive director Nancy Van Zant that Wyeth Pharmaceuticals had decided not to pursue development of MYO-029. Apparently the drug did not work at the tested doses and caused some undesirable side effects at the highest dose tested. But the e-mail did say Wyeth intended to pursue similar paths of treatment, including other myostatin inhibitors. I was disappointed by the news, more by the failure of MYO-029 than by Wyeth's decision to drop it.

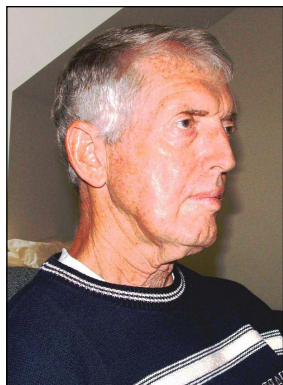
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Perspective on MYO-029 Clinical Trial, *continued from page 4*

I was glad to see they said they would continue related research.

I look forward to hearing more about Wyeth or another drug company pursuing research into myostatin inhibition or other promising areas to combat FSHD. If there is a way to help press for this – say, by writing drug companies – I would be glad to take part. I am thankful to the FSH Society for bringing the results of the MYO-029 study to my attention and keeping the issue alive.

David R. Anderson
Montross, Virginia



I am much more fortunate than many with this disease but each day brings many reminders of the slow but steady progression of muscle loss associated

with FSHD. Things that I used to take for granted like climbing stairs, getting out of a chair or carrying a bag of groceries now require a “plan” or special efforts. The thought of a drug that could slow the muscle wasting or even result in some slight recovery of strength and mobility is always on my mind, and I am sure on the minds of everyone with FSHD.

In 2005, I read with great interest an article in *Quest* magazine about a myostatin safety drug trial. Wyeth Pharmaceuticals was looking for participants in various locations including Children’s National Medical Center (CNMC), Washington, D.C., a two-hour drive from my home in Virginia. After exchanging information by mail and phone, I was disappointed to find I was not a candidate for further screening because I was taking a daily supplement of CoQ10.

In February of 2006, I made a fol-

low-up call to CNMC and to my surprise was told they were looking for participants for the fourth phase of the trial (the highest dosage). After reviewing all the documentation about the trial, I was eager to give it a try although not confident I would be accepted. As excited as I would be to participate, I had some concerns. How would I handle the physical logistics of commuting to Washington, getting around for all the testing required over four or five days, and being away from my own comfortable environment? Did I really want to make a commitment for thirteen infusions if I was accepted? Did I want my wife, JoAn, to endure the same long days and commute to Washington? Could I pass all the various screening tests? Did I want to go through the MRI process? I have never heard of anyone dying from an MRI, but personally I would rather have teeth extracted than to squeeze into one of those tight tunnels. Of least concern to me was safety of the drug which was the objective of the trial. I suspect many of us would “try about anything” to gain strength. Some might even consider using that stuff baseball players are accused of using. (Just kidding.)

With some anxiety, my wife and I made the initial visit to CNMC in February, 2006. After several weeks and thirteen different appointments, I was accepted into the trial. The comprehensive screening was the best physical and neurological assessment I had ever had. It included heart, lungs, muscle strength, eyesight, hearing, bone density, etc. I even “survived” the MRI although I did use a blindfold.

Robert Leschner, M.D., was the neurologist who conducted the initial neurological exam and would be monitoring me through the thirteen infusions over the next twenty-six-week period. He explained that there were eight people in this high dosage phase of the trial and two of the eight would be receiving a placebo. Before starting the initial infusion, Dr. Leschn-

er gave me his cell phone and home phone numbers and told me to call him anytime day or night if I had any concerns or problems. A doctor’s home phone number! It finally penetrated my thick head that this really is a “safety” study and there may be some risk involved. I experienced a brief moment of apprehension; it quickly passed because I knew that I would take a lot of risk for a little improvement and that I was lucky to have been accepted into the trial.

Beginning in mid-March 2006, I had an infusion every other week and had completed a total of three of the thirteen that were scheduled. I was feeling good and in fact thought I might even be a little stronger although I realized that this feeling may have been a placebo effect. I was extremely disappointed when just before the fourth infusion I was told everything was being put on hold because of some complications. A short time later I received a letter indicating there was a case of “suspected aseptic meningitis” and three participants experienced allergic reactions. This prompted Wyeth to discontinue the Cohort 4 high dosage test but they did continue the lower dosage Cohorts 2 and 3.

At this date in 2008, I still do not know if I had been receiving the placebo or the real article during the trial. It was very disappointing to read recently that Wyeth would not be continuing further activities on MYO-029, especially when the results indicated safety was not an issue and that there were some positive efficacy results. I really hope someone else picks this up for further testing and development.

Would I be interested in participating in another trial? Absolutely! Every person I met in the process was first rate, kind, caring and professional. It was a great experience and I still feel fortunate to have been part of the trial. To me and many others a trial like this one provides reason for optimism!

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Perspective on MYO-029 Clinical Trial, *continued from page 5*

Donald Custis Lokerson

New Carrollton, Maryland



As a student in junior high and high schools, I frequently fell while playing in the required sports. When attempting to pass gym test for the

30-foot-high-rope climb, I barely made it to the top, when loss of strength caused me to slide slowly downward, burning my hands. Back then, there was no such thing as being excused from gym activities because of muscle weakness.

Some years later in November 1979, I enjoyed a 50-mile hike down the C&O Canal tow path in 18 hours, and I felt exhilarated though very tired.

Then in early 1984, when I was 44 and visited an orthopedist, he referred me to a study being done at NIH on neuromuscular diseases. I knew I had some weakness in my arms and legs, but I had never heard of FSHD, the diagnosis that was delivered after much testing and a muscle biopsy from my arm.

In December 1990, we chanced to meet a choral group member, Karen Johnsen, who showed the shoulder winging and foot drop. She was just starting an FSH Society support group for FSHD in our greater Washington, D.C. area. In this group we shared experiences and met with others with similar symptoms and learned as much as we could from several experts who came to our group. In October 2001, as a part of the clinical registry of FSHD patients, I participated in a clinical trial at the University of Rochester where Rabi Tawil, M.D., took muscle samples from my left calf muscle.

In May 2004, Kathryn Wagner, M.D., Ph.D., was asked by the FSH Society to tell our support group about the clinical trial being established at The Johns Hopkins Hospital, among other sites,

by Wyeth Pharmaceuticals to study the effects of anti-myostatin on FSHD patients. I applied to participate in this trial and was very grateful to be accepted, even though I had to take off time from work to participate. Thorough physical exams at the beginning and end confirmed my general good health and very small changes during the testing period. My wife took detailed notes on my performance of muscle strength changes. The overall attitude of everyone who took care of me was upbeat and relaxed, keeping me at ease in what might have been quite a stressful experience.

In March 2008, we attended a "reunion" with Dr. Wagner and a few other trial participants at The Johns Hopkins Hospital, where I found out that I had been given a very low dose of anti-myostatin which was enough to improve muscle mass slightly but not enough to provide more strength. I was very disappointed that Wyeth was discontinuing the trial, as I was really looking forward to receiving whatever form of anti-myostatin which might have been approved by the FDA and experiencing some significant improvement. Dr. Wagner said that additional pharmaceutical companies are planning to continue studies for a successful myostatin inhibitor, and I fervently hope that the companies will keep the process Wyeth started going so that I, my sisters and brother, our friends in the FSH Society and all others with FSHD will share in its benefits.

Maximilian N. Teleki

Washington, D.C.

In August 2004, after the birth of our first child, our son Tibor who along



with his sisters is FSHD-free, I became aware through the FSH Society of the myostatin trial sponsored

by Wyeth and conducted at various hospitals around the country. Fortunately for me, one of the participating hospitals was Children's National Medical Center, Washington, D.C., which is 15 minutes from our home. This convenience was not afforded to most participants, whom I observed driving long distances or flying into Washington twice monthly or more.

After applying and being reviewed by a small panel of clinical specialists and physicians, I was accepted into the first cohort "safety trial," and I began my participation in September 2004. This seven-month process consisted of baseline, top to bottom tests, and bimonthly injections. These baseline tests consisted of: bone density scans, MRIs, EKGs, CT scans, eye exams, hearing exams, exercises, and a series of ongoing questionnaires. In addition to these requirements, I had to submit to regular physical examinations, along with blood and urine analysis. This process became an approximate commitment of 15 hours monthly, and it also absorbed a considerable amount of time, given the need to rest after the grueling days of injections and examinations. I suspect that the total time commitment was closer to 30 hours monthly once I factored in the time I needed to rest the day of a treatment and/or the day after.

All said, I was truly gratified to participate in this process and for the first time in years felt like I was taking a proactive approach that was somewhat empowering and had potential to help others and myself. Two very good and unrelated experiences gave me tremendous satisfaction. During my time at CNMC, I encountered children who had a variety of challenging illnesses, some fatal and some manageable. This not only reminded me of how fortunate I was personally, but it made me feel productive. I was able in some small measure to contribute to the lives of these children. While at the same facility and involved in the Wyeth trial, I experienced the same hope that this effort would collectively bear fruit for

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Perspective on MYO-029 Clinical Trial, *continued from page 6*

all FSH'ers down the road.

I do not want to give anyone the impression that it was a cakewalk. I had reservations and faced some health challenges during this period. I was exhausted, and had nausea or dizziness from time to time, and as a new father I felt somewhat removed from these first few months of my son's life. In the long run, it was a worthwhile experience that I hope will lead to Wyeth eventually resuming this study and beginning the second part of this effort. I hope the FSH Society will continue to work with Wyeth on resuming this collaborative relationship and to do what it does so well – laying groundwork for these clinical trials to come into being.

Don Burke

Alexandria, Virginia



In 2005, I participated in the Wyeth Pharmaceuticals MYO-029 medical trial that included study participants with facioscapulohumeral-, limb-girdle-, and

Becker muscular dystrophy. Deciding to participate in this trial, however, proved more difficult than I expected because of the fear and doubt that consumed my thoughts each step of the way. Would it be safe? Would I be on placebo? Am I a bad person for caring whether I was on a placebo or not? What do I tell my coworkers? What if it causes my FSHD to progress more rapidly? What if I learn something that I would rather not know?

In the end, the opportunity to participate in a trial aimed specifically at FSHD superseded all doubts. To my surprise and appreciation, I found myself immersed in a medical system that overflowed with passion for its work and for FSHD. I was surrounded by doctors, surgeons, nurses and aides who were attentive and engaged. I found myself looking forward to each visit and the exchange of information and knowledge.

Within seven days of the first infusion, I began to feel different. Just 13 days from my first infusion, my journal reads in part “I seem to be walking faster, getting out of bed easier. In general the feelings from May 11, 2005, remain intact with the addition of the increased appetite.” When I visited my family in Minnesota, my aunt commented that I looked “fuller” which had become a common refrain by those who have known me for a long time. By the end of June, I had stopped wearing my home-made foot brace and found a dexterity and balance unlike at any other time in many years. Was this real or placebo? I had no way of knowing.

My time in the trial came to its natural end and then came the long silence. Years went by without any news. I and my fellow participants were left to wonder why we bothered to contribute to the trial. We had all but given up hope that the results would ever be published when in early 2008, Kathryn

Wagner, the principal investigator called us together and gave us the news that there were no significant indications of efficacy across the cohorts. There was more to the story though as there were some indications, though not statistically significant, that FSHD study participants benefited and that I specifically had received the real drug. My test results showed measurable improvement thus confirming my personal observations and notes. I cannot imagine a more frustrating outcome than to have benefited myself and have the drug shelved. Now almost three years later, my body grows weaker; I trip and fall; and I have lost the dexterity I gained during the trial. Despite all this, I continue to feel privileged to have participated and will forever cherish the people who made me believe in the medical system. I am more confident than ever that we will find treatments and eventually a cure for FSHD. ♦

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

Dear Friends,

I am delighted to report that our members in New York, New Jersey, Connecticut, and points beyond, had a wonderful evening on April 22, at the New York Botanical Garden. Fellow members Robert and Abigail Kirsch hosted the reception and dinner for 125 people in attendance.

My wife, Duncan, and I hoped that we might attend, but commitments kept us in California. I was pleased to learn that guests enjoyed the program, presented by Kathryn Wagner, M.D., Ph.D., The Johns Hopkins Hospital; Howard Worman, M.D., Columbia University; and Michio Hirano, M.D., Columbia University Medical Center. Fellow director, James A. Chin, Sr., served as master of ceremonies. Judy Seslowe, member, invited others to join with her to plan the next event for the Society, and member, Sanford L. Batkin, offered thanks before dinner.

We would like to hold more events

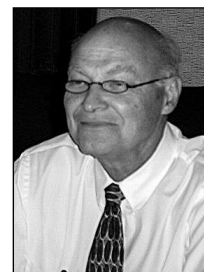
such as this one. If you or someone you know is interested in hosting something in your community, please let us know.

Similarly, the good work of the Society in the interest of FSHD patients and their families is made possible by your membership and gifts. Please consider the gift you can make at this time.

On behalf of all the patients and their families, I thank Robert and Abigail Kirsch for their generous support of the Society through this lovely evening at the New York Botanical Garden.

Sincerely,

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



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

FSHD Advocacy Efforts Continue with Testimonies to the U.S. House and U.S. Senate

The FSH Society provided testimony again this year before the U.S. Senate Appropriations Subcommittee on Labor, Health and Human Services, Education and Related Agencies on funding for fiscal year 2009 for the National Institutes of Health (NIH), making recommendations on the funding of research grants for facioscapulo-humeral muscular dystrophy (FSHD). The Society presented its testimony to Subcommittee Chairman Senator Tom Harkin (D-IA) and Ranking Member Senator Arlen Specter (R-PA) on April 27, 2008. The Society presented testimony to the U.S. House Appropriations Subcommittee Chair, Representative David Obey (D-WI), on March 20, 2008

FSH Society President & CEO, Daniel Paul Perez, asked for immediate and necessary help for those of us coping with and dying from FSHD and all muscular dystrophies. **Specifically we asked the congressional appropriations subcommittee to:**

1. Resume the five year doubling of the NIH budget. Over the past year the research funding situation has gone from bad to worse and opportunities have been lost to fund excellent research.
2. Appropriate \$80 million to MD research at the NIH in FY2009 and steadily increase this amount to at least \$125 million annually over the next five years.
3. Make NIH funding comprehensive for basic research in each of the nine types of MD as well as creating an equitable distribution for each MD across the Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center network, program projects, basic research projects, clinical research, training programs and translational research programs.

We asked that the mandate to the NIH be to have centers and a comprehensive research portfolio *in each* of the muscular dystrophies, rather than centers and a comprehensive research portfolio *in all* of the muscular dystrophies. This seemingly insignificant one word change transforms death into life

for all patients and families with MD.

The Society applauds Story Landis, M.D., Director, National Institute of Neurological Disorders and Stroke (NINDS), and current Chair of the Muscular Dystrophy Coordinating Committee (MDCC); Stephen I. Katz, M.D., Ph.D., Director, National Institute of Arthritis and Musculoskeletal Disorders (NIAMS) and past-Chair of the MDCC; John Porter, Ph.D., Program Director Muscular Dystrophy, NINDS, and Executive Secretary of the MDCC; and Glen Nuckolls, Ph.D., Program Director Muscular Dystrophy, NIAMS, for the extraordinary comprehension and insight with which the NIH Action Plan for Muscular Dystrophy was researched, compiled and approved. The NIH is making significant investments to understand muscular dystrophy research needs and has made excellent choices in recruiting program staff with the ability to understand the extremely complex nature of each of the muscular dystrophies.

The FSH Society compiles data from the NIH to measure the level of specific funding for FSHD in particular and for muscular dystrophy in general. Funding history was obtained from the NIH under the Freedom of Information Act. Steady progress has been made in increasing muscular dystrophy funding since the Society was formed in 1989.

Between fiscal year 2006 and 2007, NIH overall funding for muscular dystrophy research grew from \$39,913,000 to \$47,179,000, an 18 percent increase. From the inception of the MD CARE Act in 2001, funding has doubled for muscular dystrophy.

Between fiscal year 2006 and 2007, NIH funding for FSHD increased from \$1,732,655 to \$4,108,555, a 137.1% increase. In fiscal 2007, FSHD was 8.7% of the total muscular dystrophy funding (\$4,109,000 out of \$47,179,000).

In 1988, 20 years ago and a year after we began to organize the FSH Society, \$4.3 million was the total NIH commitment for all muscular dystrophies. ♦

National Institutes of Health (NIH) Appropriations History

Source: NIH/OD Budget Office & NIH OCPL (Dollars in millions)

Fiscal Year	NIH Overall Dollars	MD Research Dollars	FSHD Research Dollars	FSHD % of MD
2000	\$17,821	\$12.6	\$0.4	3%
2001	\$20,458	\$21.0	\$0.5	2%
2002	\$23,296	\$27.6	\$1.3	5%
2003	\$27,067	\$39.1	\$1.5	4%
2004	\$27,887	\$38.7	\$2.2	6%
2005	\$28,494	\$39.5	\$2.0	5%
2006	\$28,587	\$39.9	\$1.7	4%
2007	\$28,899	\$47.2	\$4.1	8.7%
2008	\$29,230 <i>Est</i>	\$47.2 <i>Est</i>	-	-

To see more details on how each NIH Institute and other federal agencies are doing with respect to FSHD, please see complete testimonies at www.fshsociety.org. FSH Society advocacy and educational efforts on FSHD are yielding excellent dividends! Your continued support and contributions to the Society are vital in helping create large scale research and clinical efforts. If you are interested in becoming more involved in grassroots efforts, please contact us! ♦

Facebook and MySpace: The Society Steps Into Social Networking

By Mickey Slevin

The way people are connecting is changing, and that includes those affected by FSHD. The FSH Society, in keeping with web 2.0 technologies, has published important social networking tools on MySpace™, www.myspace.com/fshsociety, and Facebook™, www.facebook.com; search for “FSH Society” or “facioscapulohumeral” in the search box.

By becoming “friends” of the Society online, members can connect directly with others involved with FSHD and more specifically, the FSH Society. The page will host updates about research and Society events.

The Society and/or members can post pictures, videos, or other media that they find relevant or that other members might find interesting. If there are breakthroughs in research or if FSHD is in the news, it will be found right

on the page. If members have their own insights, there is the wall feature where registered “friends” can post thoughts and questions. For more specific topics, members, with only a click of the mouse, can create a forum of their own within the webpage to open discussion on a subject of their choice. Both sites are monitored



daily to avoid spam or virtual vandalism.

Signing up for either website takes only a few minutes. Anyone, no matter age, location, or occupation, can register as long as one has an e-mail address. The websites are easy to use and becoming a registered “friend” of the Society takes only a few clicks of the mouse.

Photos from the gathering of members at the New York Botanical Garden on April 22, 2008, are up on the Facebook™ website. If you missed the dinner in New York, please check the events section for information regarding the 2008 FSH Society International Patient and Researcher Network Meeting, which takes place July 26-28 in Iowa City/Coralville, Iowa. People attending the conference can arrange to meet others by connecting in advance on MySpace™ and Facebook™. Since people are flying in from around the world, forming a network before you reach the conference can help to make arrival easier and the conference that much more worthwhile. The websites will also allow those who meet through the gatherings or other Society happenings, to stay connected long after they leave.

Check it out! ✧

Patients and Researchers to Meet in Coralville/Iowa City, July 27, 2008

Dear Friends,

We invite you to join with patients, family members, researchers and clinicians concerned with facioscapulohumeral muscular dystrophy (FSHD) for the 2008 FSH Society International Patient and Researcher Network Meeting, July 26-28, 2008.

Saturday, July 26, is an unstructured day for patients and families with time for touring early in the day, and a

Reception and Greeting, 4:00 – 7:00 p.m. The conference will begin at 7:30 a.m. on Sunday with Registration and Breakfast. We have planned a program of lectures and discussions to bring current clinical and research advances in FSHD to our community. We conclude with a continental breakfast and farewell on Monday. Katherine Mathews, M.D., will also offer a clinic on Monday for patients with infantile FSHD. (See page 12 for more information.)



Nancy Van Zant

The program and the registration form are in this issue. Please mail your registration in the enclosed envelope by June 27. Hotel reservations must be made by July 4, to secure the special group rate. Childcare may also be arranged for a limited number of children.

The Society holds this meeting because you – patients and families – have requested it. Many of you tell us that it is a life-changing event. We hope that you will join us in Iowa in July.

The 2008 FSHD patient and researcher meeting and the Society’s programs of education and research are made possible only through generous donations and membership in the FSH Society. Please consider renewing your 2008 membership at this time or make a special contribution to support this meeting.

Sincerely,

Nancy Van Zant
Executive Director

GoodSearch: You Search, We Give

By Arlene Cohen

How would you like to raise money for the FSH Society without leaving your house, or asking friends one more time to buy something that you know they do not need? Better yet, the longer you keep your computer chair warm the more money we raise. How so, you say? By searching the internet.

GoodSearch is an internet search engine launched in 2005, by Ken Ramberg who realized that search engine advertisements generated \$8 billion annually. *GoodSearch* donates 50 percent of its revenue to the charities designated by its users; the FSH Society is already set up to benefit. In 2007, *GoodShop* was added to the search engine as

an online shopping mall also able to benefit charities by its use.

What the FSH Society is asking you to consider is to use *GoodSearch.com* as your primary choice of search engines.

To set it up on your computer please follow these simple instructions:

- Go to:
www.goodsearch.com
- Click on: WHO DO YOU GOODSEARCH FOR?
- Enter: FSH Society
- Click on: Add Google search to your Internet Explorer, Firefox, or Mac Toolbar

It is simple to install and even easier to spread the word. Tell your friends, family and co-workers, so

they too can start using *GoodSearch* to support FSHD! What a great way to support the FSH Society! More funding, more research, closer to a cure...

Read about *GoodShop*, and earn money for the FSH Society when you shop online at Best Buy, Target, PetSmart, Staples, Gap and others! ♦



Justin Cohen powering up GoodSearch

FSHD Future Fund Launched

Earlier this year, members of the board of directors shared with the FSH Society that their wills or other trust documents include a bequest to the Society. By way of this good news, the Society launched the **FSHD Future Fund**, and declared Barbara and Jim Chin, Bill and Ginny Michael, Judy and Bill Herzberg, M.D., JoAnn Forance, Bob Smith, Howard Chabner

and Michele DeSha, charter members.

We can all help the Society and its future work – become a charter member of the **FSHD Future Fund** by including a bequest to the Society in your will or other estate planning documents.

If you have already included the FSH Society in your will, we hope you will let us know. If you will allow the Soci-

ety to recognize your dedication in our **Annual Donor Report**, your example might inspire others. If you have questions about your planning and how it can support the work of the Society in the future, or if you would like a copy of the booklet, *Questions and Answers about Wills and Bequests*, contact the Society office: (617) 658-7878, or e-mail nancy.vanzant@fshsociety.org. ♦

Gathering of FSH Society members at the New York Botanical Garden, April 22, 2008

Robert and Abigail Kirsch, hosts for the reception and dinner in the Garden Terrace Room



Left to right: Stuart Lai, Barbara and James A. Chin, Sr.

United Airlines Foundation Honors Employees for Community Service and Makes a Gift to the Society

The United Airlines Foundation, the charitable arm of United Airlines, awarded grants to 46 organizations, including the FSH Society, for which employees performed volunteer service. Glenn Tilton, chairman, president and CEO, and Pete McDonald, executive vice president and chief operating officer, presented the awards to recipients in November 2007.

United individual volunteer grants recognize employees for their individual commitments to community service and are awarded to individual organizations in the names of their employee volunteers. The Society's award was made in the name of FSH Society member and volunteer Janice Gilligan, change management analyst for United. The grant of \$1,000 resulted through a random drawing from the many grant applications submitted.

The award ceremony, which took place at the United Training Center in Elk Grove Village, Illinois, honored employees for their dedication to community service. ♦

Glenn Tilton, chairman, president and CEO of United Airlines, FSH Society member, Janice Gilligan, and Pete McDonald, executive vice president and chief operating officer at United



Easy Way to Contribute to the FSH Society

Member Deborah Schwartz writes...

I had a thought regarding fundraising. Every month when we get credit card statements, there are points accumulating. I noticed that one can actually write checks using these points: 2500 points = \$25.00. This seems an easy way to make a gift to the FSH Society.

So, I decided to try. I was successful in sending the FSH Society a check for \$25.00 using Chase credit card points. The Society received the check in five days.

I hope others will try this, too! ♦



10th Annual End of Tax Season Fundraiser: for the Record Books

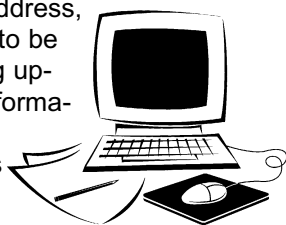
Christopher and Ellen Stenmon, together with friends, family, co-workers and his firm, O'Connor and Drew, P.C., once again held an end-of-tax-season party and raised important funds for the FSH Society. The 2008 event is the tenth and raised over \$15,000 – a record for the event!

The Society is grateful to Chris and Ellen and all the other generous people who contributed to this great event in Quincy, Massachusetts, on April 19. Chris, a member of the Society's board of directors, has been involved with the Society since he was sixteen years old. ♦

Is Your E-mail Address Current at the Society?

We communicate with many of you by e-mail and many of you encourage the Society to use more electronic media. New databases and software will make this easier in the coming months. If we do not have your current e-mail address, and if you want to be sure of receiving up-to-the-minute information from the Society as news breaks, please e-mail your address to us – for the first time or when you have changes.

E-mail jennifer.lazzaro@fshsociety.org. Thank you! ♦



Combined Federal Campaign (CFC) 2008 Campaign

The FSH Society has been approved by the Office of Personnel Management for the 2008 CFC campaign. The CFC is the world's largest and most successful annual workplace charity campaign, with more than 300 CFC campaigns throughout the country and internationally to help to raise millions of dollars each year. Pledges made by Federal civilian, postal and military donors during the campaign season (September 1st to December 15th) support eligible non-profit organizations that provide health and human service benefits throughout the world. **The FSH Society's CFC identification number is 10239.** ♦

Betsy Lewis Conron, daughter of Duncan and William R. Lewis, Sr., M.D., chairman of the Society's board, asked her friends to make contributions to the Society instead of gifts to her at her recent birthday party. From left to right: Katrina Losee, Melissa Eason, Betsy, her sister Linda Lewis Stapleton, and Janis Taormina.



2008 FSH Society International Patient Researcher Network Day

Saturday, July 26, 2008 – Monday, July 28, 2008

Coralville Marriott Hotel & Conference Center

300 East 9th Street, Coralville, Iowa 52241 Phone: (319) 688-4000

<http://www.marriott.com/hotels/travel/cidic-coralville-marriott-hotel-and-conference-center/>

Saturday, July 26, 2008

4:00 – 7:00 p.m. Greeting and Reception. Registration.

Sunday, July 27, 2008

7:30 – 8:25 a.m. Registration and Breakfast Buffet

8:25 a.m. Welcome

Daniel Paul Perez, *President & CEO, FSH Society*
Nancy Van Zant, *Executive Director, FSH Society*

I. Health Information You Need to Know! 8:25 a.m. – 9:55 p.m.

8:25 – 8:30 a.m. Welcome

William R. Lewis, Sr., M.D.,
Chairman, Board of Directors, FSH Society, Inc.

The William T. "Billy" Michael Memorial Lecture for Research on Infantile FSHD

8:30 – 8:55 a.m. Clinical Medicine and Research

Advances in Infantile and Adult FSHD

Katherine Mathews, M.D., *Division Head, Child Neurology*
University of Iowa, Department of Neurology, Iowa City, Iowa USA

8:55 – 9:20 a.m. Advances in Physical Therapy and FSHD

Wendy M. King, P.T., *Assistant Professor, Neurology*
The Ohio State University and the Research
Institute at Nationwide Children's Hospital, Columbus, Ohio USA

9:20 – 9:45 a.m. Respiratory Therapy and Issues in FSHD

Noah Lechtzin, M.D., MHS, *Assistant Professor, Medicine and*
Neurology; Pulmonary Director, Johns Hopkins ALS Center, Johns
Hopkins University School of Medicine, Baltimore, Maryland, USA

9:45 – 9:55 a.m. Break

II. Helping to Solve FSHD! 9:55 a.m. – 12:30 p.m.

9:55 – 10:00 a.m. Reconvene

William R. Lewis, Sr., M.D., *Chairman, Board of Directors, FSH Society, Inc.*

10:00 – 10:25 a.m. New Insights in FSHD Research

Louis M. Kunkel, Ph.D., *Professor of Pediatrics and of Genetics*
at Children's Hospital Boston and Harvard Medical School,
and Howard Hughes Medical Institute Investigator,
Children's Hospital, Boston, Massachusetts USA

10:25 – 10:50 a.m. Therapies, Compounds and

Strategies to Treat FSHD

Kathryn Wagner, M.D., Ph.D., *Associate Professor of Neurology and*
Neuro-science; co-director, Johns Hopkins MDAUSA Clinic and
co-director, Johns Hopkins/University of Pennsylvania Senator Paul
Wellstone Muscular Dystrophy Cooperative Research Center
The Johns Hopkins Hospital, Baltimore, Maryland USA

10:50 – 11:15 a.m. FSHD Research Program and Funding

Advances at the Federal Agency Level

John D. Porter, Ph.D., *Executive Secretary, Muscular Dystrophy*
Coordinating Committee; and Program Director, National Institutes of
Health National Institute of Neurological Disorders and Stroke (NINDS)
Neuromuscular Disease Neurogenetics Cluster; and the NINDS
Technology Development Program, Bethesda, Maryland USA

11:15 – 11:25 a.m. Break

11:25 – 12:30 p.m. Panel/Audience Discussion: Questions and Answers

III. Lunch 12:30 – 1:30 p.m.

IV. Breakout Discussion Groups 1:30 p.m. – 4:15 p.m.

1:30 – 2:45 p.m. First session: Four Concurrent Workshops

3:00 – 4:15 p.m. Second session: Four Concurrent Workshops

1:30 – 2:45 p.m. Workshop IA: Getting to Know You/Taking Care of Ourselves – A session to talk with others about FSHD

3:00 – 4:15 p.m. Workshop IB: Taking Care of Ourselves for Teenagers and Young Adults with FSHD – A session for patients to talk with each other and with physicians and researchers about FSHD

1:30 – 2:45 p.m. and 3:00 – 4:15 p.m. Workshop II: Caregivers: Spouses, Significant Others and Family Members
A session to talk with other caregivers

1:30 – 2:45 p.m. and 3:00 – 4:15 p.m. Workshop III: More Q&A with Researchers and Clinicians – A session to meet and collaborate with physicians and researchers

1:30 – 2:45 p.m. Workshop IVA: Teenagers and Young Adults: Networking and Making Plans – A session to share experiences and to consider how to take a greater role in the Society

3:00 – 4:15 p.m. Workshop IVB: Self-Advocacy and Patient Advocacy
A session to learn about patient rights and legal issues

V. Conclusion and Adjournment: 4:15 – 5:00 p.m.

4:20 p.m. Concluding Remarks

5:00 p.m. Adjourn

Monday, July 28, 2008

8:00 – 10:00 a.m. Continental Breakfast at the Coralville Marriott

8:00 a.m. – 5:00 p.m. Infantile FSHD Clinic at the University of Iowa Hospitals and Clinic

Katherine Mathews, M.D., and Clinic Staff

Clinic Overview

The early onset (infantile) FSHD clinic to be held at the University of Iowa, Department of Neurology, in Iowa City will offer the opportunity to meet with specialized staff with experience in treating patients with FSHD. Personnel at the clinic will include a pediatric neuromuscular physician, neuromuscular nurse/genetic counselor, speech and hearing specialists, and physical therapist. Through collection of information from families prior to the clinic, the clinic experience will be tailored to insure that issues of greatest interest are addressed.

The clinic is open to persons with FSHD who are 20 years of age and younger who experienced weakness (excluding those with isolated face weakness) by age 10 years. Patients (or parents) will be asked to complete a short medical history which will be mailed prior to the date of the clinic. In addition, selected medical records including DNA results, recent physical therapy, speech pathology, audiology, and ophthalmology notes should be sent to the University of Iowa prior to the date of the clinic, if possible.

Anyone interested in more information about the clinic should call Carrie Stephan, R.N., (319) 356-2673 or e-mail carrie-stephan@uiowa.edu.

The FSH Society wishes to thank the 2008 Network Conference Speakers, the 2008 Network Conference Committee and all attendees for their contributions to the success of this meeting. Sponsors to date include:

The Association Française Contre les Myopathies (AFM)
Athena Diagnostics, Inc. • Acceleron Pharma

The Facioscapulohumeral Muscular Dystrophy Society (FSH Society) is an independent, non-profit 501(c)(3) and tax-exempt U.S. corporation organized to address issues and needs specifically related to Facioscapulohumeral Muscular Dystrophy (FSHD). Contributions are acknowledged for tax purposes. All inquiries should be addressed to the FSH Society, Inc., Nancy Van Zant, BBRI R353, 64 Grove Street, Massachusetts 02472 Phone: (617) 658-7878, fax: (617) 658-7879, e-mail: 2008Meeting@fshsociety.org, website: <http://www.fshsociety.org>