



UNITED
MITOCHONDRIAL
DISEASE
FOUNDATION®



United Mitochondrial Disease Foundation 2012-2013 Annual Report

Making a Difference



Lori Martin, of Houston, TX has endeavored diligently to raise awareness about mitochondrial disease as her son, Will, battles Leigh's Disease.

Lori's blog about living with mitochondrial disease has resulted in an interview with ABC News and an appearance on CNN.com.

Message from the CEO

Last year I used the quote from Peter Ferdinand Drucker (1909 – 2005), ***“Efficiency is doing things right; effectiveness is doing the right things,”*** as the UMDF mantra as we move forward with our strategic plans addressing our mission: *“Promoting research and education for the diagnosis, treatment and cure of mitochondrial disorders and providing support to affected individuals and families.”*

I am pleased to report that the UMDF trustees and staff, with your continued support, have effectively, and efficiently, provided many services and benefits to our members and scientific communities.

1) Drug discovery is an inherently inefficient, expensive and slow process. Our Science and Alliance Officer, Phil Yeske, PhD, has established the UMDF as an “honest broker” among diverse groups working to address mitochondrial dysfunction. The UMDF provides leadership, energy and a singular focus on the affected community. The success we are having breaking down silos and coordinating the sharing of information is already paying dividends. We collect the most promising basic science and match it to the researcher or company poised with resources to advance a project.

2) Just recently, we were able to link a researcher with promising early data in neurodegeneration with a physician-scientist who has cell lines carrying primary mitochondrial dysfunction – neither knew of the others work, but both will benefit by the sharing of data and results because we connected the dots. Here's the surprising thing – these two scientists were from the same

university! We are making sure that all stakeholders, including the pharmaceutical community, are talking to each other. This helps “de-risk” the usual path of drug discovery by placing a patient advocacy group at the center of the process. “De-risking” makes it more likely that clinical drug trials can move forward.

3) We are monitoring the efforts of established mitochondrial diseased-focused assets such as the Mayo Clinic bio-repository and the National Institutes of Health (NIH) backed North American Mitochondrial Disease Consortium (NAMDC) ensuring the greatest effectiveness of both. As a result, the NIH is now putting more mitochondrial resources within the National Center for Advancing Translational Sciences. And, with our involvement and support, NAMDC has just been awarded an extended five year NIH grant. We continue to help these groups collect the elements necessary to characterize mitochondrial disease – a critical standardization process essential for clinical trials.

4) Given that drugs are ultimately brought to market by companies and not academic labs, we have established relationships with numerous companies who are already invested, or are most likely to invest, in mitochondrial therapeutic development. We will also help guide FDA reviews from the patient's perspective.

5) Through extensive bench-marking of other rare disease groups, we have determined the importance of initiating our own patient-populated registry and biobank to complement the scientifically-populated NAMDC project. A robust patient registry has been described as

Making a Difference

the most important asset that a rare disease community can possess in the quest for treatments and cures. Our registry will cast a wide net to ensure maximum patient engagement in upcoming research studies and clinical trials. We intend for our registry to become the “gold standard” international patient registry.

To enhance the usefulness of our registry, we helped launch and support a Mitochondrial Sequencing Data Resource Tool (MSeqDR). With the input from over 100 international mitochondrial disease experts, we are creating a genomic data resource that will help diagnose primary mitochondrial diseases (bringing together genomic data already being collected in clinics and labs around the world), and increasing the potential for accurate diagnosis and new treatments targeted to precise disorders.

We realize the need and value in taking a global approach to mission success and are working to engage our European counterparts by supporting the formation of the International Mitochondrial Patients (IMP) group. We are now partnering with other stakeholders from Canada, Australia, Germany, Belgium, The Netherlands, France, Spain, United Kingdom, and Italy. We believe that by combining forces we can be a stronger voice and research supporter for the mitochondrial community. A result of this collaboration is recent participation by our Italian friends at Mitocon who co-sponsored a research grant selected by our grant review committee and a memorandum of understanding with our counterparts from Tokyo, Koinobori.

We attended the most recent meeting of the International Rare Disease Research Consortium (IRDiRC). This is a global consortium teaming researchers and organizations committed to investing in rare disease research in order to achieve two main objectives by the year 2020, namely to deliver 200 new therapies for rare diseases and means to diagnose most rare diseases. We will be working diligently making sure mitochondrial disorders are included in their objectives. Working across borders is not an option, it is an imperative; the IRDiRC consists of 30 country members on three continents.

The North American Mitochondrial Disease Consortium (NAMDC) continues to gain momentum. With your support, there are now 13 active sites across the U.S. with over 400 patients entered in the registry producing clinical trials, a scientific populated patient registry, establishment of diagnostic standards, and a certified NAMDC diagnosis. The NAMDC registry will continue to grow and support opportunities for more clinical trials and ultimately treatments.

The Euromit Conference is a European based conference that is the largest scientific conference in the world dedicated to understanding how mitochondria are involved in disease. Euromit Conference 2014 will be the ninth in a series of international conferences and for the first time, Euromit 2014 sponsored a parallel meeting for mitochondrial families, patients, caregivers, and support organizations from many countries. Because of the recognized success of UMDF Symposia, we were asked to guide the patient platform and have involved members of

the IMP to support this new initiative. There were over 150 European families attending.

Because of your continued help and support and the success of our focus on the UMDF mission, the J. Willard and Alice S. Marriott Foundation has awarded UMDF over one million dollars to assist us in our efforts of finding treatments and cures for an affected community that deserves our strength and resolve. The UMDF trustees and staff understand that were it not for your past and continued support, and belief in our efforts, we would never have been able to progress to the point where an influential Marriott family foundation, a foundation that is approached by thousands of worthy causes, has seen the value of our plans and made a significant investment to help us move forward.

I began with a quote by Peter Drucker and I will end with another: ***“Effective leadership is not about making speeches or being liked; leadership is defined by results not attributes.”*** We firmly believe that with your continued help and support, we will be successful in bringing beneficial results to the mitochondrial community.



Charles A Mohan Jr.
UMDF CEO

Making a Difference

A Decade of Difference

- In 2003, our cumulative dollars spent on mitochondrial research had not yet reached a million. Today, UMDF has contributed more than \$10,000,000 leading to new clinical trials and potential treatments.
- In 2003, there were three clinical trials related to mitochondrial disorders. Today, there are 305 mitochondrial-related clinical trials ongoing.
- In 2003, researchers were just beginning to understand the link between mitochondrial dysfunction and other more common diseases. Today, a clear link has been established between dysfunctional mitochondria and Alzheimer's, Parkinson's, diabetes, certain cancers, and even the aging process itself.
- In 2003, UMDF had six chapters and 20 support groups. Today, UMDF has representation in every state and in 152 countries.
- In 2003, only two members of Congress knew about mitochondrial disease. Today, 372 members of the House and Senate have been informed. When they make decisions about how to spend significant federal money on health-related issues, mitochondrial disease will be on their minds.
- In 2003, there were 124 participants at the UMDF symposium, including only three from other nations. Today, more than 500 scientists, clinicians, allied health professionals, and family members attend each year, representing 16 countries.
- In 2003, gene sequencing was just a dream. Today, gene sequencing is a reality, identifying areas where mutations occur and targeting potential treatments.
- In 2003, 36 researchers applied to UMDF with research proposals. Today, over 200 new research proposals are received annually.
- In 2003, there were no UMDF "Grand Rounds" (programs where mitochondrial specialists travel to different hospitals to brief medical personnel on mitochondrial disease). Today, over 80 grand rounds have taken place, serving approximately 50 people each time, leading to 4,000 medical professionals being informed about the diagnosis and treatment of mitochondrial disorders.
- In 2003, there were only 21 identified "mito docs" worldwide. Today, there are over 300 identified mito docs worldwide.



Christopher Adkins of Danville, IL, won World Champion in Taekwondo for Sparring at the ATA World Championship in Little Rock, AR.

He is also the 2012 and 2013 Illinois State Champion for Forms and Sparring, as well as the Mid Atlantic 2013 District Champion. He competes in the Special Abilities-Physical-Black Belt Division ages 13 to 17.

Christopher is affected with mitochondrial disease.

Making a Difference



Top: The UMDF staff poses with Rachel Pipp during her recent visit to the UMDF office in Pittsburgh.

Center: Dr. Doug Wallace evaluates a clinician abstract on display at the 2013 Symposium.

Bottom: UMDF magnets, keychains, temporary tattoos and more are available to help raise awareness of mitochondrial disease.

Education

When a parent or affected adult is diagnosed with a mitochondrial disease, in most cases, the UMDF is one of the first few calls they make. They are seeking information about the disease. UMDF's Member Services team is ready to help with resource materials, physician referrals, and support opportunities. We are the calm voice that helps direct the affected community through the mitochondrial maze.

On average, UMDF fields more than 6,000 phone calls a year seeking help and information. While a majority of the calls are from the newly diagnosed community, we are here to help current patients seeking additional resources and information.

The UMDF Member services team has an abundance of information that is provided to patients and for patients to provide to their physicians. In FY 12-13, the UMDF Member Services team provided 132,000 copies of informational collateral to our members. In addition, the UMDF connected more than 600 patients seeking answers to questions in our "Ask the Mito Doc" program.

Educating physicians and clinicians about mitochondrial disease is equally important. That is why, with help from various sponsors, the UMDF is proud to present our Grand Rounds program. Grand Rounds enables us to bring a mitochondrial disease expert into a medical facility or university to present a lecture on the disease. The lecture may include information about diagnosis, treatment, therapies, etc. In this last fiscal year, the UMDF presented more than 20 Grand Rounds lectures at major medical institutions and academic centers.



The UMDf Chicago Support Group celebrates its ten year anniversary. Over the last decade, they have held 80 support and educational meetings. Many of their original members are still involved.



Making a Difference



The Houston S.P.A. (*Support, Play, Acknowledge*) event provided much needed respite care to patients and families.

Support

Don't become a victim of the disease, become part of the cure. UMDF provides multiple opportunities for affected individuals, their families, friends, and coworkers to be involved in support of our mission.

In FY 12-13, the UMDF Member Services Department helped organize 114 family meetings around the country attracting more than 2,100 participants wanting to learn more about their condition or its impact on a family member. A UMDF family meeting is an opportunity for affected individuals or their family to hear from and ask questions of a well-known mitochondrial disease specialist. They are provided the latest medical information about mitochondrial disease as well as information about UMDF programming.

UMDF is proud to provide opportunities such as family meetings, information, referrals, website information, collateral materials, and networking opportunities free of charge to the affected community and their families.

Making a Difference

Collaboration



Without patient participation, clinical trials leading towards potential treatments and cures cannot get off the starting block. Realizing the need to provide patients with relative information about clinical trials and other studies, UMDF has been a longtime supporter of the North American Mitochondrial Disease Consortium (NAMDC). UMDF provides a grant to NAMDC to help facilitate the entry of patient information into the NAMDC database so that the affected community can be kept informed about current and new clinical trials and studies.

UMDF participates as a patient advocate representative in the Rare Disease Clinical Research Network (RDCRN). The RDCRN is a network of research consortia funded by the National Institutes of Health (NIH) and its Office of Rare Diseases Research (ORDR) to interact collaboratively in the research of rare diseases. Its purpose is to work together to contribute to the research and treatment of rare diseases by identifying biomarkers for disease risk, disease severity and activity, and clinical outcome, while also encouraging development of new approaches to diagnosis, prevention, and treatment.

In FY 2012-2013, the UMDF agreed to collaborate and assist with the planning of EUROMIT, a conference on mitochondrial pathology that will take place in Finland in 2014. The conference attracts more than 500 experts from around the globe.

In FY 2012-13, representatives from Japan traveled to the UMDF offices in Pittsburgh to learn how to begin a patient advocacy group in their country (*pictured above*). UMDF continues to provide them with information that is being translated into Japanese for the patient population of that nation. The UMDF also shares information and resources in a collaborative way with MitoCanada, and the Australian Mitochondrial Disease Foundation.

The UMDF is also a participant in MitoCon, an Italian based non-profit with similar interests of the UMDF. In fact, MitoCon and the UMDF have jointly funded two research grants approved by the UMDF Research Grant Advisory Panel. The last grant jointly funded by the UMDF and Mitocon was in FY 12-13.



Pittsburgh Magazine named Jerry Vockley, M.D. and Amy Goldstein, M.D. among Pittsburgh's Best Doctors in 2013.

Doctors Vockley and Goldstien are the Chair and Co-Chair of the UMDF's 2014 Symposium to be held in Pittsburgh.

Pittsburgh
MAGAZINE®



Advocacy

It was standing room only in Cannon House Office Building Room 122 when the UMDF conducted its first ever meeting of the Congressional Mitochondrial Disease Caucus. The September 2012 meeting was chaired by sponsors Rep. Anna Eshoo (D-18-CA) and Rep. Tim Murphy (R-18-PA). This first session focused on the mitochondrial diseases and the role mitochondria play in the human health for all of us. The caucus was formed at the request of more than 230 UMDF members who participated in the 'Day on the Hill' meetings scheduled by the UMDF in June of 2012. The Congressional Mitochondrial Disease Caucus is a critical educational component for members of Congress as it enables the UMDF, as a patient advocacy group, to communicate the needs of our members and the urgent need for research dollars dedicated to the discovery of potential treatments and cures.

It was also in September 2012, that Senator Barbara Boxer (D-CA) introduced Senate Resolution 490. This resolution mandated that the National Institutes of Health (NIH) follow through with the recommendations that came out of the UMDF sponsored workshop in March 2012. The workshop identified major barriers to the development of potential treatments for mitochondrial disorders. The resolution, which passed in the Senate, directs the NIH to work with scientists and researchers both inside and outside of the NIH to address these barriers and find solutions.

Over the years, the UMDF focused its advocacy efforts primarily on the legislative branch. In early 2013, the focus expanded to the executive branch. UMDF held a meeting with Carole Johnson, President Obama's Senior Advisory for Healthcare. UMDF brought to the White House two mitochondrial disease experts to explain to Johnson the importance of research into primary mitochondrial disorders and its implication for many other diseases (Parkinson's Alzheimer's, diabetes) on human health.

Making a Difference

Treatments

Who could imagine that a bake sale, car wash, gala, or Energy for Life Walkathon could produce more than \$11 million in research funding over the past 15 years? Because of our dedicated members and their urgent need to find and fund better treatments and potential cures, UMDF could not have achieved this. Research funded over the life of the UMDF is now starting to have a major impact.

There was exciting information presented from the industry side of drug development highlighting the potential therapeutic effects of Bendavia, a product of the company Stealth Peptides. Because of its mechanism of action, Bendavia has the potential for therapeutic efficacy across a broad spectrum of mitochondrial diseases.

UMDF has also been on the forefront of providing information for patients about the work coming out of Edison Pharmaceuticals. According to the California based company, a study of patients with the mitochondrial disease Leigh's Syndrome have seen significant improvement in their neuromuscular function and quality of life while taking EPI -743. UMDF continues to work with Edison to spread the word to our patient community about the exciting work coming out of Mountainview, CA.

In June 2013 at Mitochondrial Medicine: Newport Beach, researchers outlined more than 20 potential therapies online for those who suffer with mitochondrial disease. Many of these therapies are the direct result of donor dollars provided to the UMDF for research. For example, 2012 UMDF research grant recipient, Dr. Carla Giordano, of the University of Rome, reported that cells treated with naturally occurring plant compounds may be useful in treating LHON. Dr. Fernando Scaglia, of Baylor College of Medicine and a member of the UMDF Scientific and Medical Advisory Board, reported that two other naturally occurring compounds, arginine and citrulline could be useful in the treatment of MELAS. And then there's dark chocolate. Dr. George Schreiner of California based Cardero Therapeutics presented data on the chocolate derived flavanol known as epicatechin. According to Dr. Schreiner, the compound has been shown to increase the number of mitochondria in the muscle cells of elderly patients improving their overall metabolism.



ESPN reported on making 9-year-old Kyle Felver's dream come true. Kyle is an avid midget-car racer and a huge fan of NASCAR driver Kyle Busch.

The Stanwood, WA, resident got the opportunity to meet NASCAR star Kyle Busch as part of the "Make A Wish" program. Kyle and his family visited Kyle Busch Motorsports in Mooresville, NC, where he received a personalized tour. The next day he took a tour of Charlotte Motor Speedway and was on pit road for All-Star Race qualifying. The trip concluded with Kyle and his family watching the NASCAR Sprint Cup All-Star Race in Charlotte.

"Little" Kyle is suspected of having a mitochondrial disease.

2013 Grant Award Winners

In FY 2012-2013, the UMDf Awarded \$509,000 in research grants. Our cumulative amount of research grants is now more than \$11 million. UMDf remains the largest non-governmental funder of research into mitochondrial disease.

James Stewart, PhD

Max Planck Institute for the Biology of Ageing
Cologne, Germany
Chairman's Award - \$90,000 for two years

“Using mtDNA mutator mouse-derived lineages to generate mouse models of human mitochondrial diseases.”

By working with mice that are prone to mitochondrial mutations, Dr. Stewart will develop new genetic models of human disease. Once established, these mouse models can be used for the development of new drug therapies.

Alberto Sanz-Monterro, PhD

University of Tampere, Tampere, Finland
\$100,000 for two years

“A Genome-wide RNAi Screening to Identify New Genes Involved in Mitochondrial Diseases.”

Dr. Sanz-Monterro will use a well-understood fruit-fly model to discover previously unknown genetic defects that can cause mitochondrial disease. Many mitochondrial disease patients have not had a specific genetic mutation linked with their disease, and this research will help to fill that gap.

Rajesh Ambasudhan, PhD

Sanford-Burnham Medical Research Institute,
La Jolla, California
\$84,000 grant for two years

“A Human Reprogrammed-Cell Model of MELAS.”

Dr. Ambasudhan will obtain skin cells from MELAS patients and reprogram them as nerve cells to be grown in culture. This “disease-in-a-dish” model will be used to gain insights into mitochondrial dysfunction in MELAS and other mitochondrial diseases.

Natalie Niemi, PhD

University of Wisconsin, Madison, Wisconsin
\$75,000 for two years

“Utilizing dynamically regulated phosphorylation as a means to modulate mitochondrial metabolism.”

Dr. Niemi will study mechanisms that activate enzymes in the mitochondria, with the goal of understanding how this regulation is impaired in mitochondrial disease. This could lead to new therapeutic options for mitochondrial disease patients.

Alicia Pickrell, PhD

National Institute of Neurological Disorders & Stroke
Bethesda, Maryland
\$75,000 for two years

“Therapy for mitochondrial diseases: an investigation into the potential to stimulate Parkin-mediated mitophagy.”

Dr. Pickrell is studying the effects of the drug Rapamycin on the removal of abnormal mitochondria from cells in mice. This FDA-approved drug has the potential to selectively eliminate dysfunctional mitochondria in humans, helping to restore normal energy metabolism in mitochondrial disease patients.



UNITED MITOCHONDRIAL DISEASE FOUNDATION

Date: Jun

UMDF connect
Resources for help & support



UMDF recognized Salvatore DiMauro, MD, with its first even Vanguard Award. The Vanguard Award is given to an individual to recognize their leadership and commitment towards a cure for mitochondrial disease, and whose inspiration has resulted in bringing new scientific and medical experts into the field. Dr. DiMauro is a past member of the UMD Board of Trustees and a past member of the UMD Scientific and Medical Advisory Board.





Top: The scientific program offers doctors and clinicians insights into cutting-edge therapies and treatments.



Middle: The teen sessions feature round-table discussions and strategies specifically for teens living with mitochondrial disease.

Bottom: A popular event during Symposium, "The Doctor is In" allows parents and patients to sit with mitochondrial disease specialists from around the country and directly discuss their issues and treatments.

Symposium

The UMDF is also proud to host what is known as the premier medical, scientific and family meeting in the world. The annual UMDF Symposium rotates around the country each year. It is the place where the latest in mitochondrial disease information is shared among the medical and scientific community. It is also the place where families and affected individuals learn the latest information and are able to network with each other.

In FY 2012-2013, the UMDF Symposium was held in Newport Beach, CA. More than 500 representatives from the scientific, medical, and patient community attended. UMDF was proud to provide more than \$40,000 in scholarship dollars to affected individuals and families so that they would be able to attend to gain information, network and learn.



Much to the surprise of attendees at the 2013 Symposium, Mitochondrial Medicine 2013: Newport Beach, actor/musician, and longtime UMDF supporter, Jack Black, dropped by for a visit.

Empowerment

Fundraising

In addition to the Energy for Life Fundraisers, hundreds of events are held annually around the nation in an effort to raise money for support, education and research. These events range from bake sales to car washes to galas.

Awareness Week

The third week of September is 'Mitochondrial Disease Awareness Week'. It is a week in which we encourage the affected community to do their part to raise awareness. UMDF provides specific collateral, support, information and instructions to those who wish to spread awareness. In FY 2012-2013, more than 250 awareness activities were scheduled across the country. UMDF received media attention through print and broadcast media with more than 30 placements nationwide. More than 1,500 pieces of collateral and other information was distributed at no charge to those wishing to spread awareness about mitochondrial disease.



Top: Dawgs for Mito, the country's first collegiate chapter of the United Mitochondrial Disease Foundation, helped organize the second annual Carson's Classic golf outing.



Middle: The tenth annual Bet on Baylee was hosted in Roseville, OH. Baylee's family, friends and supporters kept the festivities going for 13 continuous hours.



Bottom: Cecelia Coloret, a fifth grade teacher at Hunter's Green Elementary School in Tampa, FL, formed "The Mighty Mito Fighters" to help raise awareness of mitochondrial disease. A child in kindergarten at the school is affected.





Left: Scenes from Energy for Life Walks (from top) in Atlanta, Central Texas and Akron.

Far Left: The Energy for Life Walk in Pittsburgh travels along the Allegheny River Riverwalk.



Energy for Life

Our signature event is the UMDF Energy for Life Walkathon. In FY 2012-13, more than 25 walks took place in numerous cities across the United States. These walkathons not only spread awareness about mitochondrial disease, but they also raise funds for research and education. During the fiscal year, more than 56,000 people participated in a walk.

In the fall of 2011, there were eight Energy for Life Walkathons held across the United States in these cities: Camden, NJ; Charlotte, NC; Chicago, IL; Bloomington, MN; Kansas City, KS; Murray City, UT; Sugar Grove, PA; and Williamsville, NY. These eight walks raised nearly \$380,000.

There were ten Energy for Life Walkathons held in the winter and spring of 2012 in these cities: Ann Arbor, MI; Atlanta, GA; Binghamton, NY; Cedar Falls, IA; Evansville, IN; Houston, TX; Indianapolis, IN; Nashville, TN; Pittsburgh, PA; San Francisco, CA; and St. Louis, MO. These ten walks raised approximately \$1,627,000.

Research Funds

UMDF Research Funds are established by families as a way to honor or memorialize a loved one affected by mitochondrial disease. Donations to one of the funds listed below ensures that the world's top mitochondrial scientists are receiving the support they need to perform breakthrough research. Research Funds from July 1, 2012 to June 30, 2013:

The Anthony Demarko Maccarelli Research Fund	The Corynna Strawser Research Fund	The Katherine Dickens Research Fund
The Ainsley Paige Higgins Research Fund	The Dawnta & Levi Kendall Family Research Fund	The Kristen Charleston Research Fund
The Andrew Radney Research Fund	The Elena's Hope Research Fund	The Lauren Benney Research Fund
The Angelray Research Fund	The Elizabeth Piro Research Fund	The Leslie Whitt-Williams Research Fund
The Ayden and Faith Hingsbergen Research Fund	The Emily Steadman Research Fund	The Lex Santo Research Fund
The Bishop/Lauer Family Research Fund	The Emma Frances Dalton Research Fund	The Nicholas James Torpey Research Fund
The Brady Sterchi Family Research Fund	The Hunt Michael Hollis Research Fund	The Oliver Scheier Research Fund
The Brandon David Harris Research Fund	The Isabella Magee Research Fund	The Olivia Paige Goldberg Research Fund
The Brandon Heschel Leach Research Fund	The Isabella Lin Ramirez Research Fund	The Rachael Albertson Research Fund
The Breylon Senn Research Fund	The Isabelle Sherman Research Fund	The Samuel Cutliff Research Fund
The Brittany Wilkinson Research Fund	The Jack Edwards Research Fund	The Spry Research Fund
The Caleb Jacobs Research Fund	The Jackson Rothschild Research Fund	The T.J. Amber Research Fund
The Carter Buffum Research Fund	The Jaethan Myers Research Fund	The Taryn Fogel Research Fund
The Champions for Chad Research Fund	The Jonah Ritterbush Research Fund	The Will Martin Family Research Fund
The Christopher Schindler Research Fund	The Jude Manley Research Fund	
The Connor McArthur Research Fund: Kids Like Connor	The Kaidon Andrew Stamper Research Fund	



Certified Public Accountants and Business Consultants

3328 Washington Road
McMurray, PA 15317-3005
Tel (724) 260-0900
Fax (724) 260-5210

Joseph S. Stelmack, CPA
Joseph T. Dobransky, CPA
Vincent M. Eannace, CPA

INDEPENDENT AUDITOR'S REPORT

To the Board of Trustees of the United Mitochondrial Disease Foundation, Inc.

We have audited the accompanying financial statements of the United Mitochondrial Disease Foundation, Inc. ("the Foundation") (a nonprofit organization), which comprise the statements of financial position of June 30, 2013 and 2012, and the related statements of activities, functional expenses and cash flows for the years then ended, and the related notes to the related notes to the financial statements.

Management is responsible for the preparation and fair presentation of these financial statements in accordance with the accounting principles generally accepted in the United States; this includes the design, implementation and maintenance of the internal control relevant to the preparation and fair presentation of financial statements that are free from material misstatement, whether due to fraud or error.

Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of the United Mitochondrial Disease Foundation, Inc. as of June 30, 2013 and 2012, and the changes in its net assets and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

STELMACK DOBRANSKY & EANNACE, LLC
McMurray, Pennsylvania

May 1, 2014

Financials

**UNITED MITOCHONDRIAL
DISEASE FOUNDATION, INC.**

**STATEMENTS OF FINANCIAL POSITION
JUNE 30, 2013 AND 2012**

	2013	2012
<u>ASSETS</u>		
Cash and cash equivalents	\$ 586,269	\$ 667,724
Accounts receivable	93,265	73,801
Pledges receivable (Note 3)	125,000	0
Inventories	50,798	29,595
Investments (Note 4)	1,584,356	1,396,035
Prepaid expenses	28,240	24,573
Fixed assets - net (Note 5)	108,213	48,648
TOTAL ASSETS	<u>\$2,576,141</u>	<u>\$ 2,240,376</u>
<u>LIABILITIES AND NET ASSETS</u>		
LIABILITIES		
Accounts payable	\$ 310,391	\$ 283,750
Accrued liabilities	57,277	45,628
Grants payable (Note 6)	1,171,360	989,484
Deferred revenue	0	6,096
Total liabilities	<u>1,539,028</u>	<u>1,324,958</u>
NET ASSETS		
Unrestricted	644,700	503,742
Temporarily restricted (Note 8)	392,413	411,676
Total net assets	<u>1,037,113</u>	<u>915,418</u>
TOTAL LIABILITIES AND NET ASSETS	<u>\$2,576,141</u>	<u>\$ 2,240,376</u>

**UNITED MITOCHONDRIAL
DISEASE FOUNDATION, INC.**

**STATEMENTS OF ACTIVITIES AND CHANGES IN NET ASSETS
FOR THE YEARS ENDED JUNE 30, 2013 AND 2012**

	2013			2012		
	Unrestricted	Temporarily Restricted	Total	Unrestricted	Temporarily Restricted	Total
PUBLIC SUPPORT AND REVENUE						
Support:						
Fundraising	\$1,396,670	\$ 63,384	\$1,460,054	\$ 1,425,637	\$ 102,611	\$ 1,528,248
Contributions	826,897	3,465	830,362	342,683	8,000	350,683
In honor of	113,312	0	113,312	113,592	0	113,592
In memory of	70,240	0	70,240	86,230	0	86,230
In kind	63,247	0	63,247	37,733	0	37,733
Grants	117,135	114,588	231,723	107,158	98,226	205,384
Cancellation of grants payable	23,893	0	23,893	135,000	0	135,000
Total support	2,611,394	181,437	2,792,831	2,248,033	208,837	2,456,870
Revenue:						
Symposium and seminars	274,764	2,000	276,764	263,315	0	263,315
Membership	0	0	0	17,330	0	17,330
Sales	7,055	0	7,055	15,475	0	15,475
Miscellaneous	1,157	0	1,157	0	0	0
Total revenue	282,976	2,000	284,976	296,120	0	296,120
Investment income	52,745	0	52,745	43,702	0	43,702
Net unrealized gain (loss) on investments	135,637	0	135,637	(33,701)	0	(33,701)
Net assets released from program restrictions	202,700	(202,700)	0	119,688	(119,688)	0
Total support and revenue	3,285,452	(19,263)	3,266,189	2,673,842	89,149	2,762,991
FUNCTIONAL EXPENSES						
Program services:						
Research	987,583	0	987,583	785,012	0	785,012
Public awareness	357,829	0	357,829	288,946	0	288,946
Education/member support	998,610	0	998,610	870,703	0	870,703
Total program services	2,344,022	0	2,344,022	1,944,661	0	1,944,661
Supporting services:						
Administrative and general	134,178	0	134,178	126,403	0	126,403
Fundraising	666,294	0	666,294	692,435	0	692,435
Total supporting services	800,472	0	800,472	818,838	0	818,838
Total expenses	3,144,494	0	3,144,494	2,763,499	0	2,763,499
CHANGES IN NET ASSETS	140,958	(19,263)	121,695	(89,657)	89,149	(508)
NET ASSETS - Beginning of year	503,742	411,676	915,418	593,399	322,527	915,926
NET ASSETS - End of year	\$ 644,700	\$ 392,413	\$1,037,113	\$ 503,742	\$ 411,676	\$ 915,418

See Independent Auditor's Report and
Notes to the Financial Statements

Administration

Scientific & Medical Advisory Board

Marni Falk, MD - Chair

The Children's Hospital of Philadelphia
Philadelphia, PA

Laurence Bindoff, MD

Haukeland University Hospital
Bergen, Norway

David Chan, MD, PhD

California Institute of Technology
Pasadena, CA

William C. Copeland, PhD

National Institute of Environmental Health Sciences (NIEH)
Research Triangle Park, NC

Richard H. Haas, MB, BChir

UCSD Medical Center
San Diego, CA

Marcia Haigis, PhD

Harvard Medical School
Boston, MA

Michio Hirano, MD

Columbia University Medical Center
New York, NY

Giovanni Manfredi, MD, PhD

Weill Medical College of Cornell University
New York, NY

Edward E. McKee, PhD

Indiana University School of Medicine
South Bend, IN

Sumit Parikh, MD

Cleveland Clinic
Cleveland, OH

Russell P. Saneto, DO, PhD - Secretary

Seattle Children's Hospital
Seattle, WA

Fernando Scaglia, MD

Baylor College of Medicine
Houston, TX

Mark Tarnopolsky, MD, PhD, FRCP (C)

McMaster University
Hamilton, Ontario



The UMDF sends its condolences to the Marriott Family on the passing of Stephen Marriott from complications of mitochondrial disease.

“Stephen was an inspiration to us every day as he struggled with a challenging disease that cost him his eyesight and hearing, and he never complained,” Stephen’s father, Bill. “He came to work right until the end to champion the values that his grandparents established when they opened their first root beer stand. Stephen was convinced that our Marriott culture was our greatest competitive advantage. His wife and children will remember his ability to find joy and happiness, despite his daily hardships.”

Stephen Marriott was 54.

UMDF Staff

- **Charles A. Mohan Jr.**
CEO/Executive Director
- **Mark Campbell**
Chief Financial Officer
- **Phil Yeske, PhD**
Science and Alliance Officer
- **Kara Strittmatter**
Director of Member Services
- **Clifford Gorski**
Director of Communications
- **Tania Hanscom**
Special Events Coordinator
- **Leslie Heilman, JD**
Associate Director of Development
- **Donna Nameth**
Data Entry Manager
- **Melinda O'Toole**
Member Services Associate
- **Jean Bassett**
Research Grants Coordinator
- **Liz Weiss**
Special Events & Member Services Associate
- **Rachel Mazur**
Special Events Associate
- **Nicole Shanter**
Special Events & Development Associate
- **Fred Prefling**
Development Associate
- **Barbara Podowski**
Administrative Assistant
- **Janet Owens**
Executive Administrative Assistant
- **Alison Cooley**
Communications Assistant
- **Jeff Gamza**
Multimedia Coordinator
- **Cassie Franklin**
Regional Coordinator - Central
- **Anne Simonsen**
Regional Coordinator - Great Lakes
- **Margaret Moore**
Regional Coordinator - Southeast

Board of Directors

- W. Dan Wright**
Chairman
- Sharon Shaw**
Vice Chair
- Marty Lyman**
Secretary
- John A. DiCecco**
Treasurer
- Carl Daniels**
- Bruce H. Cohen, MD**
- Gerald Cook, Esq.**
- Linda Cooper**
- Marnie Falk, MD**
- Norma Gibson**
- Amy Goldstein, MD**
- Richard Leach**
- Patrick Kelley**
- Joseph Rice**
- Tyler Reimschisel, MD**
- Debra Schindler-Boultinghouse**



**UNITED
MITOCHONDRIAL
DISEASE
FOUNDATION.**