Friedreich's Ataxia Research Alliance UPDATE

Friedreich's Ataxia Research Alliance (FARA)
2001 Jefferson Davis Highway, Suite 209
Arlington, Virginia, USA 22202
PHONE 703/413-4468 FAX 703/413-4467 EMAIL fara@frda.org WEB: www.frda.org

Special Conference Issue 2003

FLASH! FARA Grants Pass $1 Million Mark

FARA, established in September 1998, gave its first research grant in April 1999. With the generosity of donors, the tireless commitment of people conducting fundraisers, co-funding by Seek A Miracle/MDA, and the increasing tempo of scientific progress, **FARA has awarded research grants now totaling more than a million dollars.**

FARA Assembles World's Top Scientists at NIH

In February 2003, FARA and NIH again collaborated to fund, organize and co-host the second international Friedreich's ataxia (FRDA) scientific research conference at NIH in Bethesda, Maryland, outside Washington, D.C. About **100 scientists from 12 different countries came together to compare findings, share insights and chart the course ahead in the search for treatments and a cure for FRDA.** Dr. Audrey Penn, Acting Director of NIH's National Institute of Neurological Disorders and Stroke (NINDS), made the opening presentation. She thanked FARA and the participants and highlighted her Institute's active role in FRDA research.

On the "final" day of the conference, the "Blizzard of '03" struck the Washington D.C. area, and its two feet of snow were a blessing in disguise. All the scientists were able to make their presentations on schedule before they were stranded in their hotel for two additional days, providing them the opportunity to continue their discussions and explore additional collaborations. A great deal was accomplished across what became a five-day conference.(*Cont'd on p. 2)*

IN THIS UPDATE...

FARA Officers, Directors, and volunteers at the '03 conference. (L to R) Ron Bartek, Rochelle Litke, Terry Downing, BJ Acker-Hitta, Sandy Lane, Bronya Keats, Mary Caruso, Steve Lane, Sue Kittel, Marilyn Downing. Not pictured: Raychel Bartek, Fraser Goodmurphy, Bill Hartnett, Marty Litke, Massimo Pandolfo, Rob Wilson, David Doremus, Barbara MacDonald. Photo: Art Rossomando.

Friedreich's ataxia is a life shortening, debilitating and rare genetic neurodegenerative disorder. Onset of symptoms usually occurs between the ages of 5 and 15. Symptoms include muscle weakness and loss of coordination in the arms and legs; impairment of vision, hearing and speech; aggressive scoliosis (curvature of the spine); diabetes; and a serious heart condition. Most patients need a wheelchair full-time by their late teens or early twenties. There is no cure. Most childhood-onset patients with this disease die in early adulthood. FARA is a 501(c)(3) tax-exempt non-profit organization. 100% of FARA donations are dedicated to supporting research leading to a treatment or cure for this relentless and devastating disease.

FARA/NIH Conference (Cont'd from p. 1)

100 top scientists from 12 countries meet at the FARA/NIH International Scientific Research Conference on Friedreich's Ataxia at NIH, February 14-18, 2003. FARA organized the conference and co-funded and co-hosted it with NIH.

FARA held its first scientific conference at NIH in 1999 and much of the 2003 conference evolved from that earlier workshop. **FRDA researchers say this is one of the fastest tracks they have ever seen in medical research: FRDA research has moved from gene discovery to promising clinical trials in only seven years.**

Therapeutic Approaches Explored — the Gene, Protein, Antioxidants

The recent conference made clear that **much progress has been made on the FRDA gene and on antioxidant development and therapies,** though there is a need to understand more fully the **function of the frataxin protein**. As articles in this Update will demonstrate, FARA is supporting excellent research across the full spectrum of these FRDA questions, and the 2003 conference allowed research groups from around the world to discuss their ideas in detail, face to face, testing their insights against those of their peers.

For both the 1999 and the 2003 conferences, **Dr. Rob Wilson** of the University of Pennsylvania served as the **Principal Investigator**. In both cases, Dr. Wilson applied his comprehensive knowledge of FRDA science and the FRDA scientific community to the Herculean efforts involved in organizing the agenda, assembling the list of participants, preparing and submitting the NIH conference grant application, and chairing the conference proceedings. FARA and all FRDA families are deeply indebted to Dr. Wilson. He is a member of FARA's Board of Directors and Chairman of FARA's Scientific Review Committee. He is also featured in this issue of the Update as FARA's first Featured FRDA Scientist.

Ataxia Scales — Required Clinical Measures Being Developed

The recent conference also brought to light **progress on developing the kinds of clinical measures -- or ataxia scales** -- that are absolutely **essential in verifying** to Food and Drug Administration (FDA) satisfaction whether any of the proposed **therapies will be effective**. FRDA researchers must show that the FRDA drugs on trial meet FDA standards for safety and efficacy if a drug is to be prescribed in the US, and such ataxia scales are used to demonstrate when those standards have been met. Once a drug is shown to be safe and effective to FDA standards, the FDA can approve it for use in FRDA so it can be prescribed and covered according to the patient's health insurance benefits.

FARA/NIH Conference (cont'd from p. 2)

Progress on Animal Models

Giant strides have also been made in developing the kinds of **animal models** that are an essential bridge between basic scientific research and clinical trials in humans. Scientists have been successful in producing mouse models that approximate the human FRDA condition and are distributing those mice to laboratories ready to use the models to develop therapeutic approaches. **FARA has helped fund the development of these animal models, has helped facilitate their distribution, is helping fund drug experiments using the models, and is helping fund the attempts underway to improve the mouse models so that they more closely replicate the human FRDA condition.**

High-Throughput Screening Accelerates Search for Effective Treatment

Another important research tool discussed at both the 1999 and 2003 conferences is called **high-throughput drug screening**. In this procedure, genetic or cellular FRDA samples are placed in hundreds or even thousands of small "wells" etched into glass laboratory plates and a different drug compound is placed in each of these wells. Also added to each well is a chemical that will change color in the presence of the molecule of key interest – the frataxin protein, or the messenger RNA used in making frataxin protein, or free iron, for example. Drug compounds that show promise in this procedure by producing favorable effects (an increase of frataxin protein, for example), can then be considered for use in the FRDA mice and, eventually, in human patients. Several FRDA scientists that participated in both FRDA conferences are now using this rapid drug-screening technique to identify the most promising drug compounds for clinical trials. **FARA has been supporting much of this work and has been successful, too, in helping integrate it with the centralized NIH program designed to accelerate the transition to clinical trials of drug compounds identified in such high-throughput screening and animal studies.**

Drug Development Companies and Partner Advocacy Organizations

Another important difference between the 1999 conference and the one held in 2003 was that a number of drug development companies asked to be invited to participate in this year's meeting. Not only did they participate but it became evident at the conference that each of these companies is eager to play a role in FRDA drug therapies. These companies would not be interested if there were not very promising prospects on the near horizon. In fact, it is now clear that these companies are interested in playing important roles in FRDA clinical trials right away.

This conference also **brought together representatives of key groups that support FRDA research** around the world. FARA invited representatives from **Seek A Miracle/MDA, MDA, the Friedreich's Ataxia Parents Group (FAPG), the National Ataxia Foundation, Euro-Ataxia, UK Ataxia, and the New South Wales Australia Ataxia Support Group**. FARA and all the groups that attended had the opportunity to collaborate and discuss how we all might work even better together to support FRDA research.

Conclusion

All of this is promising news. Many FRDA patients today are taking antioxidants, including Idebenone, which makes ours the first generation ever to use compounds that have the promise of slowing down or possibly even reversing some of the FRDA symptoms — only a dream just a few years ago. Now on the horizon and under investigation are gene-based and protein-based therapies and therapeutic compounds that target the mitochondria. FRDA research is advancing at an impressive pace. We have come a long way and we can't stop now. **We must all keep working to cross the finish line together by supporting the research through our donations and participation in clinical trials.** [A summary of the February conference written by scientific participants will be available in "Neuromuscular Disorders" 2004 Jan;14(1):70-82; Seznec H, Wilson RB, Puccio H.].

Advances Toward FRDA Gene-Based Therapy

February Conference Highlights Progress

At February's scientific conference, the first session focused on the FRDA gene and it illustrated that **FRDA scientists understand the disease gene** quite well, both in its classic triplet-repeat form and FRDA point mutations as well. **Dr. Robert Wells** of Texas A&M chaired this first session. Dr.Wells is one of the world's leading experts on triplet-repeat disorders like FRDA, serves on FARA's Scientific Advisory Board, and has received several FARA grants. Also making presentations during the session were **Dr. Sanjay Bidichandani** (FARA grant recipient) of the University of Oklahoma, **Dr. Ed Grabczyk** (FARA grant recipient) of Louisiana State University, **Dr. Marek Napierala** of Texas A&M, **Dr. Cizia Gellera** of the National Neurological Institute of Milan, **Dr. Chris Everett** of the National Institute of Neurology, London, and **Dr. Michael Brown** of Mercer University.

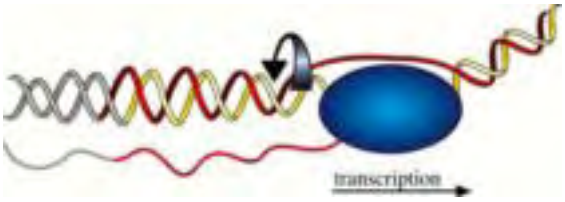
Dr. Robert Wells

These scientists are intently **exploring prospective gene-based repair mechanisms**. Their various research projects include a wide range of promising approaches. One such approach involves what Dr. Wells has referred to as **molecular surgery – an attempt to design and deliver a molecule that would sever the triplet-repeat expansion from the FRDA gene**. If the triplet-repeat expansion were removed from the gene, the gene's code could be transcribed more readily and more frataxin protein could be produced.

Another approach is to attempt to **complex onto the triplet-repeat expansion region** of the FRDA gene additional pieces of **DNA (oligonucleotides) that would interrupt or stabilize the expansion**. If the expansion could be interrupted or stabilized effectively, it might not "supercoil" and tangle into the "sticky DNA" that makes it difficult for the gene's code to be transcribed and the frataxin protein to be produced. **Dr. Chris Everett** and other scientists working at the National Institute of Neurology in London believe they have evidence indicating that **the triplet-repeat expansions might have some negative impact on chromatin – the substance in which DNA is packaged** in the chromosome -- and that the consequent changes in chromatin might be responsible for the FRDA gene expressing less frataxin protein. **If so, a different therapeutic avenue would open**, in that scientists could identify agents able to reverse the changes in chromatin. (Cont'd on p. 5)

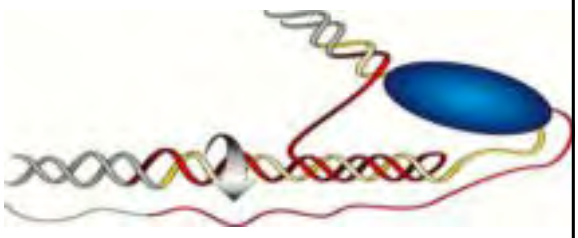
.

Miriam Martinez, Ed Grabczyk
Mimi Sammarco at LSU



Transcription molecule reading FRDA gene.

Slides by
E. Grabczyk



Expansion begins to supercoil into sticky DNA

FARA would like to acknowledge gratefully the corporate sponsor who has so generously funded, once again, the publication and distribution of this Update - Mehl, Griffin and Bartek Ltd. of Arlington, VA. Such generous support makes it possible for FARA to assure donors that **Every dollar donated to FARA goes to support FRDA research**. FARA is an all-volunteer organization with no administrative or overhead costs.

Gene-Based Therapy (cont'd from p. 4)

Another approach is based on the knowledge that the FRDA **triplet-repeat expansions are naturally unstable between generations** and differ among cell types. FRDA scientists, for example, estimate that **fathers, 90 percent of the time, pass along to their offspring shorter FRDA triplet-repeat expansions** than they have themselves, while **mothers pass along shorter expansions at about the same rate as they pass along longer expansions**. FRDA scientists also know that FRDA expansion lengths are unstable in somatic (non-reproductive) cell division, leading to some variation in repeat lengths among cells – a phenomenon referred to as **mosaicism**. The more FRDA scientists can learn about such generational and cellular differences and the factors that cause them, the more likely it is that they **will be able to manipulate or replicate such differences so as to encourage or force reductions in expansions and consequent reductions in the severity of FRDA symptoms**.

According to **Dr. Massimo Pandolfo**, a member of FARA's Board of Directors and its Scientific Review Committee, a direct **correlation has been established between the size of the GAA repeat lengths (especially the smaller of the two) and earlier age of onset, earlier need for a wheelchair, more rapid rate of disease progression, and presence of "non-obligatory" disease manifestations (e.g., cardiomyopathy, diabetes)**. However, Dr. Pandolfo adds that differences in repeat lengths account for only about half of the variations in age of onset, indicating that **other factors**, such as mosaicism, modifying genes and the environment, could **possibly influence disease progression**. A number of FRDA scientists are attempting **to decipher some of those modifying factors in hopes of establishing the basis of a therapeutic avenue**.

Knowing, for example, that **Acadian (Cajun) FRDA patients apparently have somewhat later onset and milder symptoms** than would be anticipated from their expansion lengths, some FRDA scientists have undertaken to identify the differences between Cajun and non-Cajun FRDA genes. They hope to determine what differences in the Cajun genes result in milder symptoms so as to explore how **such differences could be replicated in a therapeutic approach**. **Dr. Bronya Keats** of Louisiana State University, a member of FARA's Board of Directors and Acting Chairperson of its Scientific Review Committee, has been a leader in this effort. **Dr. Grabczyk** and **Dr. Wells** have also made valuable contributions in this effort, and **Dr. Karen Usdin** at NIH is pursuing a project in this area as well.

Dr. Bronya Keats

Dr. Michael Brown, a geneticist specializing in mitochondria at Mercer University, recently received a FARA grant to investigate another possible explanation of these FRDA variations. Mitochondria have their own DNA and Dr. Brown is studying samples from FRDA patients to test his hypothesis that **mitochondrial DNA might have a modifying impact on FRDA** phenotypes (symptoms). He is studying Cajun-patient samples being provided by **Dr. Keats**, and non-Acadian patients being provided by **Dr. David Lynch** at the University of Pennsylvania and **Dr. "Chip" Wilmot** at Emory University.

Just prior to the February conference, FARA had heard about an exciting new gene-based approach being explored in Duchenne Muscular Dystrophy by a team in Australia. FARA contacted the Australian team, asked if its approach might be applicable to FRDA, and invited the team's leader, **Dr. Robert Kapsa**, to present his work at the FRDA conference.

Dr. Kapsa explained that his approach involves introducing to the appropriate portion of the disease gene a small amount of DNA that would serve to bypass or "patch over" the genetic defect. The approach was developed for Duchenne Muscular Dystrophy which, like the vast majority of diseases, is a point-mutation

(Cont'd on p. 6)

Gene-Based Therapies (cont'd from p. 5)

disorder, but Dr. Kapsa reported that he believed that, if the technique could be developed effectively, it could be applied to FRDA point mutations as well as the triplet-repeat expansions. **In a point mutation, the attempt would be to replace the incorrect or missing nucleotide, whereas for triplet-repeat expansions, the attempt would be to "patch over" the expansions.**

An additional potential gene-based approach is referred to as **gene replacement therapy**, in which the entire FRDA gene would be replaced by an unaffected gene. In still another approach, researchers are beginning to explore **pharmacological approaches** to addressing the FRDA genetic defects. In all these cases, the aim is **to manipulate the gene so that it expresses more frataxin protein**. All of these potential gene-based therapies would require a **delivery vehicle**, usually called a vector, that would take the therapeutic mechanism or material to all the right cells in the body. FARA is supporting a team in Australia, led by FARA grant recipients **Dr. Ian Alexander and Dr. Jane Fleming,** that is making excellent progress in developing such vectors for FRDA. Drs. Alexander and Fleming told the February conference participants that they are testing virus vectors in delivering FRDA genes to human and mouse model cell cultures and **will soon attempt delivery "in vivo" into a living mouse model – very important work being funded by FARA grants**.

Please Help FARA Receive $5,000.00 Matching Gift -- Please Donate by Valentine's Day!

An anonymous donor will match donations received before Valentine's Day, February 14, 2004, up to a total of $5,000.00. What a wonderful Valentine's gift to all of us. This matching gift will provide FARA an additional $10,000.00 to award a research grant to a scientist or team studying Friedreich's ataxia. Please give generously by using the enclosed pre-addressed envelope to mail in your tax-deductible contribution today!

Here's Leo… Once Again!

Krissa and Leo Lazaropoulos

For the fourth year in a row Leo has raised money for Friedreich's ataxia research. Leo and his family hosted a charity yard sale and donated their proceeds to FARA. They had about 2,500 items donated by friends and neighbors. "People give beautiful things," said Leo's mother, Krissa Lazaropoulos, noting donations were solicited through more than 1,000 notices to Leo's schoolmates. "Some people give a bag. Others donate a truckload."

According to an article in his local paper, the Courier-Post : "Leo says the toughest thing about having Friedreich's Ataxia is that he can't run like he used to. But like everything else, Leo makes up for his lost ability, especially when he's playing with his brothers Tasso, 12, and Michael, 10. "I can't catch them, so I throw stuff at them."

A big THANK YOU to Leo and his family for their support of Friedreich's ataxia research and their positive, uplifting spirit—Keep up the good work!

No one alone can solve the puzzle of Friedreich's ataxia, nor can we wait for someone else to solve this one for us. Acting alone, there is little we can do. Acting together, there is little we can NOT do! Please consider making a tax-deductible contribution to FARA today. Every dollar donated goes to support Friedreich's ataxia research.

Dr. Robert B. Wilson, MD, PhD

FARA's Featured FRDA Scientist

In early 1998 – before FARA was established – FARA's founders knew that, if the organization were to be effective, it must be devoted to supporting research that was judged to be meritorious by a review of the applicants' scientific peers. It became clear that FARA's Board of Directors would have to include a core of top-notch FRDA scientists that would comprise a Scientific Review Committee to lead the peer reviews, and that Committee would need a chairperson. Acting on advice from the National Institutes of Health (NIH), FARA's founders met with Dr. Robert Wilson of the University of Pennsylvania.

Dr. Wilson had already conducted important FRDA research, having done much of the work on yeast homologues that helped determine that iron played a critical role in the disorder. He agreed immediately to serve as a founding member of FARA's Board of Directors and as Chairman of its Scientific Review Committee. He also agreed that the first objective of the organization should be that of conducting the first international FRDA scientific conference. He began immediately preparing a conference grant application to NIH and on September 29, 1998 -- the day after FARA was officially established – FARA submitted the application to the NIH. That conference was held early in 1999, with Dr. Wilson serving as its Principal Investigator, Chairman and scientific organizer. He again prepared the grant application and served in the same capacities for the February 2003 conference. He co-authored the scientific report on the 2003 conference, to be available in "Neuromuscular Disorders" 2004 Jan;14(1):70-82; Seznec H, Wilson RB, Puccio H.

In the years between those two conferences and since, Dr. Wilson has led FARA's scientific endeavors. He has chaired the Scientific Review Committee's conduct of most of FARA's peer reviews, making the key recommendations involved in awarding about one million dollars in FARA research grants. He has also continued his own FRDA research, participated in the concerted effort to develop ataxia scales, and serves as the Principal Investigator responsible for preparing and conducting Phases II and III of the U.S. Idebenone clinical trial. In addition, Dr. Wilson somehow finds the time to teach at the University of Pennsylvania School of Medicine and fulfill clinical responsibilities at the University Hospital, while being a husband and father of two boys.

Dr. Wilson is Associate Professor of Pathology and Laboratory Medicine, Department of Pathology and Laboratory Medicine, University of Pennsylvania School of Medicine, and has served as Acting Director and Associate Director of that Department's Molecular Diagnosis Laboratory. He holds baccalaureate degrees in both Music and Biochemistry from Brown University, where he was an Honors Graduate in both disciplines, Phi Beta Kappa, Sigma Xi, and Magna Cum Laude. He earned his M.D. and PhD from the University of Pennsylvania. He has received a number of prestigious research and teaching awards and has published numerous articles, abstracts and lectures. He has received research grants from NIH, the National Science Foundation, Veterans Administration, MDA, NAF, the McCabe Fund, the American Cancer Society, and the University of Pennsylvania.

Dr. Rob Wilson has earned the respect and gratitude of FRDA families around the world. He is FARA's first Featured FRDA Scientist.

Seven U.S. Centers Open FRDA Scales Study

—To Provide Clinical Measures Needed for Drug Trials—

FRDA research "has entered the treatment era." Clinical drug trials are underway and it is critically important that ataxia scales be available to document the therapeutic effect of the drugs being tested. Without such clinical measures to demonstrate that a drug has beneficial effect on patients, the Food and Drug Administration (FDA) will not approve the drug, doctors will not be able to prescribe it for FRDA and it will not be covered by U.S. health insurance plans.

The current, multi-center study designed to put the required ataxia scales in final form is being led by **Dr. David Lynch** at the Children's Hospital of Philadelphia and University of Pennsylvania and also involves six other centers around the United States –Emory University, UCLA, and the Universities of Iowa, Minnesota, Mississippi, and Texas. In addition to Dr. Lynch in Philadelphia, the study's Investigators are **Dr. George (Chip) Wilmot** (Emory); **Dr. Susan Perlman** (UCLA); **Dr. Henry Paul**son (U. of Iowa); **Dr. Christopher Gomez** (U. of Minnesota); **Dr. S.H. (Sub) Subr**amony (U. of Mississippi); and **Dr.Tetsuo (Tee) Ashizawa** (U. of Texas).

Dr, David Lynch

FARA and MDA are cooperating to fund this multi-center study. FARA also funded the research Dr. Lynch conducted to build the preliminary data and structure of the study, as well as the two previous sessions at the National Institutes of Health (NIH) designed to test previously available ataxia scales for potential application to FRDA trials. The current study will not involve the administration of any drugs and will not require participants to alter their current practices regarding drugs and medications they might already be taking.

The clinical measures to be tested and refined in this study include the 9-hole pegboard test, quantitative speech measure, timed 25-foot walk (when possible), quantitative visual function measure, and quality of life measures. **The preliminary research funded to date by FARA and Seek A Miracle/MDA has demonstrated that these measures do correlate with markers of disease severity**. The multi-center study will determine the extent to which the measures are sensitive to changes resulting from disease progression and the drugs to be tested in clinical trials.

This large, multi-center effort and future clinical trials would be far more difficult, time-consuming and error-prone if researchers relied on hand-written data entries. **The FARA volunteers (see FARA IT Volunteers article) will insure that this study and subsequent FRDA clinical trials are "paperless trials" in which data is electronically entered, verified, transmitted and collated, and in which patient registries and databases are generated, secured, maintained and mined.**

FRDA clinical trials will not be successful without the clinical measures to be developed in this study. This study will be successful only with patient participation. **Treatments and a cure will be developed only if FRDA patients enroll and participate in this study. Please see the information on Patient Recruitment and contact the center nearest you to enroll as soon as possible. The sooner this study is completed, the sooner FRDA clinical trials can be successful.**

FARA *IT* Working Group--Volunteers are "IT"!

The leadership, courage, commitment and efforts of a team of highly capable and motivated volunteers, combined with the vision of FARA's President, is resulting in an Information Technology (IT) infrastructure that represents hundreds of thousands of dollars of in-kind services, software and equipment—freeing up more FARA donations to further research efforts and multiplying their impact many times over!

Bill Hartnett

The FARA Information Technology (IT) Working Group is composed primarily of lay professionals (not medical scientists) who have volunteered their time, educational and professional expertise to develop a long-awaited and revolutionary change in a critical aspect of FRDA research – especially in clinical trials. **The IT Working Group will insure that the multi-center ataxia scales study and subsequent FRDA clinical trials are "paperless trials"** in which data is electronically entered, verified, transmitted and collated, and in which patient registries and databases are generated, secured, maintained and mined through computer project design and engineering.

As FRDA research continues its welcome transition from basic science to clinical activity, **large amounts of data will be developed and collected in multiple centers around the world** on an increasing number of patients being administered a growing number of potential therapies. FRDA scientists have worked toward this transition for years and have long known that it will require automated data collection and handling. Information on participants in clinical studies and trials will need to be entered into securely protected patient registries and databases. Data from studies and trials at multiple centers will need to be collected and entered accurately and promptly. All such data will need to be "warehoused" and "mined" so as to instruct decisions about the conduct of trials and the effectiveness of treatments.

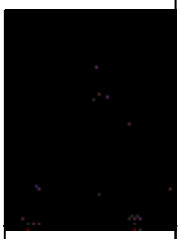
Alice Bearce, Sherri Stone, Bill Hartnett, Christine Ward, Jill Werner, and Margaret Ferrarone

The leading edge of this revolutionary FARA Information Technology (IT) Working Group effort in FRDA research is a team of **volunteers working hard with the critically important, multi-center, ataxia scales study being led by Dr. David Lynch at the Children's Hospital of Philadelphia and University of Pennsylvania and involving seven other centers around the United States**. This large, multi-center effort and future clinical drug trials for FRDA would be far more difficult, time-consuming and error-prone if they relied on conventional, hand-written data entries.

This project is led by **Bill Hartnett**, who is the newest Member of FARA's Board of Directors and an extremely generous donor, talented volunteer and leader. The medical-professional coordinator is **Jennifer Farmer**, genetic counselor and FRDA researcher at the University of Pennsylvania, assisted by her husband **Alan Farmer**. The other volunteers, from Electronic Data Systems (EDS), include **Margaret Ferrarone (a "FRDA mom"), Marianne Wilcox, Jill Werner, Christine Ward, Leo Bellew, Craig Dennstedt, John Young, Rich Dusse, Alice Bearse, Dawn Catanese, Shannon Bielaska, Sheri Stone, and Andrei Coler.** These EDS professionals are leading the project design and engineering and a growing group of volunteers contributing to the development and testing of the final application.

Volunteers (Cont'd from p.9)

Kerry Westfall is a remarkable young man who hasn't allowed Friedreich's ataxia to slow him down. He is a Microsoft database engineer who has been part of the core design and engineering team for this application. In addition to his intellectual contributions, he has secured **Microsoft's donation to FARA of the Microsoft SQL database and operating system software**.



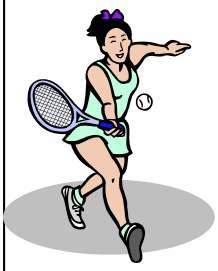
Leo Bellew

FARA is funding the three computer servers necessary for the project as well as the contract to establish the multi-center network, monitor the hardware and software, and provide support services for all users. "The novel and customized application the IT Working Group has been developing is a web-based infrastructure that supports communication between sites of a multi-center study, access to essential documents, and efficient data entry to a central database for rapid analysis and secure storage. This initial application has been designed so that it can be expanded to support future clinical studies at a national and international level. **This most generous contribution by the IT Working Group has tremendous value to FRDA researchers and will directly impact the pace and quality of clinical research.**" (Jennifer Farmer, Medical Professional Coordinator, University of Pennsylvania)

Craig Denstedt

As one researcher remarked—"we've been trying to accomplish this for years. " What a huge difference volunteers are making in advancing FRDA research!

Tennis Tourney Raises Research Dollars



BJ Acker-Hitta once again organized an athletic event in California to raise funds for FARA research. BJ is part of the Aerobic Team at Rolling Hills Club in Novato. She and her husband, Jean-Luc Hitta, have three beautiful daughters who keep them quite busy. BJ also serves on the FARA Board of Directors. Despite all these responsibilities, BJ has hosted two benefit Cardio Jams and, this year, she raised $21,000 for Friedreich's ataxia research through a Tennis Tournament for Hope and a Cure. To help or participate in BJ's events in the San Francisco area, please email fara@frda.org

BJ Acker-Hitta

Speaking of exercise…Ken Jacquin ran the New York City Marathon to raise funds for FARA. A record 34,729 participants made their way from the Verrazano-Narrows Bridge to Tavern on the Green, cheered on by more than 2 million onlookers who lined the streets of New York City's five boroughs. Ken's supporters made generous donations to FARA.

There are many other touching ways people raised funds for research recently—e.g., **in honor of a 50th Wedding Anniversary and donations in lieu of wedding gifts**. FARA appreciates ALL the generous support that has enabled the funding of more than $1,000,000.00 in Friedreich's ataxia research.

Share **YOUR** FARA fundraising story. Hopefully the fundraising stories featured in this issue will help inspire you to think of a creative way to help. Email news and photos of your event to fara@frda.org so we can pass along your success story. Also email any questions you might have about setting up a fundraiser.

TWO Clinical Trials in Progress— Patient Enrollment Information

FRDA ATAXIA SCALES STUDY — PATIENT RECRUITMENT

The clinical measures to be tested and refined in this study include patient participation in the 9-hole peg-board test, quantitative speech measure, timed 25-foot walk (when possible), quantitative visual function measure, and quality of life measures (see in-depth article, p. 8).

CONTACT A CENTER NEAR YOU FOR INFORMATION**:**

*Philadelphia, PA: U. of Pennsylvania/Children's Hospital of Philadelphia
Dr. David Lynch, (215)590-2242, lynch@pharm.med.upenn.edu; Jennifer Farmer, (215)614-0937, farmerj@uphs.upenn.edu

***Atlanta, GA**: Emory University– Sue Gronka, (404)778-3075, sgronka@emory.edu

***Los Angeles, CA**: UCLA – Lyndsay Elliott(310)794-1225, lyndsayelliott@aol.com

***Iowa City, Iowa**: U. of Iowa – Dr. Hank Paulson, henry-paulson@uiowa.edu

***Minneapolis, MN** U. of Minnesota – Jodi Lowary, (612)625-0656, jlowary@umphysicians.umn.edu

***Jackson, MS**: U. of Mississippi – Leigh Langford, (601)984-5500, llangford@neurology.umsmed.edu

***Galveston, TX**: U. of Texas – Penny Stanton, (409)747-4567, pstanton@utmb.edu

IDEBENONE CLINICAL TRIAL — PATIENT RECRUITMENT

The National Institutes of Health (NIH) Institute for Neurological Disorders and Stroke (NINDS) is currently recruiting FRDA patients for the Idebenone clinical trial. (See Antioxidant update for detailed explanation of trial). This study will determine the highest dose of the anti-oxidant, Idebenone, that can safely be given to patients with Friedrich's ataxia. Participants are divided into 3 patient groups based on age.

Children: ages 5 through 11
Adolescents: 12 through 17
Adults: 18 and older

Patients for all age groups are needed. Children (ages 5 through 11) are especially needed. In fact, the shortage of participants in this age group has slowed trial progress considerably.

The Details: Phase I is being conducted at the NIH Clinical Center located on the main NIH campus in Bethesda, Maryland, just outside Washington, DC. NIH will pay all expenses for travel, food and lodging for patient participants and a family member. Phase II will be planned for multiple locations.

For further information, please contact:
Ms. Amy Jewell
Email: jewella@ninds.nih.gov
Phone (301) 496-8969
Fax (301) 480-3365

FARA Remembers Grant R. Curtis

Founding Director, Vice President, Friend

All of us who knew him continue to be inspired by Grant R. Curtis of Atlanta, Georgia, and to grieve his loss. Grant was a founding Member of FARA's Board of Directors and served as the organization's Vice President. He was instrumental in establishing, shaping, energizing and operating FARA and was extremely generous with his time, talents, energy and donations in the support of FARA and ataxia research.

Grant Curtis was a truly exceptional and fine man. He was born in New Jersey on October 4, 1938. Following graduation from Princeton University and its Navy ROTC program in 1960, he served on aircraft carriers and destroyers and saw action in the Caribbean during the Cuban Missile Crisis. After his Navy service, Grant went to the Harvard Business School and launched his 30-year career with the Coca Cola Company in marketing, strategic planning, and management. His contributions at Coca Cola were global, including service in Atlanta, where he retired, Sydney, Tokyo, Brussels and -- his favorite -- Paris.

Always tireless and relentless in pursuit of a treatment for his son's early-onset, unidentified ataxia, Grant Curtis was a man of vision who took a comprehensive and passionate approach to supporting the research that provided hope for such treatment. Recognizing the importance of the U.S. Government's role in funding and guiding medical research, he was active with the National Institutes of Health and in the halls of Congress, encouraging increased support for the medical research community. Recognizing, too, that scientific breakthroughs in Friedreich's ataxia would lead to powerful insights into a considerable number of other, closely related disorders such as early-onset, unidentified ataxia, Grant devoted his endless energy, wisdom, insight, experience and generosity to helping FARA build support for research. He was successful in pursuing corporate donations to FARA. He was a sage voice in the effort to design for FARA an effective research grant application process and peer-review system. He helped inspire and lead the concerted effort to design effective ataxia measurement scales so essential in ataxia clinical trials and services. He helped set the course for our common effort and to keep us on track. We are forever indebted to him.

Grant was a person of real character and the highest integrity – a dedicated family man, a gifted and accomplished professional, a tremendous champion of medical research, and a genuinely good friend. Grant died of cancer on September 27, 2002. He is survived by his wife, Carol, his son, James, and his daughter, Sara. FARA and the ataxia community feel Grant's loss very deeply and personally. We join together in keeping Grant and his family in our hearts and prayers.

Annual Memorial Bro Golf Association Tournament

The Bro Golf Association is dedicated to promoting the spirit of camaraderie, competition, and fair play. Friends in the Association gather annually from around the country to play a memorial tournament in honor of a friend who died of Friedreich's ataxia several years ago — Jeff "Rosey" Rosenkranz. FARA would like to thank the members of the Bro Golf Association for all their hard work to advance this cause and for donating the proceeds to FARA. The 2002 golf tournament and auction brought in more than $10,000.00, and the 2003 event raised about $4,000, all of which will be dedicated promptly to Friedreich's ataxia research. Thank you all very much. To inquire about participating in the annual event, email fara@frda.org.

Small community in Orange County, California has raised $500,000

Orange County, California declared war on Friedreich's Ataxia in 1998 when Chelsea Lane was diagnosed with Friedreich's ataxia. The community took up the challenge of raising money to fund the necessary research to find a treatment – and one day a cure – for this disorder. People like Steve and Sandy Lane -- Chelsea's parents -- their friends, community, parish church and school, and local businesses and news media, were prepared to fight. For the past four years this amazing community has sponsored the "Walk for Hope and a Cure" which raises funds for FARA's Friedreich's ataxia research.

"Many of you may say, 'I don't know if I could do something like that.' Or you may be intimidated when you hear the amount of money we have raised. I say to you, please come forward and lend a helping hand. Every dollar raised puts another dollar in our account in finding a cure for Friedreich's Ataxia." (Sandy Lane, mother of Chelsea Lane)

Chelsea & Marines

Sponsors Brenda & Mike Carver (R.J. Noble), Donna Oldham (organizer extraordinaire)

You can help make the '04 Walk for Hope and A Cure even bigger. See notice below and try to help.

The Stars of the Show

Next Year's Walk Now Scheduled. Please Try to Help!

R.J. Noble Company

Walk for Hope and a Cure

5th Annual R.J. Noble Company / FARA Walk for Hope and a Cure

Please Join the Lane Family at St. Norbert's

Saturday, May 15, 2004 -Orange, California

"Our last walk raised over $150,000. We have raised a total of **half a million dollars** over the past four years. This would not be possible without the help of family, friends, and those parents of children afflicted with FRDA who have also stepped forward to help. It would also not be possible without the generous, unflagging support of our corporate sponsors

To become a volunteer, donate money, or for more information, please call the Orange County Chapter of FARA at 714/685-0096. All donations are tax deductible and every dollar donated goes to support Friedreich's ataxia research.

Bill and Paige Barnett embrace (above) as "Fiddler on the Roof" cast reacts joyfully to the receipt totals being held aloft as the curtain fell on the final performance. Donations for their three shows amounted to about $70,000.

Barnett Family Produces Second Blockbuster to fund FARA Research!

In Chapel Hill, NC, the United Church of Chapel Hill was packed to overflowing for three fund-raising performances of "Fiddler on the Roof", March 21-23, 2003. The FARA fundraiser featured a cast of 89 and a 21-piece orchestra. "Fiddler" was the second research-benefit production by the United Church Players and Senior High Youth. The group staged "Anything Goes" in 2001. Paige Barnett produced and directed the shows. Husband Bill, daughters Carrie and Joanna, and son Kevin are in the cast. Son Thomas is the soundboard manager. With the two productions, the Barnetts and community have raised $100,000 for Friedreich's ataxia research. Congratulations on a job well done! Bravo!!! Encore!!! Congratulations, also, to Thomas Barnett, a National Merit Scholarship Finalist, who was the recent recipient of a GlaxoSmithKline Opportunity Scholarship. Thomas is completing his first semester at Elon University, not allowing Friedreich's ataxia to slow him down. Congratulations and gratitude, also, to 6th-grader Joanna Barnett, who came to NIH in December 2003 to participate in the Idebenone clinical trial and presented FARA with a donation of $100 of her own money. Thank you so much, Barnett family. Applause!! Applause!!

A $5,000.00 Matching Gift to FARA…Please Help!

An anonymous donor has offered a Matching Gift to FARA. The donor will match donations received before Valentine's Day, February 14, 2004, up to a total of $5,000.00. What a wonderful Valentine's gift to all of us. This matching gift will provide FARA an additional $10,000.00 to award a research grant to a scientist or team studying Friedreich's ataxia. Please give generously by using the enclosed pre-addressed envelope to mail in your tax-deductible contribution today!

You've got the power to give your gift a greater impact!

PLEASE HELP FARA

Support research aimed at a treatment for Friedreich's Ataxia. Your donation promises a new legacy of scientific advancement and gives families genuine hope for the future. **NO overhead costs** --100% of your **tax-deductible** donation funds research. Donors receive this Update. Thank you.

Yes, I would like to help FARA with the enclosed tax-deductible donation in the amount of $_____.

Use enclosed envelope or mail to FARA, 2001 Jefferson Davis Hwy, Suite 209, Arlington, VA 22202

FRDA Protein Detectives Close In

February Conference Explores Protein's Function

The second session of the February conference was chaired by Dr. Grazia Isaya of the Mayo Clinic and targeted the frataxin protein. Presentations were made by Dr. Isaya, Dr. Helen Nichol of Stanford University, Dr. Anna Ramazzotti of the Catholic University of Louvain, Belgium, Ms. Heather O'Neill of the Mayo Clinic, and Dr. Viviana Seveso of the National Institute of Neurology, Milan.

Dr. Grazia Isaya

The intensive discussion of the frataxin protein continued in session IIIA, which was chaired by Dr. Massimo Pandolfo and was centered on yeast studies of FRDA cells. Presentations were made by Dr. Roland Lill of the University of Marburg, Germany, Dr. Jerry Kaplan and Dr. Opal Chen of the University of Utah, Dr. Paolo Arosio of the University of Brescia, Italy, Dr. Andrew Dancis of the University of Pennsylvania, and Dr. Michael Resnick of the National Institute of Environmental Health Sciences, NIH.

FRDA scientists know much about the frataxin protein. They know that when the FRDA gene is transcribed correctly, the gene's code results in the assembly of 210 amino acids in a particular order and that these amino acids "fold elegantly" into the frataxin protein that moves to perform its function at the walls of the cell's mitochondria. They know, too, that the frataxin protein plays an important role with iron. The key unanswered question concerns the protein's precise function with iron. Does the protein escort iron, maintaining the iron in its benign, plus 2 state (Fe+2), so that the iron does not give up an electron, transition to Fe+3 and contribute to the production of the free radicals that damage and kill cells? Or, does the frataxin protein not only maintain the iron in its benign state but actually participate along with other proteins in the assembly of the iron-sulfur clusters important in the mitochondria's production of energy? The scientists who presented their insights at the February conference are leading the way in determining the answer.

Dr. Roland Lill

In the meantime, other scientists are launching their own attempts, even before the protein function issue is resolved, to make progress toward a protein-based FRDA therapy. For example, FARA recently awarded a grant aimed in that direction to a Wake Forest University (WFU) team led by Dr. Mark Payne. The WFU team's extensive work on cancer and heart disease included research on proteins that are expressed in the cell's nucleus but function in the cell's mitochondria. The team developed a technique for synthesizing proteins when they are in short supply and then delivering them to mitochondria. The delivery device was a fusion protein that can be attached to the target protein, escort it across the blood-brain barrier into the cell and directly into the cell's mitochondria, where the target protein is left to perform its function. Knowing that the frataxin protein is expressed in the cell's nucleus, functions in mitochondria, and is in short supply in FRDA patients, the WFU team aims to synthesize frataxin and deliver it to mitochondria, first in cell cultures and then in FRDA mice. FARA is assisting the WFU team by funding this research and in obtaining FRDA mice for the project.

Bridget Registers Walkers

Walking to Seek A Miracle

Fifth Walk to Seek A Miracle in the Buffalo, New York Area

In September, Terry and Marilyn Downing held their fifth Walk to Seek A Miracle in the Buffalo, New York area and raised over $55,000. Their daughter, Bridget, has Friedreich's Ataxia. The events included a DJ, a rest stop with games and prizes, Chinese and silent auctions, hot dogs, pizza and subs for all.

The Buffalo Walk was dedicated to the memory of Mary Keyes, a twenty-year-old with Friedreich's ataxia when she passed away in January 2003. A tree was planted along the Walk route in memory of Mary and her father, who was instrumental in planning earlier walks.

Marilyn is FARA's Secretary while Terry is FARA's Treasurer. They have been instrumental in all FARA activities and in establishing the FARA/Seek A Miracle collaboration that brings the resources of both organizations together to fund Friedreich's ataxia research.

COOKING FOR A CAUSE

<u>Please Purchase A Cookbook</u> to Raise Funds for FRDA Research!!

Nick and Aubrey Olson's aunt, Julie Karjalahti, has compiled a cookbook called Cooking for a Cause. All the proceeds from the sale of the book go to support Friedreich's Ataxia research. THE PROCEEDS WILL KEEP RESEARCH MOVING AND ACCELERATE THE PROGRESS FOR A TREATMENT AND CURE FOR FRIEDREICH'S ATAXIA.
Ordering info:
Price: $14 (includes $4 for shipping and handling). Check made payable either to FARA or NAF. Mail check to:
Cooking For A Cause Cookbook
Julie Karjalahti
12361 Hillsboro Ave. S.
Savage, MN 55378

A HUGE THANK YOU TO JULIE FOR ALL OF HER HARD WORK AND DEDICATION IN PREPARING THIS COOKBOOK AND TO ALL OF YOU WHO CONTRIBUTED RECIPES.

THE RESEARCHERS' CORNER

REQUEST FOR GRANT APPLICATIONS

FARA will accept applications at any time from US & International Researchers

Research Grants. FARA will support research with grants provided by the organization itself and by assisting in seeking grants from other public, non-profit organizations that are exempt from federal income taxes under section 501(a) as organizations described in section 501(c)(3). **FARA** pursues a multi-tiered approach. **FARA** provides smaller, shorter-term "seed" grants to attract new research investigators and assist existing investigators by supporting the early phases of their research (including funds for equipment and post-doctoral fellowships). These "seed" grants will permit investigators to collect preliminary data and test initial hypotheses. In some cases, significant findings might result, or additional investigators might be attracted to the field. In other cases, this preliminary research will better prepare the investigators to submit successful applications for larger, longer-term grants from FARA or other, larger grant-making institutions such as the NIH.

Workshop Grants. FARA supports scientific workshops designed to advance the research into treatments and cures for Friedreich's ataxia and the related sporadic ataxias. These workshops will be of two types - full-scale workshops and project-specific workshops.

Project-Specific Workshops - **FARA** intends also to support workshops arranged on shorter notice for small groups of investigators when face-to-face collaboration on a specific approach or insight promises a significant advance in **FARA**'s mission. In such cases, the workshop site will be selected so as to optimize collaboration and minimize costs.

Application Submission. Applications are to be submitted in electronic or paper form to **FARA** at the addresses below, from which they will be distributed to the scientific members of **FARA**'s Board of Directors and the Scientific Advisory Committee appointed to serve in the peer-review process. The Scientific Advisory Committee will request additional information from the P.I. when necessary.

Friedreich's Ataxia Research Alliance (FARA)
c/o Ronald J. Bartek, President
2001 Jefferson Davis Highway; Suite 209
Arlington, Virginia 22202 USA
Electronic application submissions will be accepted at fara@frda.org

Recent Letters from FRDA researchers to FARA's President

Dear Ron: "It is clear to me that this entire endeavor will succeed. I can assure you no single body of neurologists has ever approached its task of assessing patients for study purposes with this concerted and earnest an effort." "I am overwhelmed by the foresight and initiative shown by FARA in pushing forward with this project. I cannot thank you enough, both for the financial support of FARA and for pulling together the group of researchers involved and lighting the fire under them." (Comments regarding the multi-center ataxia scales study, the IT Working Group and the Computerized Paperless Trial)

Dear Ron**:** "I long ago realized the complex nature of the task at hand-to treat and cure ataxias. Particularly for a disorder like ataxia, progress was only going to be made with the help of many good and talented individuals. The ataxia field is now rich with such people. The ball is now rolling. We must now keep it up."

Dear Ron**:** "FARA has done a lot for FA patients and researchers, and continues to impress me as the strongest advocate of FA research."

Dear Ron: "Thank you all again for organizing such a wonderful FARA meeting in Washington. It provided the platform for many useful scientific collaborations and it was a pleasure to meet everyone." "Thanks again for organizing this conference. It was a great success. We also felt your warmth in treating us all." " I extremely enjoyed the conference and all of us very much appreciated your enormous efforts on this great event."

Researchers' Corner (Cont'd from p. 17)

FARA Research Grants Pass Million Dollar Mark

FARA was established in September of 1998 and gave its first research grant in April of 1999. As a result of the generosity of donors, the tireless commitment of the people conducting fundraisers, co-funding by Seek A Miracle/MDA, and the increasing tempo of FRDA scientific progress, **FARA has awarded research grants that now total <u>more than a million dollars</u>**. The following is a brief summary of FARA-supported research:

- FRDA Scientific Conference at NIH (80 scientists), 1999
- FRDA Scientific Conference at NIH (100 scientists), 2003
- Clinical Trial Planning (20 scientists)
- Ataxia Scales Development (9 scientists, 14 patients), 2000
- Ataxia Scales Development (9 scientists, 14 patients), 2001
- Ataxia Symposium Support (A. Koeppen – Chairman)
- Ataxia Conference (supported two scientific participants)
- Alexander, Ian — Gene Therapy Vectors
- Anderson, John — Quantifiable Physiological Measures
- Ashizawa, Tetsuo — Multi-Center Clinical Measures Development
- Bidichandani, Sanjay — Molecular Biology of GAA Triplet
- Brown, Michael — Mitochondrial Modifiers of FRDA*
- Delatycki, Martin — Ataxia Scales and Prep for Clinical Trial*
- Fitzmaurice, Paul — Brain Glutathione and FRDA*
- Fleming, Jane — Gene Therapy Vectors*
- Gomez, Christoper — Multi-Center Clinical Measures Development
- Grabczyk, Edward — Transcription thru Triplets
- Grabczyk, Edward — Comparative Analysis of Acadian & Non-Acadian FRDA*
- Grabczyk, Edward — Continuation Grant
- Ioannou, Panos — Drug Screening and FRDA Mice
- Lynch, David — Ataxia Scales
- Lynch, David — Ataxia Scales & Prep of Multi-Center Effort*
- Murphy, Michael — Mitochondria-Targeted Antioxidants*
- Pastore, Annalisa — Frataxin Protein Function*
- Payne, Mark — Frataxin Protein Synthesis and Delivery*
- Perlman, Susan — Multi-Center Clinical Measures Development
- Pook, Mark — Frataxin Expression in Mice W/Different Repeat Lengths
- Puccio, Helene — Therapeutic Trials on FRDA Mice*
- Richardson, Des — Mitochondrial Iron Chelation in FRDA Mouse Model
- Rustin, Pierre — Construction of Human FRDA Cell Model
- Rustin, Pierre — Screening Drugs in FRDA Cell Culture*
- Subramony, S.H. — Multi-Center Clinical Measures Development
- Tan, Guolin — Use of Microarrays in FRDA Fibroblasts*
- Wells, Robert — DNA Triplexes in FRDA
- Wells, Robert — DNA Triplexes in FRDA-continued*
- Wilmot, Chip — Multi-Center Clinical Measures Development

*Jointly funded by FARA and SAM/MDA

FARA uses the NIH model of peer review. FARA's Scientific Review Committee (Drs. Rob Wilson, Bronya Keats and Massimo Pandolfo) leads the peer review process and reaches out to additional scientists for particular expertise, especially to members of FARA's Scientific Advisory Board (Drs. Henry Paulson, Robert Wells, and Arnie Koeppen). Based on the peer review, the Scientific Review Committee makes a recommendation to FARA's Board of Directors. The Board votes and grants are awarded if approved.

Availability of FRDA Cell Lines

The Coriell Cell Repositories provide essential research reagents to the scientific community by establishing, maintaining, and distributing cell cultures and DNA derived from the cell cultures. These collections are supported by funds from the National Institutes of Health (NIH) and several foundations. Coriell maintains and provides lymphoblast and fibroblast samples obtained from FRDA patients. These samples are reasonably priced and used by scientists around the world studying FRDA. To view the collection or place an order, visit the Coriell website at http://locus.umdnj.edu/

Internaf-Pro Invitation to health professionals
International Network of Ataxia Friends

Internaf, an internet listserv supporting the ataxia community, is very privileged to have noted researchers in the field of hereditary ataxia, along with many other medical professionals, who participate via an anonymous invitation-only list which runs in tandem to the main Internaf list which serves patients and families coping with "an ataxia." If you are a health professional with an interest in ataxia and would like further details or an invitation to join, please email internaf-pro-owner@yahoogroups.com

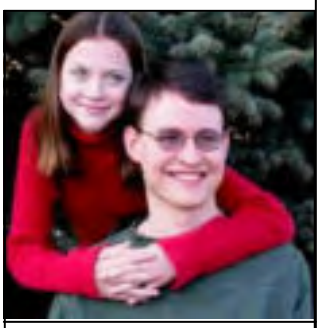
Jumping for a Cure

a Charity Hunter Jumper Show

Horseshow managers Jon Knight and Trisha Hussey sponsored this charity event to fund FARA research in honor of their niece and nephew, Allie and Aaron Kittel

Jumping for a Cure was held October 11, 2003 at Alta Hills Farm in Sandy, Utah. Trisha Hussey and Jon Knight, aunt and uncle to Aaron and Allie Kittel, organized and ran the fundraiser horseshow. Aaron (26), Allie (11) and their mother, Sue Kittel, drove to the event from their home in Loveland, Colorado. It was a perfect autumn day, and so breathtakingly beautiful to be in the foothills of the great Rocky Mountains. The day began with jumpers competing for prizes in different classes, lunch was sold at Blaine's Ringside Cafe (grandfather to Aaron and Allie), and the culminating event -- a fun raffle for donated prizes, including an original watercolor painted by Allie.

Horsing around for research!



Allie & Aaron Kittel

Hats off to Alta Hills Farm in Sandy, Utah! Thank you very much from the FARA research community & patient family community.

The Walk Before the Ride! Allie and Schoolmates Raise $4,821.40 for FRDA Research!

Allie Kittel is shy when it comes to talking about her disease, Friedreich's ataxia, but that didn't stop her from planning a Walk-a-Thon in her hometown to raise money for research and a cure. Both she and her 26-year- old brother Aaron, have Friedreich's ataxia. It seemed natural to ask the student council of her elementary school to sponsor the walk, so Allie (then a 5th grader) wrote a letter asking for their help. They quickly embraced the idea and planned a walk to take place before school ended that spring. All of the students at Centennial, over 300, where invited to gather pledges and walk the perimeter of the building during two recess periods. Following a rainy week, the weather improved and the Colorado sun did shine down on Allie's walk, as seen in this photo. The money was raised, and over 300 new families in Northern Colorado are now better informed about this rare disease.

A Fabulous Four in a Row for the Ferrarone Family

A Family, Friends and Community Go the Extra Miles for FRDA Research

Sara Ferrarone and friends

Walkers to Seek A Miracle

For the fourth consecutive year, the **Ferrarone family** organized a Walk to Seek A Miracle to raise money to support Friedreich's ataxia research. Their most recent walk, in Rochester, NY, was their most successful to date — **raising $100,000** to support FRDA research through Seek A Miracle/MDA. The check for that amount was presented during the Jerry Lewis MDA Telethon by **Margaret Ferrarone**, mother of **Sara and Laura**, and FARA Board Member **Bill Hartnett**. Our congratulations and deep gratitude to the **Ferrarone family** and their generous supporters and donors.

Nice fish, Brent! Great idea! Thank you!

Brent Moore's 2nd Annual Bass Tourney

Having developed a way to combine his life-long passion for fishing with his desire to help FARA support research, **Brent Moore**, organized his second annual Friedreich's Ataxia Bass Tournament on a warm sunny day in Cambridge, Ontario, Canada. Brent and his dear friend Mary both have Friedreich's ataxia, but that didn't stop them from parking their wheelchairs, climbing into their boat and leading the charge looking for the best fishing holes. Judging from the size of those two large-mouth bass, they must have found them. Brent's tourney netted several thousand dollars for FARA research. Tight lines landing good research. Thank you, Brent!

Thanks, Haley Sims!

"My name is **Haley Sims**. I recently sent a donation to you from my Brownie troop. After that I had a birthday party to celebrate my 9th birthday. I decided that instead of presents I would ask all the guests to bring a contribution for FARA. My neighbor **Janet** has Friedreich's and she is the person who taught me about the disease and about FARA. I hope this money helps to find a cure for this disease. Maybe it won't happen soon, but maybe someday we will find a cure. Maybe one day we will just be able to go to the doctor and get rid of the disease just like that. Please accept this donation from me and my friends. Yours truly, **Haley Sims**"

Haley Sims

Haley's Birthday Party

Mary, Sam, Alex

One Enchanted Evening

Mary Caruso, mother of two teenage daughters with Friedreich's ataxia, **Sam and Alex**, is also a Member of FARA's Board of Directors. She has organized a variety of successful fundraisers to support FRDA research. Most recently, she hosted **One Enchanted Evening - a Dinner Dance and Silent Auction** in North Branford, CT. As a single mom raising two daughters with physical challenges, Mary says she's making the best of what can present heartbreaking situations. According to an article in her hometown paper, Mary stated, "I think I just finally made a decision one night that I was going to be optimistic and look this in the eye and deal with this the best I can and teach my daughters that everybody is dealt something in life, and this is what we're dealt and we're going to deal with this as best we can."

The Heart and Friedreich's Ataxia - A Beginner's Primer and Introduction

According to the National Institutes of Health, "Most people with Friedreich's ataxia die in early adulthood if there is significant heart disease, the most common cause of death."
The FARA website now has a Beginner's Primer and Introduction to FRDA heart conditions **to assist families** trying to understand the science and the treatments available**. It is also helpful to medical professionals**, especially if they have not examined patients with Friedreich's ataxia. The Primer draws from many medical resources, as well as a patient family's perspective. **Visit the FARA website at http://www.frda.org/education/beginners-primer.shtml** to learn more about:
What is Hypertrophic cardiomyopathy (HCM) or left ventricular hypertrophy?
What are Arrhythmias? Types of Arrhythmias? Common Treatments for Arrhythmias?
Anti-arrhythmic medications
Anti-coagulant (anti-clotting) medications
Electronic devices
Cardiac pacemakers
Implantable cardioverter-defibrillators (ICDs)
Catherization
Chart with Types of Medications/How it Works/Examples/Generic (Brand Name)
An Appointment with the Cardiologist - Physical Exam
An Appointment with the Cardiologist - Diagnostic Testing (ECG/Echo/Holter/Event Monitors)
Preparing for the Cardiology Appointment
Suggested Questions to Ask the Cardiologist
A Trip to the Emergency Room!
Warning Signs and Tips
Genetically Speaking, Why does FRDA damage the Heart?
Cardiac Function - Different Rates of Progression
Variations of cardiac involvement - Research Abstracts
Idebenone or CoQ10/Vitamin E - Helpful in Controlling Cardiac Hypertrophy?
Participation in the Idebenone Clinical Trial in the US
Dictionary of Heart Definitions and Terms

WHAT THE NEWLY DIAGNOSED REALLY WANT TO KNOW

Most patients and families had never heard the words "Friedreich's ataxia" until a loved one received the diagnosis. Many families enter into a very confusing and emotionally draining time, coupled with an intense desire to learn as much as possible about what to expect and what to do. **Patients, families and their medical providers now have understandable and helpful information to address their concerns**. Written by a patient family in collaboration with the scientific and medical community, the topics include:
Receiving the Diagnosis/Telling Your Child
Could My Other Children Have Friedreich's ataxia ?
What do the two numbers on the FRDA genetic test mean?
Genetically speaking, what happens in the body due to FRDA?
School - Your Child's Education
Telling Your Child's Classmates
Medical Care for Your Child
Coping - Where can I turn?
"A Message to the Newly Diagnosed" can be found on the FARA website at http://www.frda.org/education/message.htm.

Friedreich's Ataxia Parents' Group - FAPG

Are you are a parent coping with a child diagnosed with childhood onset ataxia? ***This forum is a MUST!*** FAPG has remedied the isolation and loneliness many parents feel dealing with the challenges of raising children with a rare degenerative disease. The forum is a closed email group with over 200 subscribers. Discussions revolve around coping, research, parenting issues, accessibility issues, education, employment, and medical care. To join, visit http://www.fortnet.org/fapg or email Sue Kittel at kittel@webaccess.net

FARA and Seek A Miracle/MDA Collaboration

FARA and Seek A Miracle/MDA (SAM/MDA) have established an arrangement whereby they draw from the resources of both organizations to award peer-reviewed grants to FRDA scientists.

WHY? "Two pots of money are better than one!" More research funds available through FARA and SAM/MDA mean more and larger grants for promising scientific research.

WHEN? Researchers can apply for a grant at ANY time—not just several times per year as with most other non-profit organizations. When researchers are ready to submit a grant application to FARA, they are not tied down to waiting for the opening of a "Request for Applications" period. The FARA grant mechanism accelerates the funding of qualified grants.

Time is valuable to patients fighting the battle of progression and to researchers who are ready to proceed with promising avenues of research.

For those who donate to MDA, please consider making your donations payable to "Seek A Miracle/MDA," so your donations go to fund FRDA research in this collaborative way.

RESEARCHERS - a summary of the process:

FARA receives grant applications at any time during the year from FRDA scientists around the world and submits them to FARA's Scientific Review Committee for peer review. If the scientific peer review results in a favorable recommendation, that recommendation is submitted to FARA's Board of Directors, which includes the three scientists serving on the Scientific Review Committee and ten lay Members.

If FARA's Board of Directors votes to award the grant, it can request that SAM/MDA provide a portion of the funds required. In most such cases, SAM/MDA has agreed and has provided up to half the total funds for the grant. FARA's Board of Directors includes the founders of SAM/MDA, who were instrumental in concluding and implementing this arrangement that significantly increases the resources being devoted to promising FRDA research around the world. For further information contact Ron Bartek at fara@frda.org

To seek our miracle, FARA and SAM/MDA are working together to make a difference more quickly.

International Network of Ataxia Friends

Ataxia patients of all ages & Family - Internet mailing list

Ataxia patients and their families are invited to join an internet listserv that provides support and serves as an information exchange vehicle. Subscribers help each other by asking questions, making comments and providing answers on how to make life with ataxia easier. There are currently over 400 subscribers from more than 40 countries worldwide. Subscriptions to INTERNAF are free and the list is unmoderated.

To subscribe to INTERNAF, send an email to internaf-subscribe@yahoogroups.com

United Way -Another "Way" to Help!

The United Way provides a way for charities to obtain funds from individuals contributing one-time donations or a portion of their monthly paycheck through payroll deduction. Most organizations listed in United Way campaigns are based locally. However, most United Way Chapters allow individuals to write in the charity of their choice. FARA's 501(c)(3) nonprofit status and participation in United Way's Combined Federal Campaign (#7970) has **qualified FARA for the local United Way write-in option**. *Ask your company or community's United Way chairperson if there is a write-in option. Your coworkers may also want to donate to a cause dealing with someone they personally know. If you have questions, contact us at* ***fara@frda.org*** One very good-natured, FARA/United Way donor approached FARA President Ron Bartek at a conference and said… *I hope you appreciate the monthly contribution I give to FARA through my payroll deduction to United Way!* It was nice to meet this talented professional who, with many others, gives a substantial donation to FARA via United Way. 100% of these payroll deductions goes to FRDA research!

Combined Federal Campaign (CFC): Federal employees can contribute to FARA through the CFC at their workplace. Each year, Federal employees and military personnel raise millions of dollars that benefit non-profit charities. *"I have designated FARA for my Combined Federal Campaign contribution in the amount of $100 per month. The main reason I chose your organization was that I noted that 100 per cent of my donation goes towards research. Hope this helps and good luck."* (an anonymous CFC Donor)

To designate FARA, enter the four digit code 7970 on your pledge card.

YOUR PERSONAL CHECK or CREDIT CARD (NEW!!!!)

Personal checks: "*Today I turned 50. The only wish I have is to help find a cure for my son with Friedreich's ataxia. As a parent, I see the time ticking away as his physical capabilities slip away. Please find enclosed a check for $1,000. A cure would be the best present my family could ever receive. Thank you so much for all you do." (anonymous FARA donor)* Mail checks to the FARA address on page 1.

Credit Card Donations: NEW!! Go to the FARA homepage (www.frda.org); at the very bottom, click on fundraising; at the bottom of the fundraising page, click on the button that says "Donate Now Through Network for Good"; follow the easy instructions for submitting a credit card donation online. NEW!!!

Is There a Foundation in Your Life?

FARA has received a number of significant donations from charitable foundations around the country with which FARA supporters have established contact. Many companies, churches and individual trusts have charitable foundations willing to support worthy causes like ours. Most recently, FARA received very generous donations from the Charles See Foundation of Bellevue, WA; The Microsoft Corporation, and a church-based foundation in Virginia. FARA has also received a generous donations from the Ted Turner Foundation. If there is a charitable foundation in your life, you might find that the foundation would be willing to support your worthy cause — Friedreich's ataxia research.

The Corporate Business Connection -- Matching Gift Programs

If your employer offers a Matching Gift Program, your contribution to FARA could be doubled or even tripled! Because each employer has different requirements, please take a moment to contact your personnel office for your company's specific matching gift form and information. Complete your company's matching gift form and send it with your gift to FARA. Thanks to the many donors who have doubled or tripled their donation to FRDA research with their company's matching gift.

ANTIOXIDANT UPDATE - CLINICAL TRIALS

Conference Assembles Trial Scientists and Pharmaceutical Companies

FRDA scientists are increasingly impressed with the potential for antioxidant therapies. Antioxidants -- some natural and some synthetic -- are compounds that bind up free radicals and reduce the oxidative stress that kills cells. **Some of the best known antioxidants of interest in FRDA are Idebenone, Coenzyme Q10 (CoQ10), Vitamin E, Selenium, Alpha Lipoic Acid, N-Acetyl-L-Cysteine (NAC), green tea, and blueberries**. The FRDA community's interest in Idebenone sprang largely from FARA's 1999 conference, where Dr. Pierre Rustin briefed the group on the preliminary results of his open trial giving Idebenone to a small number of patients. At this year's FARA conference, a number of scientists presented their insights from open antioxidant trials as well as double-blind, placebo-controlled trials they have completed. Other participants presented **the plan for the large, double-blind, placebo-controlled clinical trial of Idebenone in the United States that FARA has helped initiate**.

The February conference session on Clinical Trials was chaired by **Dr. Rob Wilson** of the University of Pennsylvania. Scientists who presented results from their clinical trials included **Dr. Massimo Pandolfo** of the Erasmus Hospital, Brussels; **Dr. Alexandra Durr** of INSERM, Paris; **Dr. Alessandra Solari** of the Besta National Neurological Institute, Milan; **Dr. Paul Taylor then** of NINDS/NIH; **Dr. Rafael Lodi** of the University of Bologna; **Dr. Ludger Schols** and Dr. **Thorsten Schulte** of the Ruhr University, Bochum, Germany.

Drug Trials Mark "Treatment Era"

The Idebenone trials thus far have used a very small dose, usually 5 milligrams per kilogram of body weight (5 mg/kg). They have demonstrated that Idebenone does seem to reduce cardiac hypertrophy significantly (5%-20 %). At these low doses, however, there has been no convincing evidence of any benefit in terms of the neurological symptoms – the ataxia. The purpose of this large U.S. trial is to prove safety and efficacy of Idebenone in FRDA. The **plan is to administer Idebenone at a high enough dose to explore benefit not only in the cardiac symptoms but in neurological symptoms as well**. Also in this trial, patients will be observed to determine if the changes observed in the heart – in cardiac wall thickness, for example – are actually beneficial to the patient. That is, are such changes accompanied by indications of improved heart function.

Phase I of the trial is being conducted at the NIH with the purpose of establishing the maximum tolerated dose. It has been led by **Dr. Kenneth Fischbeck and Dr. Paul Taylor**. Dr. Taylor recently moved to the University of Pennsylvania where he is continuing his FRDA work and his role in the NIH Idebenone trial was taken up by **Dr. Nick Di Prospero**. Twenty-five adults (18 yrs. and older) completed the Phase Ia study and tolerated doses much higher than in previous trials – 75 mg/kg. Adolescents (12-17 yrs.) and children (5-11 yrs) began Phase Ia receiving 2.5 mg/kg followed by gradually increased doses. As of this writing, **three additional adolescents are needed to complete phase Ia and eight more children are needed**. This portion of Phase I has been slowed by the fact that there are fewer children who have enrolled in the trial and they have experienced some scheduling problems. We would urge all of you, regardless of where you live, to **consider participating in this trial.** Even though the most urgent need now is for children, Phases II and III of the trial will require large numbers of patients in all age groups. In Phase I, the NIH will pay expenses for participants from outside the U.S. as well. **SEE PAGE 11 FOR PATIENT RECRUITMENT INFORMATION.**

Idebenone is not the only antioxidant in clinical trial. In a four-year pilot study in London, one team of researchers led by **Dr. Anthony Schapira** and **Dr. Mark Cooper** has administered **CoQ10 and Vitamin E** and monitored impact on heart, skeletal muscle and ataxia scales. The preliminary results of the study indicated a **substantial increase in energy metabolism in both cardiac and skeletal muscle**. There was **no worsening among these patients in ataxia scale scores or cardiac hypertrophy**. **Cardiac fractional shortening increased significantly**.

Mitoquinone: Currently in development and impressively briefed at the conference is MitoQ (mitoquinone). The New Zealand scientists developing MitoQ are attempting **to target antioxidants specifically to the mitochondria, which is where FRDA's oxidative stress originates**. These scientists, led by **Dr. Michael Murphy and Dr. Robin Smith**, are trying to concentrate the antioxidant -- in this case a quinone very similar to CoQ10 -- in the mitochondria by attaching it to an element whose electrical charge pulls it through the membranes of the mitochondria. **Early experiments in healthy mice seem to indicate that the technique is effective in getting the antioxidant to mitochondria in the heart, skeletal muscle, liver and brain**. The current MitoQ effort, **under a FARA grant**, is **to test the compound in cell cultures and FRDA mouse models in England with hopes of trying it in human subjects in 2004**. The same scientists are exploring additional antioxidants targeted to the mitochondria in the same way. For example, they are investigating the effectiveness of directing Vitamin E to the mitochondria in a form they call **MitoVit E**. It was evident at the conference that this new approach of targeting antioxidants to the mitochondria is very promising and we are eager to see the results as the project moves through animal studies and then to human trials. **In the words of Dr. Murphy, "While MitoQ, and other derivatives of Coenzyme Q, look promising as potential therapies, these molecules are just one possible type of antioxidant. Therefore, in work funded by the Friedreich's Ataxia Research Alliance (FARA) we are also testing different types of mitochondria-targeted antioxidants which may (or may not) turn out to be more effective than MitoQ**. **Our hope is that molecules such as MitoQ may help slow the progression of Friedreich's Ataxia." FARA is assisting the MitoQ team in coordinating its efforts with NIH, FDA and U.S. scientists so as to accelerate progress for this and other promising compounds.**

Caution: FRDA families have been fortunate to be able to obtain Idebenone on the internet and at pharmacies in some countries, while **MitoQ** has not been formulated and **is not yet available for consumer purchase.**

WHAT IS BOCCE BALL?

A great way to raise research dollars for Friedreich's ataxia! Once again, **Lisa Carmassi**, a friend of FRDA Family- **Cindy and Bruce Olson**, hosted a bocce ball tournament for FARA. According to **BJ Acker-Hitta** who attended the event… "Once again Lisa did a fabulous job and everybody had a ball." "Bocce originated in Italy and is one of the oldest of all lawn bowling games. It is now gaining popularity in the United States." (from http://www.centralconnector.com/GAMES/bocce.html)

Enthusiastic core of "Team Laura"

"TEAM LAURA"

The "MDA Great Walk" was held at Forest Park in Springfield, MA. The local chapter considered the event a great success, and a big reason was the support for Laura. "TEAM LAURA" was by far the biggest team in the walk! The support for Laura had a really big impact on her and she really appreciated it. It made her feel good to know that in some manner she was doing something positive to help her situation and that of others. More than 150 people participated in the walkathon event. All of the donations raised by TEAM LAURA will go specifically to helping support Friedreich's ataxia research through Seek A Miracle/MDA.

Letter-Writing Campaign

One family raised over $10,000 for research in a single letter-writing campaign. This is the easiest way to raise funds and can be extremely successful. This enables you to inform people -- family, friends, colleagues, and community about Friedreich's ataxia. You might consider including on your mailing list special sets of people such as your high school or college classmates, the families of classmates of students with Friedreich's ataxia, and other members of civic organizations to which you belong. FARA will gladly provide you copies of letters other people have used successfully or you can see samples at http://www.fortnet.org/fapg/sample.htm or email FARA at fara@frda.org

So what is Mitoquinone (MitoQ) and why is it relevant to Friedreich's ataxia?

An article by Dr. Martin Delatycki
Director, Bruce Lefroy Centre for Genetic Health Research
Clinical Geneticist, Murdoch Childrens Research Institute
Royal Children's Hospital, Australia

Mitoquinone (MitoQ) is a drug that was developed in New Zealand by **Mike Murphy** and **Rob Smith** in Dunedin. It is **an antioxidant that is linked to a chemical that makes the antioxidant localize to mitochondria**.

In Friedreich's ataxia there is damage to mitochondria that is due at least in part to the build up of free radicals. Free radicals are tiny particles that are exceedingly damaging to the cells in the body, and in particular to structures within the cells. Mitochondria are one such structure in the cells. They can be thought of as batteries that provide cells with energy.

Dr. Martin Delatycki in Sydney w/ son Tommy

Antioxidants are drugs which detoxify free radicals and therefore protect cells from damage. Antioxidants that you may have heard of include Coenzyme Q10 and Idebenone. Mitoquinone is the antioxidant Coenzyme Q10 linked to a particular chemical that ensures that the Mitoquinone is concentrated about 1,000 times in mitochondria compared to other antioxidants such as Coenzyme Q10. It is known that Mitoquinone reaches the heart and the central nervous system, both sites that are affected in Friedreich's ataxia. Tests in cells from people with Friedreich's ataxia show that the Mitoquinone protects those cells from damage far better than other antioxidants.

So does Mitoquinone help people with Friedreich's ataxia? The answer is simply that without properly testing it on people with Friedreich's ataxia we cannot know the answer. It is for this reason that much effort is being made to ensure that the studies to test Mitoquinone will give the absolute best chance of detecting benefit (or harm, for that matter) in those with Friedreich's ataxia. Studies to find the best tests to look at how people with Friedreich's ataxia are affected, and therefore whether drugs are beneficial or not, are being examined. We are very hopeful, therefore, that if Mitoquinone does benefit people with Friedreich's ataxia that we will be able to detect this.

It is important to be clear that whilst we are hopeful that Mitoquinone will be beneficial in Friedreich's ataxia, we cannot be certain about this. It is imperative that studies are done in the best possible way so that everyone can know as soon as possible whether treatments are beneficial or not. If they are beneficial then the next task is to ensure that people with Friedreich's ataxia have access to the treatment. If it is not beneficial then we can all move on to looking at other treatments.

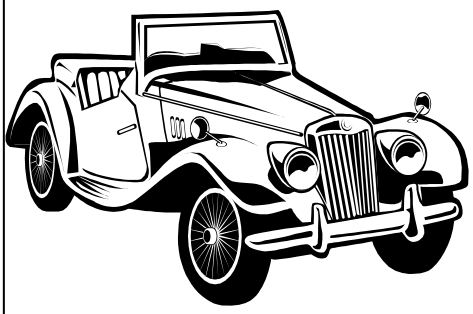
Editor's note: FARA is working closely with Dr. Delatycki, the developers of MitoQ, the Friedreich's ataxia research support organization in Australia, the NIH, and other elements of the Friedreich's ataxia research community to expedite the pre-clinical and clinical studies of this promising compound. FARA is also supporting the further development of MitoQ with a research grant to Dr. Mike Murphy, one of the principal scientific developers. For additional information, **see the Antioxidant Update on pages 24,25**.

Cherry Hill, New Jersey – Walk to Seek a Miracle

Rochelle (far right) and Samantha (front row, center) at the starting line.

Rochelle and Marty Litke's daughter, Samantha, has Friedreich's ataxia. The Litke response to that diagnosis was to rally other families and a host of supporters to work with the Muscular Dystrophy Association (MDA) to start Seek a Miracle (SAM)/MDA to help fund Friedreich's ataxia research. Since that time, SAM and FARA have established a collaborative relationship that allows the two organizations to co-fund research grant awards for research found meritorious by FARA's scientific peer-review process (See "FARA, and SAM/MDA Collaboration" p. 22). To increase the funds available for that purpose, the Litke family, once again, rallied friends, family and the community for a recent walkathon that successfully lifted spirits and raised additional money for research.

Like Cars? Come to the 2004 Charity Car Show for FARA – Marietta, Georgia



Dianne Thigpen is working with the annual Charity Car Show in North Georgia so that a portion of the proceeds go to FARA to support FRDA research. The Show is scheduled for April 3, 2004 at the North Georgia Fairgrounds (Miller Park) on Calloway Road between Powder Springs Road and Austell Road in Marietta, GA. Hundreds of fantastic cars and many more car enthusiasts will be enjoying the day and contributing to our cause. Dianne needs volunteers to help with event admissions, parking, etc. If you can help, contact Dianne [dthigpen259@yahoo.com] or FARA at fara@frda.org. Even if you can't help, please come to the show if possible.

FARA adds Credit Card Donations

Until recently, FARA had no way for donors to make contributions by credit card. Now, just go to the FARA website (www.frda.org); at the very bottom of the homepage, click on fundraising; at the bottom of the fundraising page, click on the button that says "Donate Now Through Network for Good"; follow the easy instructions for submitting a credit card donation online.

"I would like to make an annual donation of $1,000 to FARA using my credit card. Please tell me how to do that." (anonymous donor)

" Thank you for adding credit card donations to the FARA website. I went on the website today and made a donation in honor of my wife's birthday." (anonymous donor)

SPINAL SURGERY GUIDE: TWO PARENTS' PERSPECTIVES

Keith's tattoo

Spinal fusion surgery is most often recommended due to the aggressive scoliosis that accompanies FRDA. For parents and patients searching for information on surgery you can *turn to the Friedreich's Ataxia Parents Group (FAPG) website*. Read parents' perspectives regarding Planning Steps from Six Months prior to the Operation, then up to and including Operation Day. Intensive Care, Hospital and Home Post-operative recoveries and school issues are also discussed at length. Visit FAPG at http://www.fortnet.org/fapg/scoliosi.htm

Keith Andrus secured a promise from his parents that he could get a tattoo after his spinal fusion surgery. Six months after the surgery, his orthopedic surgeon and his cardiologist gave the OK for the tattoo. The tribal design tattoo runs the length of Keith's scar. *Work by Patriot's Tattoo Artist Chris Hewitt, Fairfax, VA*

Friedreich's Ataxia Research Alliance

2001 Jefferson Davis Highway, Suite 209
Arlington, Virginia, USA 22202

PHONE (703) 413-4468

FAX (703) 413-4467

EMAIL fara@frda.org

WEB: www.frda.org

Mail To:

To be removed from this mailing list, email FARA at fara@frda.org

FARA DIRECTORS & OFFICERS

Ronald J. Bartek, President
US Military Academy, BS
Georgetown University, MA
Business/gov consultant, Washington, DC

Robert Wilson, MD, PhD
Brown University, Sc.B. Biochemistry
University of PA, MD, Medicine;
Ph.D, Genetics; Residency Clinical Pathology;
Postdoc, Transcription
Stellar Chance Labs,
Philadelphia, PA

Massimo Pandolfo, MD
University of Milan, Italy, MD, Medicine;
Residency, Neurology; University of Calif.,
Post-Doc, Internal Medicine
Chef de Service, Service de Neurologie
Université Libre de Bruxelles - Hôpital Erasme
Brussels, Belgium

Bronya J. B. Keats, Ph.D.
Australian National University, Genetics; Professor & Director, LSU Center of Excellence in Molecular & Human Genetics, New Orleans, LA

BJ Acker-Hitta
City College of San Francisco
Hotel/Restaurant Business Degree
McKinnen Institute of Massage/ CMT
Novato, CA

Raychel Bartek
University of SW Louisiana, BS, Business Administration,
Staff, US House of Representatives; Washington, DC

Mary Caruso, Fundraising Director
Small Business Owner, Northford, CT

Marilyn Downing, Secretary
St. Joseph College, BS, Special Education, StateUniversity of New York College, MS; Buffalo, NY

Terrence Downing, Treasurer
Canisius College, BS Accounting
Certified Financial Planner and Certified Public Accountant, Buffalo, NY

Fraser Goodmurphy, Communications Director
B.A. Honors Psychology
Special Student Status Biology
London, Ontario, Canada

William Hartnett
B.A.,Franklin &Marshall College
Program Manager, EDS

Sandy Lane
B.A., Psychology
Small Business Owner; Orange, CA

Rochelle and Marty Litke, Special Advisors; Founders, Seek A Miracle (SAM) Cherry Hill, NJ

Laura Kalick, Attorney at Law, General Counsel
Chevy Chase, MD

Milton Cerny, Attorney at Law, Legal Advisor
Washington, DC