UMDF connect

UNITED MITOCHONDRIAL DISEASE FOUNDATION

HOPE. ENERGY. LIFE.

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21st Century Cures Act Passes the House

It is now up to the United States Senate to vote on the 21st Century Cures Act. During the UMDF's "Day on the Hill" in June, we encouraged participants to ask their House and Senate members to support the measure. Also known as H.R. 6, the bill passed the U.S. House on July 10, 2015, with bi-partisan support.

The 21st Century Cures Act was introduced in the House because health care innovation is moving very fast. From the mapping of the human genome to personalized medicines linked to advances in molecular medicine, breakthroughs continue that are changing the way disease is treated, managed and cured. Those who proposed the legislation say federal drug and device approvals lag far behind the advances in medical science. That is why the legislation, led by House Energy and Commerce Committee Chairman, Fred Upton (R-MI-06), was introduced.

The 21st Century Cures Act helps to modernize the research infrastructure and streamlines the bureaucracy between patients and cures for conditions like mitochondrial disease. The National Institutes of Health (NIH), whose budget has been stagnant over the past few years, will see an increase of $10 billion over the next five years. More resources to speed along the testing and approval of new treatments will be provided to the Food and Drug Administration (FDA).

Under the measure, clinical research trials, vital in providing new medications and therapies, will be expanded.

The 21st Century Cures Act has received widespread praise from an array of doctors, researchers and experts in medical innovation. Dr. Francis Collins, Director of the National Institutes of Health, says he has "never seen a more exciting time" in transforming biomedical research.

Life sciences research drives resources and dollars into our hospitals and educational and scientific institutions, helping them prepare the next generation of scientists and doctors. The bill will help new scientists begin careers in research and apply their skills to tackling disease. It enables scientists and caregivers to more effectively communicate, collaborate and share data on the diseases they study.

The measure now rests within the U.S. Senate. If approved there, it will be sent to the President for his signature.

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From the Chairman

We are coming off another very successful UMDF Symposium, and I am always amazed by the number of people who travel from near and far to attend. No matter how much we know about mitochondrial disease, it is our job as patients and caregivers to continually expand that knowledge. It should be our personal mission to stay on top of the latest information and developments, because they seem to be coming at a very rapid pace. I always come away from the UMDF Symposium energized after hearing the presentations and sessions that clearly point to the fact that we are moving forward toward potential treatments and cures. If you were able to be with us in Washington, DC, in June, you no doubt heard about the many clinical trials that are underway or about to start. These trials are critical to the affected community, because they are paving the way towards better treatments and potential cures.

I am also delighted to report that UMDF awarded nearly $500,000 towards research. If successful, two of these research projects could result in a faster diagnosis; an additional two could result in potential therapies - one supports a clinical trial and one supports a potential treatment. You can read more about these projects in this newsletter, but we are very excited about the potential these research projects could bring to patients and families in the not so distant future.

As you read this, the calendar is rapidly approaching the month of September. It's a busy month. Awareness week will begin on September 20th. Awareness Week is sandwiched in between our 17 Energy for Life Walkathons that will continue into November. I encourage you to do your part in raising awareness. Anything that helps us get the word out about mitochondrial disease and its impact is considered a success. Check out the fall Energy for Life schedule at *www.energyforlifewalk.org*. Find a walk near you and sign up a team. Encourage family and friends to exercise their mitochondria by participating in a walk.

The path to a cure is made possible through awareness and the power of the Energy for Life Walkathon!

Patty Kelley

Patrick Kelley, UMDF Chairman

DAY ON THE HILL

More than 230 UMDF members and their families took part in the third "Day on the Hill" on June 18, 2015. After being bussed to the Hill in the morning, members fanned out across the United States Capitol for scheduled meetings with their Congressmen and two U.S. Senators.

UMDF Members were educated on the issues that are affecting the mitochondrial disease community and were instructed to provide three requests of their House Members and one for each U.S. Senator.

Among the "asks" for Congress was a request for each Member to join the Congressional Mitochondrial Disease Caucus. Currently, 20 members of Congress are active on the caucus. The goal for our "Day on the Hill" participants was to double the amount of Congressmen sitting on the caucus and participating in the numerous briefings held each year to educate elected officials about mitochondrial disease and its impact on individuals and families.

UMDF members were also instructed to ask their Congressmen to sign onto a letter, written by Rep. Jim McGovern (D-MA-02) and Rep. Tim Murphy (R-PA-18) to the NIH Director Francis Collins, MD, PhD. The letter to Dr. Collins asks the NIH to ensure that each of the relevant NIH institutes are issuing mitochondrial-specific Requests for Applications and Program Announcements in an effort to bolster inclusion of such research in their various research portfolios; utilize a portion of Common Fund resources to stimulate mitochondrial research; make the pre-clinical development of therapeutics for the treatment of mitochondrial diseases a focus area for the Therapeutics for Rare and Neglected Diseases (TRND) program within the National Center for Advancing Translational Sciences (NCATS); work to develop novel, safe and effective nutritional interventions for mitochondrial disease; and The Office of the Director should create a coordinator for mitochondrial research who is empowered to hold the various institutes accountable for their commitment and progress.

Congress members were also asked to support the 21st Century Cures Act, which does several things to help with the clinical trial process and the review and approval of new drugs and biologicals.

For their meetings with U.S. Senators, UMDF members asked each to sign onto a similar letter to Dr. Collins. That letter was written and signed by Senator Barbara Boxer (D-CA). Numerous Senators said they would review the letter and consider adding their name.

If you were unable to participate in Washington, DC, you can add your voice from home. UMDF invites you to visit the UMDF Legisilative Initiatives page and make appointments in your community with your Congressman and U.S. Senators. A complete outline of talking points, "asks", and materials for your meeting can be viewed and downloaded at www.umdf.org/legislation. Our fourth "Day on the Hill" will be held in Washington, DC, in June 2017.


Dino Scanio and his son, Giovanni, talk about how mitochondrial disease impacts their family with Rep. Gus Bilirakis of Florida. Dino's son, Gianluca, has mitochondrial disease.


Micheal and Peggy Wichert of Husdon, Ohio, meet with U.S. Senator Sherrod Brown.


Melissa Lane and her son, Parker, meet with Congressman Evan H. Jenkins of West Virginia.

New Congressional Caucus Members

At the time the newsletter went to press, eight congressmen joined the Congressional Mitochondrial Caucus as a direct result of "Day on the Hill". They are:

Rep. Hank Johnson (D-GA-04)
Rep. David P. Joyce (R-OH-16)
Rep. Ryan Costello (R-PA-06)
Rep. Jared Polis (D-CO-02)
Rep. Julia Brownley (D-CA-26)
Rep. Mike Doyle (D-PA-14)
Rep. Doug Collins (R-GA-09)
Rep. Matt Salmon (R-AZ-05)

We now have 27 House Members on the Caucus. Great work and please don't forget to ask your Congressman to join!

Dysfunction Linked to Aging?

Salk Institute Researchers in California have discovered an association between a longevity hormone, fat metabolism and mitochondrial disease in mice. Researchers analyzed mouse models of mitochondrial disease and premature aging and discovered a hormone associated with longevity and the body's response to starvation was very active even though the mice showed signs of a fast aging process.

Mitochondrial Dysfunction and Colorectal Cancer Connection?

The journal *Diagnostic Pathology* reports on a new potential biomarker. Kuwaiti researchers analyzed tumor samples from 30 people with colorectal cancer. In the study, researchers assessed the possible link between colorectal cancer and the mitochondrial A12308G alteration in tRNA Leu (CUN), as well as its utility as a disease biomarker. tRNA genes are crucial for the biological synthesis of new proteins. Mutations in mitochondrial tRNA genes have been considered a useful biomarker for different cancers, as defects in mitochondrial function have been suggested to have an impact on the development and progression of cancer.

New Therapeutic Approach Revealed

Human Molecular Genetics recently published a study revealing a novel, promising therapeutic approach for human mitochondrial disease based on the findings in human cells and animal models.

The study is entitled "Inhibiting cytosolic translation and autophagy improves health in mitochondrial disease" and was conducted by researchers at The Children's Hospital of Philadelphia and Perelman School of Medicine at University of Pennsylvania.

"In this study, we investigated the effects of modulating not just signaling molecules, but two of the basic biological processes they regulate: protein translation and autophagy," explained the study's senior author, Dr. Marni J. Falk, in a news release. "Both of these cellular activities are abnormal in mitochondrial disease, and we showed that using drugs that partially inhibit them offer novel treatment strategies."

Stem Cell Therapy

A new study recently published in the renowned journal, *Nature,* revealed a breakthrough on combined stem cell and gene therapies for the treatment of mitochondrial diseases. The study is entitled "Metabolic rescue in pluripotent cells from patients with mtDNA disease" and was led by researchers at Oregon Health & Science University. Scientists collected skin cells from patients with mitochondrial DNA mutations and, through a technique based on mitochondrial replacement via nuclear transfer (MRT), they were able to generate embryonic stem cells with healthy mitochondria.


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H-Q
Chew-Q 600 mg
QGel Forte
QGel MEGA 100
QGel Ultra
Liquid Q

UMDF

members

Uplifting stories about our members

Calling All Artists!

UMDF is looking for a design for our annual holiday card. We are looking for art that features a holiday theme,spreads mitochondrial disease awareness, and is created by patients or their family members.

How can you participate?

1. Create your artwork and take a picture of it.
2. Save your artwork in a JPEG format
3. Email your JPEG file to jeff.gamza@umdf.org
4. YOU MUST INCLUDE the name of the artist, age, city/ state and a contact phone number.
5. Submission deadline is Friday, September 11, 2015, at 5:00 pm Eastern.

On Monday, September 21, 2015, during Awareness Week, a panel of judges will choose three pictures and post them on the UMDF Facebook Page.

The artwork with the most votes on Friday, September 25, 2015, at 5:00pm Eastern will be used for the cover of our 2015 UMDF Holiday Card!

2014 Holiday Card Artist Matt Calhoun

2013 Holiday Card by Rachel Pipp

2008 Holiday Card by Kelsee Pollak

SAVE THE DATE!

Mitochondrial Medicine 2016: Seattle

Scientific Program: June 15 - 18, 2016
Family Program: June 17 - 18, 2016

Doubletree by Hilton
Seattle Airport
18740 International Boulevard
Seattle, WA 98188

Scientific Course Chair:
Russell Saneto, DO, PhD
Seattle Children's Hospital / University of Washington

CME Chair:
Bruce H. Cohen, MD
Akron Children's Hospital

Walking to Raise Awareness

Karen Loftus joined the Energy for Life Walkathon: Chicago in 2009. She was starting a lifelong journey to raise awareness and advocate for not only herself, but all those affected by mitochondrial disease. It wasn't until 2010 that she would learn of her own diagnosis (her mother, sister, niece and nephew also have mitochondrial disease). Since that time, she has participated in countless events benefitting the UMDF, became an ambassador and brought an Energy for Life Walkathon to her hometown (Milwaukee/Southern Wisconsin).

"It is empowering to raise money for a cure, and the EFL's are a great way for our community to come together and embrace each other. The support is great not only for affected people but also for families and friends," Loftus said.

During the past 13 months, Karen had been using Cam Walkers on her legs; and as soon as she was able to take them off, she decided she was going to start walking. This past spring, Karen began working with Dave Dobke in Wisconsin on his annual golf outing, "The Greater Mito Open," in honor of his daughter, Brianna, who passed away from mitochondrial disease. Karen was so inspired by their story that she decided to join the UMDF's ***Activate your Mitochondria*** program and turn her daily walks into something larger - she was going to hike the length of the Appalachian Trail (2,180 miles)!

Soon Karen was plotting long distance walks and hikes, something she hadn't been able to do in a long time! She found that plotting gorgeous walks helped to keep her inspired. Mentally, she said it was a huge relief to get her first walk out of the way. That walk was 8 minutes long, now she can do up to 20 miles in one day! Her favorite place to hike is Seven Bridges on Lake Michigan, it was her first elevation challenge. "It is beautiful and as realistic as we can get to the Appalachian Trail in Wisconsin."
Karen's next challenge is to walk the circumference of Lake Geneva, which she will attempt sometime this year. Although the physical activity is challenging, many days Loftus feels that walking has helped to improve some of her mito symptoms.

"In doing this challenge, it forces me to get up every day and get moving," she said.

Karen's favorite thing about the ***Activate your Mitochondria*** program is that it gives those affected outside of an Energy for Life Walkathon city the ability to participate and make a difference.

"I use this as my rallying cry," Loftus cheers.

For more information and to follow Karen's story, check out her Facebook page at https://www.facebook.com/ATMitoAwareness

Join Karen by ***activating your mitochondria***! For more, visit www.umdf.org/activemito/at_mito_awareness

Planning for the Future

In last quarter's issue, we introduced you to the importance of planning for your future by sharing information on wills and how to get started. We continue the "estate planning process" conversation this quarter by sharing some information from our partners at The Monteverde Group and Dayton & Kaikus, PC. "But I don't have an estate!", some folks may say – read on to learn more about what you can do now to protect what you do have in your "estate" regardless of size.

- Beth Whitehouse

Estate Planning: An Introduction

By definition, estate planning is a process designed to help you manage and preserve your assets while you are alive, and to conserve and control their distribution after your death according to your goals and objectives. But what estate planning means to you specifically depends on who you are. Your age, health, wealth, lifestyle, life stage, goals, and many other factors determine your particular estate planning needs. For example, you may have a small estate and may be concerned only that certain people receive particular things. A simple will is probably all you'll need. Or, you may have a large estate, and minimizing any potential estate tax impact is your foremost goal. Here, you'll need to use more sophisticated techniques in your estate plan, such as a trust.

To help you understand what estate planning means to you, the following sections address some estate planning needs that are common among some very broad groups of individuals. Think of these suggestions as simply a point in the right direction, and then seek professional advice to implement the right plan for you.

OVER 18

Since incapacity can strike anyone at anytime, all adults over 18 should consider having:

- A durable power of attorney: This document lets you name someone to manage your property for you in case you become incapacitated and cannot do so.
- An advanced medical directive: The three main types of advanced medical directives are (1) a living will, (2) a durable power of attorney for health care (also known as a health-care proxy), and (3) a Do Not Resuscitate order. Be aware that not all states allow each kind of medical directive, so make sure you execute one that will be effective for you.

YOUNG AND SINGLE

If you're young and single, you may not need much estate planning. But if you have some material possessions, you should at least write a will. If you don't, the wealth you leave behind if you die will likely go to your parents, and that might not be what you would want. A will lets you leave your possessions to anyone you choose (e.g., your significant other, siblings, other relatives, or favorite charity).

UNMARRIED COUPLES

You've committed to a life partner but aren't legally married. For you, a will is essential if you want your property to pass to your partner at your death. Without a will, state law directs that only your closest relatives will inherit your property, and your partner may get nothing. If you share certain property, such as a house or car, you may consider owning the property as joint tenants with rights of survivorship. That way, when one of you dies, the jointly held property will pass to the surviving partner automatically.

MARRIED COUPLES

For many years, married couples had to do careful estate planning, such as the creation of a credit shelter trust, in order to take advantage of their combined federal estate tax exclusions. A new law passed in 2010 allows the executor of a deceased spouse's estate to transfer any unused estate tax exclusion amount to the surviving spouse without such planning. This provision is effective for estates of decedents dying after December 31, 2010.

You may be inclined to rely on these portability rules for estate tax avoidance, using outright bequests to your spouse instead of traditional trust planning. However, portability should not be relied upon solely for utilization of the first to

die's estate tax exemption, and a credit shelter trust created at the first spouse's death may still be advantageous for several reasons:

- Portability may be lost if the surviving spouse remarries and is later widowed again.
- The trust can protect any appreciation of assets from estate tax at the second spouse's death
- The trust can provide protection of assets from the reach of the surviving spouse's creditors
- Portability does not apply to the generation-skipping transfer (GST) tax, so the trust may be needed to fully leverage the GST exemptions of both spouses

Married couples where one spouse is not a U.S. citizen have special planning concerns. The marital deduction is not allowed if the recipient spouse is a non-citizen spouse (but a $147,000 annual exclusion, for 2015, is allowed). If certain requirements are met, however, a transfer to a qualified domestic trust (QDOT) will qualify for the marital deduction.

MARRIED WITH CHILDREN

If you're married and have children, you and your spouse should each have your own will. For you, wills are vital because you can name a guardian for your minor children in case both of you die simultaneously. If you fail to name a guardian in your will, a court may appoint someone you might not have chosen. Furthermore, without a will, some states dictate that at your death some of your property goes to your children and not to your spouse. If minor children inherit directly, the surviving parent will need court permission to manage the money for them.

You may also want to consult an attorney about establishing a trust to manage your children's assets in the event that both you and your spouse die at the same time.

You may also need life insurance. Your surviving spouse may not be able to support the family on his or her own and may need to replace your earnings to maintain the family.

COMFORTABLE AND LOOKING FORWARD TO RETIREMENT

If you're in your 30s, you're probably feeling comfortable. You've accumulated some wealth and you're thinking about retirement. Here's where estate planning overlaps with retirement planning. It's just as important to plan to care for yourself during your retirement as it is to plan to provide for your beneficiaries after your death. You should keep in mind that even though Social Security may be around when you retire, those benefits alone may not provide enough income for your retirement years. Consider saving some of your accumulated wealth using other retirement and deferred vehicles, such as an individual retirement account (IRA).

WEALTHY AND WORRIED

Depending on the size of your estate, you may need to be concerned about estate taxes.

For 2015, $5,430,000 is effectively exempt from the federal gift and estate tax. Estates over that amount may be subject to the tax at a top rate of 40 percent.

Similarly, there is another tax, called the generation-skipping transfer (GST) tax, that is imposed on transfers of wealth made to grandchildren (and lower generations). For 2015, the GST tax exemption is also $5,430,000, and the top tax rate is 40 percent.

Whether your estate will be subject to state death taxes depends on the size of your estate and the tax laws in effect in the state in which you are domiciled.

ELDERLY OR ILL

If you're elderly or ill, you'll want to write a will or update your existing one, consider a revocable living trust, and make sure you have a durable power of attorney and a health-care directive. Talk with your family about your wishes, and make sure they have copies of your important papers or know where to locate them.

Dr. Doug Wallace Presented with UMDF Vanguard Award

The UMDF Vanguard Award is awarded to a veteran mitochondrial disease clinician or researcher who has demonstrated exceptional work in advancing the mitochondrial medicine field. The recipient of this award has been at the forefront of medical and scientific contributions in the fields of mitochondrial medicine and research for many years. This person has demonstrated a lifetime commitment of progress towards a cure through research and medical treatment and has diligently served the UMDF in a board or SMAB capacity. This person has also been involved with UMDF patient and educational programs and has provided inspiration to others in an effort to bring new scientific and medical experts into the fields of mitochondrial medicine and research.

The 2015 Vanguard Award was presented to Douglas C. Wallace, PhD. Dr. Wallace is Director at the Center for Mitochondrial and Epigenomic Medicine (CMEM) at Children's Hospital in Philadelphia. He is also a professor in the Department of Pathology & Laboratory Medicine at CHOP.

More than 35 years ago, Dr. Wallace and his colleagues founded the field of human mitochondrial genetics. One of his contributions has been to use mtDNA variation to reconstruct the origin and ancient migrations of women. These studies revealed that humans arose in Africa approximately 200,000 years ago, that women left Africa about 65,000 years ago to colonize Eurasia, and from Siberia, they crossed the Bering land bridge to populate the Americas. Studies on the paternally-inherited Y chromosome showed that men went along too.

A longtime supporter of UMDF and its patients and families, Dr. Wallace is a passionate supporter of UMDF efforts in advocacy and a former member of the Scientific and Medical Advisory Board. Dr. Wallace is also a member of the National Academy of Sciences and the 2012 winner of the prestigious Gruber Prize in Genetics.

Symposium Recap

by Phil Yeske, PhD, UMDF Science & Alliance Officer

The arrival of over 500 researchers, clinicians and family members in June of this year at Mitochondrial Medicine 2015 in Herndon, VA, heralded the 17th annual symposium sponsored by UMDF. As usual, there were scientific and family-oriented tracks and, as is customary for when the symposium is located nearby to Washington, DC, there was also a strong advocacy component via Day on the Hill, along with NIH-sponsored activities. The scientific registrants represented a truly international composition, with several participants traveling from as far away as Eastern and Southeast Asia.

This year's scientific program was co-chaired by Drs. Marni Falk of the Children's Hospital of Philadelphia and Danuta Krotoski of the National Institutes of Health. The first three days focused on the multitude of roles that mitochondria play within cells, while day four focused on the role of genetic counseling in mitochondrial disease and therapeutic development efforts. Following are highlights from the various platforms.

Harnessing the Power of BIG DATA to Understand Small Organelles

"Big data" is a hot topic on many scientific fronts these days as an exponential increase in computing power delivers more and more information to researchers for analysis. The keynote presentation in this platform was delivered by Dr. Eric Green, Associate Director of the National Institutes of Health, and longtime innovator in human genomics research. Dr. Green spoke to a quarter century of progress in not only mapping the human genome but also the challenges in translating the deluge of genomic information into actionable therapeutic opportunities. The healthcare system of the United States is converting into a "learning system," which is opening the door to an era of precision medicine. Precision medicine as it relates to mitochondrial disease was the topic of Dr. Falk's follow-up presentation in which she emphasized the challenge of diagnosis with a patient community that can present anywhere from 7 to 35 symptoms to varying degrees. Access to tools that tie together genomic and phenotypic (symptomatic) information will be the key to harnessing the power of "big data," and the Mitochondrial Sequencing Data Resource Tool, MSeqDR, under development through funding by UMDF and NIH, will serve as a single, powerful database for pooling such information for use by clinicians and researchers active in mitochondrial disease research. Co-developer of MSeqDR, Dr. Xiaowu Gai of Harvard Medical School, provided further details to the conference attendees about how the feature-laden database serves as a one stop shop for variant mapping, annotation, exome data analysis and disease discovery tools. With better understanding of the genes underlying mitochondrial disease comes the opportunity for therapeutic development. Dr. Massimo Zeviani of

the MRC Mitochondrial Biology Unit and University of Cambridge, described the development of new in vitro models of mitochondrial energy creation (OXPHOS) and how they have been exploited to implement novel therapeutic strategies based on gene and cell replacement, or pharmacological control of mitochondrial biogenesis (creation of new mitochondria). Dr. Vamsi Mootha closed out the session with a summary of how database-driven genomics, next generation sequencing and metabolic profiling are combined in his lab to improve the diagnosis and management of mitochondrial disease.

Mitochondrial Disease Mechanisms: Dissecting the Cellular Response to Mitochondrial Disease

Although a number of interesting talks were presented in this session on important aspects of cellular response to mitochondrial disease, two talks that focused on mimicking the benefits of exercise to restore mitochondrial function stole the show. Cutting edge work in this area has been conducted for years at McMasters University under the leadership of Dr. Mark Tarnopolsky. The Tarnopolsky lab has identified a series of exerkines- beneficial factors to cellular health that are released upon exercise. Because many mitochondrial disease patients are intolerant to exercise, the ability to develop a so-called “exercise in a bottle” pill would represent a potentially transformative therapy. With over 25 exerkines now identified, a next major challenge is how to deliver these agents within the body, and significant progress has been made in the Tarnopolsky lab utilizing exosomes- tiny cellular vesicles used to transport various biomolecules throughout the body, including across the blood-brain barrier, thus opening up the potential of addressing difficult to treat myriad neurologic symptoms often observed in mitochondrial disease. A variety of disease models have already been successfully utilized to demonstrate this concept, including a POLG1 rat as well as MELAS and LHON patient fibroblasts. A McMaster University co-worker of Dr. Tarnopolsky, Dr. Adeel Safdar, provided additional exosome therapy data showing a 4-fold increase in mitochondrial biogenesis, bringing us a step closer to safe and promising “exercise pills.” Dr. Tarnopolsky founded EXERKINE Corporation to explore the commercialization of these interesting new agents.

Mitochondrial Connections to Cellular Life

Cardiolipin is the major phospholipid building block in the mitochondrial inner membrane, where it increases substrate channeling, promotes respiratory supercomplex assembly, and facilitates the potential gradient. Genetic defects in TAZ, the protein responsible for cardiolipin remodeling, result in Barth syndrome. Dr. Steven Claypool of Johns Hopkins University described how he and his colleagues established a model yeast system for studying these defects, proving that although they remodel cardiolipin on different sides of the inner mitochondrial membrane, the loss of function mechanism is the same in yeast as seen in patients. Turning to the outer mitochondrial membrane and its important role in importation, Dr. Carla Koehler of University of California Los Angeles (and UMDF Scientific and Medical Advisory Board member) described her interesting work in developing modulators of protein import. When importation is compromised in some way mitochondrial dysfunction results, and the scientific community now accepts as fact that mitochondrial dysfunction is a contributing factor in a multitude of degenerative diseases. The session was wrapped up by this year’s UMDF Vanguard Award winner for lifetime commitment and achievement within mitochondrial disease research or medicine- Dr. Doug Wallace of University of Pennsylvania. Dr. Wallace provided a wide-ranging overview on the relationship of bioenergetics and disease, and how the single gene-single disease model has in large part been found to be the exception and not the rule.

Mitochondria-Induced Mayhem: The Role of Other Organelles in Mitochondrial Disease

The Friday morning session focused on the complex interactions between the mitochondria and other organelles in the cell. Dr. Wiep Scheper of the University of Amsterdam opened the session with a discussion of his lab’s work on metabolic stress and protein homeostasis. They proposed a model where increased metabolic stress leads to upregulation of the unfolded protein response and phosphorylation of the tau protein. If left unchecked, this response can become pathogenic, such as in Alzheimer disease patients. Dr. Scheper’s talk was followed by Dr. Michael Glickman of the Technion-Israel Institute of Technology and his work on the relationship between proteasome systems (the garbage disposal systems of the cell) and the mitochondria. His lab has detected the first evidence of proteasomic activity in mitochondria, and he showed that blocking of certain pathways leads to detrimental mitochondrial fragmentation and increased oxidative species, potentially leading to neurodegenerative disorders. Dr. Keshav Singh of the University of Alabama examined the effect of decreased mitochondrial DNA in tumor cells. His lab found a number of defects present in both healthy and tumor cells lacking mitochondria. These studies help to explain the vast array of symptoms seen with mitochondrial disorders.

Cellular Nutrition and Mitochondrial Function

The Friday afternoon session began with Kathryn Camp of the NIH’s Office

Dr. Yeske would like to thank Drs. Karen DeBalsi, Megan McManus, Rachel Krasich and James Byrnes for their contributions to this article.

of Dietary Supplements discussing the outcomes of the 2014 NIH-hosted Workshop "Nutritional Interventions in Primary Mitochondrial Disorders." The primary purpose of the workshop was to promote a research agenda with a priority on primary mitochondrial disorders (PMD). Defining PMD and identifying biomarkers, outcome measures, and endpoints for monitoring disease were key topics of the workshop. The UMDF is working closely with the NIH to coordinate a wide range of follow-up activities resulting from this workshop. Dr. Patricia Berninsone of the University of Nevada examined the impact of sugar-modified proteins on mitochondrial function. Her work suggests that such modifications impact a wide variety of important mitochondrial mechanisms and are more prevalent than previously thought. Dr. Scott Bultman of the University of North Carolina discussed the impact of epigenetics (modified genes) on cellular nutrition, emphasizing the importance of a two-way flow of information between mitochondria and the nucleus.

Genetic Counseling in the Mitochondrial Community

The Saturday morning session focused on mitochondrial clinical care in general and the role of genetic counseling in particular. Drs. Mary Kay Koenig of University of Texas and Patrick Chinnery of Newcastle University began the session with a comparison of the clinical care models in the United States and the UK. Both increasingly rely on genetic testing, and genetic counselors are often the first line of communication for patients and families. Elizabeth McCormick, a genetic counselor at Children's Hospital of Philadelphia described best practices that her institution utilizes for conveying complex information, realizing that children, adolescents and adults all process such information in different manners. Dr. Richard Boles of Courtagen Life Sciences reinforced the importance of communication, especially in identifying an appropriate level of information to disclose so as not to create unnecessary anxiety. It is an individualized process, unique to each case, and Dr. Boles encouraged patients to ask as many questions as necessary to ensure an understanding of what they are signing and receiving.

To Market, To Market: How to Prioritize Drugs for Clinical Trials and Most Effectively Achieve FDA Approval

The final session of the scientific program at Mitochondrial Medicine 2015 focused on therapies in development for mitochondrial disease and the clinical trials required for drug approval. The patient perspective was presented by Dr. Zarazuela Zolkilpi Cunningham, from Children's Hospital of Philadelphia, who spoke to the barriers and motivations of patient involvement in clinical trials. Designing said clinical trials for a highly heterogeneous disease population such as mitochondrial disease is a major challenge, reported Dr. Jeffrey Krischer of University of South Florida. With limited numbers of patients available, it is critical to develop enhanced trial designs that can still deliver the power necessary to gain FDA approval. Precision medicine based on N=1 ("N of 1") trials are the ultimate in personalized design, but also the most challenging to develop. Building the case for FDA approval was the topic of Dr. Peter Stacpoole of University of Florida's presentation. Drawing upon years of personal experience, Dr. Stacpoole spoke to the need for disease background information, natural history and a well-chosen primary outcome measure as key elements of a therapeutic regulatory strategy. Dr. Greg Enns of Stanford University closed the conference with an overview of the drugs ready (or nearly ready) for mitochondrial disease-focused clinical trials. Based on data available on www.clinicaltrials.gov, there are currently 17 trials either active or recruiting for mitochondrial disease patients. Other potential therapeutics are in pre-clinical planning, and still others remain in early research- emphasizing the need for all the stakeholders in the mitochondrial disease community to work together at this important time.

Mitochondrial Medicine 2015 was a whirlwind of scientific sharing and discussion over four days. Next year the venue will shift to the Pacific Northwest in Seattle, Washington. As always, UMDF will work closely with the scientific program chair to design a relevant, high impact agenda.

Symposium Awards

At its annual symposium, Mitochondrial Medicine 2015: Washington DC, the United Mitochondrial Disease Foundation honored several volunteers for their efforts and dedication in supporting the organization and the patients and families it represents.

Katie Parsons of Marietta, Georgia, is the recipient of this year's Heartstrings Award. Katie was diagnosed with mitochondrial disease when she was 3 years old, after numerous illnesses and hospitalizations that began in infancy. Now 13 years old, Katie is a caring, spunky young lady who enjoys percussion, dance, and making things out of duct tape. Katie turned her love of duct tape crafts into Katie's Duck Tape Kreations by making wallets, bookmarks, jewelry…and even an entire dress!...all out of duct tape. She has donated proceeds to the UMDF and other mito-related causes.

The Heartstrings award is presented to an individual under the age of 18 who has invested their time, demonstrated their talents, effectiveness and generosity in raising money or donations to enable the UMDF to continue its mission.

Katie participates every year in the Energy for Life Walk in Atlanta and proudly served as a Junior Ambassador at age 7, when she gave her first speech at a UMDF walk. Katie is determined to educate others about mitochondrial disease by talking about the condition in her school, community, social media and the news. "Who knows," she says, "maybe one of them will want to become a mito doctor!?" She wants to make a difference for not only herself but for others with mitochondrial disease, such as her mom and friends she has made through the years.

It takes a village to raise a child, and the support and encouragement Katie has received, from all of you, has helped in her resolve to make a difference and find her cure.

The Energy Award recognizes an individual who embodies the spirit of the UMDF and its mission: Promoting research and education for the diagnosis, treatment and cure of mitochondrial disorders and providing support to those affected. The 2015 Energy Award Recipient is Sherri Schultz of Buffalo, New York.

A tireless and selfless advocate for mitochondrial disease, Sherri is a main driving force for mitochondrial disease awareness and action in Buffalo. Sherri became involved with the UMDF after her diagnosis in 2007. She has volunteered and participated in the Energy for Life Walkathon in Western New York since 2009.

After a lifelong struggle to find answers, Sherri realized how little awareness there was for mitochondrial disease. Since then, she started an online group support group, Mito Café, and in 2014 became a Support Leader for UMDF. Through her work with UMDF, she leads meetings, brings speakers to local UMDF members, participates in community awareness events, networks and promotes other regional UMDF events and co-chaired the 2014 Energy for Life Walkathon in Western New York.

Sherri also advocates for the UMDF with Day on the Hill and all advocacy asks, as well as referring others to do the same. Sherri is also the UMDF representative on a legislative committee working to pass legislation which will require insurance companies to cover the 'mito cocktail' and has been meeting New York State Senators and assembly members to gain their support and educate them about mitochondrial diseases and the UMDF Mission.

Sherri has a 15 year old son, Ryan, who is clinically suspected of having a mitochondrial disease and she is a lifetime partner of Richard Schultz.

The LEAP Award recognizes an individual living positively with mitochondrial disease, highlighting the person's accomplishments and volunteer service.

Chelsea Loyd is 20 years old and just completed her freshman year of college at Columbus State University. In 2012, she was diagnosed with mitochondrial disease, which finally provided an answer for all of the health issues she has suffered from birth. When she graduated from high school, she could not start college right away because she was facing two major knee surgeries. Many people would have opted not to go after missing a full year, but Chelsea couldn't wait to get started. Chelsea was a cheerleader in middle school and high school; however, by her junior and senior years, she could not cheer through a complete football game due to the mito. Her goal was to become a college cheerleader and, in spite of having mitochondrial disease and three major knee surgeries, she is actively pursuing this dream.

Chelsea has been the co-chair for the Energy for Life Walk in Columbus, Georgia, since it began in 2013. She embraces the opportunity to bring awareness to the community about mito. She has done newspaper and radio interviews, presented at The Optimist Club civic club and spoke to the Columbus City Council about mitochondrial disease and the EFL Walk - just to name a few of her opportunities. This year, she is helping with the inaugural "Go Pro for Mito" golf tournament, another event designed to help raise awareness about mitochondrial disease and raise funds for a cure/treatment.

Chelsea learned about a fundraiser at the EFL training and has adopted the "Flamingo Fundraiser" as her way of personally raising money for her EFL team. By placing the flamingos and information signs in yards in our community, she is raising awareness and "earning" money for her team.

Cheryl Porter, who lives in Flowery Branch, Georgia with her husband David, Sr. and her son David, Jr., is the 2015 winner of the Stanley Davis Leadership Award. The award is the UMDF's most prestigious national award honoring a UMDF Leader that best represents the selfless dedication and commitment to the UMDF Mission as that of its namesake.

In 2009, Cheryl Porter just sent the last of her five children off to college and thought that life was going to slow down. In March, David began to experience dizziness and blurred vision. In May, she heard the words "mitochondrial disease" for the first time as he was diagnosed with Leigh's Disease. Once David became more stable, Cheryl and David traveled to the UMDF to meet everyone and learn as much as possible about mitochondrial disease. It was in the conference room at the UMDF that she uttered the fateful words, "I'll do anything that I can do to help!"

Since that time, she has helped with numerous fundraisers in the Atlanta area for the UMDF. She has been the Co-chair for the Atlanta Energy for Life walk for four years, raising over $200,000. Cheryl helped institute the successful Symposium Synopsis event in Atlanta which is now entering its 4th year.

"Hearing the words '"mitochondrial disease" in 2009 totally changed my life, but we are grateful every day for the support and encouragement of the friends that we have made along the way and for the UMDF," said Cheryl.

Research Grant Awards

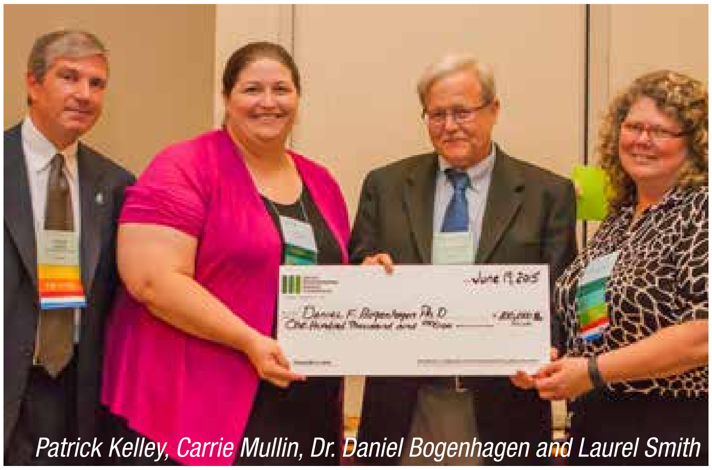
The United Mitochondrial Disease Foundation, through your generous donations, Family Research Funds and Energy for Life Walkathons, is able to support nearly $500,000 in direct grants in 2015 to the following scientists. In addition to these grant awards, UMDF Supports the North American Mitochondrial Disease Consortium (NAMDC), MSeqDr, and the Mitochondrial Disease Community Patient Registry, bringing our direct support towards treatments, therapies, and potential cures to more than $750,000 in 2015.

Dr. Lisa Riley and Patrick Kelley

John Christodoulou, PhD, of Children's Hospital at Westmead in New South Wales, Australia was awarded $100,000 for his project, "Utility of FGF21 and GDF15 as Diagnostic and Prognostic Biomarkers of Mitochondrial Respiratory Chain Disorders." Dr. Christodoulou will validate optimal methodology in a clinical diagnostic laboratory setting to determine the utility of measuring FGF-21 and GDF-15 as biomarkers of pediatric mitochondrial disease. This has become a major question in the field, as to how potentially useful in terms of sensitivity and specificity these biomarkers are for mitochondrial disease.

John Christodoulou, PhD

This grant is funded in part by the UMDF Olivia Paige Goldberg Family Research Fund and the Australian Mitochondrial Disease Foundation under the leadership of CEO Sean Murray.



Patrick Kelley, Carrie Mullin, Dr. Daniel Bogenhagen and Laurel Smith

Daniel F. Bogenhagen, MD, Department of Pharmacological Sciences at Stony Brook Universitywas the recipient of an $100,000 UMDF Research Grant.   Dr. Diaz's project is entitled "Kinetics of Mitochondrial Complex Assembly." Dr. Bogenhagen is utilizing mass spectrometry techniques to study the assembly map of the mitoribosome as well as the mitochondrial respiratory complexes. The improved understanding of both of these mitochondrial construction projects will enhance the diagnosis and future therapy of mitochondrial disorders.

This grant is funded in part by the Corynna Strawser Family Research Fund & the fundraising efforts of our UMDF families participating in Energy for Life Walkathons. Carrie Mullin and Laurel Smith, Co-Chairs of the Delaware Valley Energy for Life Walk, presented the award. This walkathon has raised over $250,000 since 2010. Carrie and Laurel have walk teams in honor of their sons, "Patrick's Parade" and "Jamie's Dirt Dogs."

Patrick Kelley, Dr. Joni Nikkanen and Brent Fields

Anu Suomalainen Wartiovaara, MD, PhD, University of Helsinki, Finland was the recipient of a $100,000 grant for the project "Vitamins B as Therapy for Disorders with mtDNA Instability." Dr. Suomalainen Wartiovaara will utilize a mouse model to build upon preliminary results indicating that vitamins B, especially B3 (Niacin) play key metabolism regulatory roles in patients with mitochondrial myopathies. Pre-clinical data generated in mice will inform the creation of a follow-up human clinical trial on the impact of Niacin supplementation for the alleviation of symptoms due to mitochondrial disease.

Dr. Suomalainen Wartiovaara

This grant is funded in part by the UMDF Brandon Leach Family Research Fund and the UMDF Chloe Fields Family Research Fund. Brent Fields, Secretary on the UMDF Board of Trustees, presented the research grant In the short time the research fund has been established, the Fields have raised over $15,000.

Patrick Kelley, Dr. Atif Towheer and Kate Crawford

Atif Towheer, PhD, Department of Pathology and Laboratory Medicine, Children's Hospital of Philadelphia, received a $70,000 UMDF Research Grant. Dr. Towheed's project is entitled "Allotopic RNA Rescue of LHON Mouse Model." The goal of Dr. Towheed's work is to develop a novel gene therapy strategy for the treatment of Lebers hereditary optic neuropathy (LHON) utilizing a mouse model developed in the labs of Dr. Douglas C. Wallace. If this therapeutic approach is successful it could inhibit the onset of the optic nerve pathology.

This grant is funded in part by the UMDF John Geraci Family Research Fund and the LHON Project Fund at UMDF, including the Poincenot family's "C.U.R.E. Ride"; the Loomer family's "Dinner in the Dark" ; Shane Stewart & family's "Birdie's for the Blind"; Lee Lyons' "Stride for Sage"; and Chaz Davis & family's "20/20 fundraiser". These families raised over $75,000.

The grant was presented by Kate Crawford for the John Geraci Research Fund. John and Kate established the John Geraci Research Fund in 2013 after John was finally diagnosed the previous year.

Patrick Kelley, Dr. Sara Nowinski and Tom and Elizabth Hefferon

Sara M. Nowinski, PhD, Department of Biochemistry University of Utah, was the recipient of a $70,000 grant award. Dr. Nowinski's project is entitled "Characterizing the Function of the Atypical Mitochondrial Kinase ADCK3." The studies in Dr. Nowinski's grant will improve the understanding of ADCK3 function in the synthesis of coenzyme Q and cerebellar ataxia. Additionally, better treatment strategies for mitochondrial disease could be developed in the future if new roles for ADCK3 are identified.

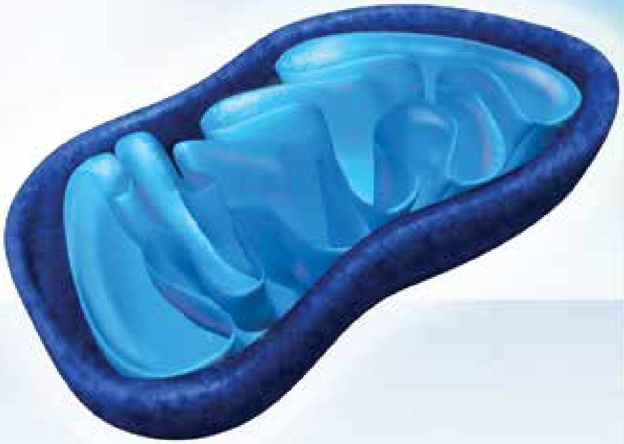
This grant is funded in part by the UMDF Olivia Paige Goldberg Family Research Fund and the fundraising efforts of many UMDF families participating in our Energy for Life Walkathons, including our presenters Garret & Lindsey Flynt, parents of Jaxson. New to the UMDF Family, their family organized the 'Fight for a Cure' Golf Tournament. Combined, they have raised nearly $35,000.

Peter Stacpoole,MD, PhD, Department of Medicine, University of Florida, received a grant award of $24,898. Dr. Stacpoole's project is entitled "Validation of an Observer Reported Outcome (ObsRO) Measure of Home Functionality in Children with Pyruvate Dehydrogenase Complex Deficiency (PDCD)." Dr. Stacpoole, in response to a specific request from the FDA for information from the patient or patient family to aid in regulatory decisions, has developed an innovative computer tool to track home functionality of pediatric PDCD patients. The pilot study in 10 PDCD families will test the feasibility of the survey instrument and refine it as needed for its eventual use in a planned Phase III trial of dichloroacetate (DCA). If the trial shows DCA is found safe and effective, it could lead to this drug being designated as the first FDA-approved therapy for PDCD.

Anne Tuccillo and her co-chair, Sharon Goldin have organized the Fall into a Cure event since 2009. Over the last six years, the event has raised nearly $225,000. Anne and Sharon plan this event in honor of their sons, Bryan Tuccillo and Jake, Cole & Clayton Didinsky.

Patrick Kelley, Dr. Peter Stacpoole and Anne Tuccillo

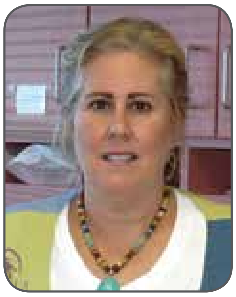
The UMDF Research Grants are funded through the work and dedication of UMDF volunteers and donors who support Energy for Life Walkathons and other special events, Holiday Appeals, UMDF's Path to A Cure, and UMDF Family Research Funds. If you would like to learn more about helping to further our research with a gift, please contact the UMDF Development Department at 888.317.UMDF (8633).



Ask the Mito Doc℠

Living with mitochondrial disease presents many twists and turns, and a maze of questions. UMDF is pleased to offer answers to some of those questions as taken from Ask the Mito Doc℠ at www.umdf.org. Please note that information contained in Ask the Mito Doc℠ is for informational and educational purposes only. Such information is not intended to replace and should not be interpreted or relied upon as professional advice, whether medical or otherwise.

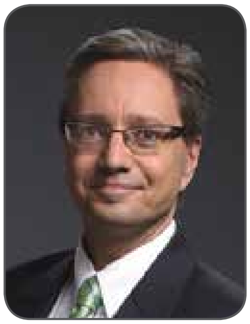
Q: I have KSS. I am 54 years old and was correctly diagnosed (muscle biopsy) in 1987. In regards to dental work, is it okay for me to have a local anesthetic agent containing Lidocaine Hydrochloride?



Fran D. Kendall, MD

A: It is not really a question for a non-treating physician as it is very specific to care and should be addressed by your treating physicians who know all of your issues and other medications, many of which may impact your response to this medication and the procedure. Having said that, the best I can answer is as follows: Lidocaine can be mito toxic and its neurotoxicity is linked to mitochondrial apoptosis (destruction and subsequently cell destruction). Having said that, the dose typically used for a minor procedure is small and applied locally and more than likely won't cause problems. However, your dentist needs to be aware that you are at greater risk than the average person so that he/she can either use the smallest dose possible or elect to utilize another anesthetic agent. - *Fran D. Kendall, MD*

Q: I want to know if you have heard of or know of the use of low level light therapy in the treatment of mitochondrial disease?



Greg Enns, MD, ChB

A: Low level laser therapy is an interesting phenomenon that in theory could have an effect on mitochondria, but I have not heard of its use in the treatment of primary mitochondrial disease. There have been some interesting reports of its use in Parkinson's disease, Alzheimer's disease and amyotropic lateral sclerosis. - *Greg Enns, MD*

You can quickly and easily find "Ask the Mito Doc" Q/As on topics of your choice by going to the UMDF home page at www.umdf.org. Go to "Find Support" and click on "Ask the Mito Doc." Click on the search "Ask the Mito Doc" link; this will pull up a search box. Type in a keyword or phrase you are interested in and click "Go." The search engine will pull up every Q/A that mentions your word or phrase. If you are not satisfied with the results, try variations or synonyms of your word/phrase.

WEBINAR

Stay tuned for our upcoming hosted webinar with updates on research and current clinical trials, followed by a question and answer session.

Visit *www.umdf.org* for more information on upcoming date and time!

AACT News

SYMPOSIUM

The AACT was proud to host the 9th Annual Adult Gathering at the 2015 UMDF Symposium in Washington DC, as well as participate in the Day on the Hill. Thanks to everyone who attended the Symposium and the Gathering! It was great to see everyone, learn the latest about mitochondiral disease in the sessions, and have many exciting discussions.

If you were not able to attend the Symposium, you still have the opportunity to watch presentations via the UMDF website! Visit *www.umdf.org/symposium* to access the Presentation archives, and watch much of the 2015 Symposium Family Program right on your computer screen!

EVENTS

Over two dozen EFL Walkathons take place across the country to help fund vital research grants to find treatments and cures for mitochondrial diseases and disorders. If you cannot walk a good distance, don't let this discourage you from forming a team, joining a team, attending or forming a virtual team. The venues are fantastic, and it's a great day to join together with energy, resilience, and hope. *www.energyforlifewalk.org*

To find and view listings of other upcoming events and meetings in your area, visit *www.umdf.org* and click on Find Support - lots happening!

AACT is honored and proud to represent, serve, and support you.

Adult Advisory Council Team (AACT)

Jennifer Schwartzott, AACT Chair, New York
Gail Wehling, AACT Co-chair, Illinois
Devin Shuman, YA Coordinator, Washington
Kailey Danks, Canada
Whit Davis, Pennsylvania
Rev. David Hamm, Maryland
Pam Johnson, MD, Missouri/Kansas
Christy Koury, North Carolina
Joy Krumdiack, Washington
Terry Livingston, Florida
Deb Makowski, Arizona
David McNees, Ohio
Sharon Shaw Reeder, California
Gregory Yellen, Maryland

Medical Advisors:

Bruce H. Cohen, MD
Amy Goldstein, MD

Purpose of AACT

To represent and serve the unique needs of the affected adult community and to ensure that those needs are adequately represented to UMDF resulting in enhanced services to the affected adult population.

AACT is a liaison to the UMDF Board of Trustees and will assess and evaluate, provide advice and guidance, and make recommendations to UMDF on adult-related issues.

Companies Match Your Efforts

Your support enables us to fund our critical mission; but did you know that many companies match cash donations and volunteer hours? Last year, these companies and foundations generously supported employee giving campaigns benefiting UMDF. Thank you to the employees who gave donations and volunteered their time!

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Questions? We're happy to help.

Leslie Heilman
leslie.heilman@umdf.org
412-793-8077 ext 122

Barbara Cullaj
barbc@umdf.org
412-793-8077 ext 100

Donor Spotlight

April 1, 2015 - June 30, 2015

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UMDF

events

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Former Ohio State Buckeye Jamal Luke and Baylee at Bet on Baylee

Fundraisers Benefitting the UMDF

March 21, 2015 – The 6th Annual Jackson Culley Mito What? 5k was held at the USA Stadium in Millington, TN and raised a whopping $21,405.95!

March 28, 2015 – The EFL Walkathon: Atlanta was held at Piedmont Park's Promenade Lawn. Despite the inclement weather, the EFL still raised $39,942!

March 28, 2015 – The EFL Walkathon: Dallas - Fort Worth was held at the Downtown Garland Square. The event was filled with fun activities and raised $69,174!

March 28, 2015 – The EFL Walkathon: Nashville managed to have a great turnout despite below freezing temperatures! Everyone stayed warm and energized and raised $25,463!

March 29, 2015 – The Second EFL Walkathon: Tampa Bay was held at Al Lopez Park and raised $17,042!

April 11, 2015 – The first annual EFL Walkathon: Shreveport/Bossier City was an exciting occasion held at Airline High School and raised $23,838!

April 18, 2015 – The 12th annual Bet on Baylee event in honor of Baylee Thompson was held at the Perry County Fairgrounds in New Lexington, OH. The event was a jam packed event filled with fun games as well as some very impressive auction items! This year's event raised over $14,000!

April 18, 2015 – The EFL Walkathon: San Francisco Bay Area was held at its new location in the Bishop Ranch Business Park in San Ramon and raised $50,825!

April 24, 2015 – Girlfriend's Journey to a Cure Glow Run was held in Cordele, GA and was a unique and fun glow in the dark fun run!

April 25, 2015 – The EFL Walkathon: Cincinnati was held at Eden Park and raised $25,298!

April 25, 2015 – The EFL Walkathon: Pittsburgh got off to an unusually snowy start; but with weather clearing up by walk time, they were able to raise $69,918!

April 25, 2015 – The EFL Walkathon: St. Louis in Tower Grove Park raised $36,467!

May 8, 2015 – The Julia Joules Splash a thon was held at the Royal Oak Middle School in Royal Oak, MI in honor of Julia Joules and her EFL team!

May 9, 2015 - The EFL Walkathon: Evansville in Burdette Park raised $35,707!

May 16, 2015 - The 6th Annual Breylon Senn 5k Run/Walk/Stroll was held at the Tri County High School in Howard City, MI. The 5k benefitted the Breylon Senn Research Fund through the UMDF and raised $3,027!

May 17, 2015 – Mito Mania/Brielle's Beauty Extravaganza was held at Charlotte's Trove in Roaring Springs, PA. The event was a week-long Chinese Auction culminating in an exciting and successful fashion show! The event raised over $17,000!

May 17, 2015 – Olga Goldberger hosted an arboretum skin care party, a skin care line that focuses on your mitochondria! She raised $1,060!

May 30, 2015 – A bike run hosted by the Ford City Eagles Club in memory of Connor Hadden was held in Armstrong County, PA. The bikers rode to several different towns in the county before live music and raffles! The event raised over $4,000!

June 20, 2015 – U Kan Jam 4 UMDF, a Kan Jam tournament, was held at the Apex in Getzville, NY. The event also had games, raffles, and food!

June 13, 2015 – The annual Nicholas J. Torpey Butterfly Golf Classic was held at the Sycamore Hills Golf Course in Macomb, MI. The outing included a golf scramble and dinner, as well as raffles and auctions.

Greater Mito Open

June 13, 2015 – The 9th Annual Greater Mito Open ("Birdies for Brianna") was held at the Broadlands Golf Club in North Prairie, WI. The event held in memory of Brianna Dobke boasted a successful golf tournament and a beautiful dinner.

June 26, 2015 – The 3rd annual Thomas' Golf for a Cure was held at the River Bend Country Club in West Bridgewater, MA. The event not only had golf and other golf related competitions but a silent auction, 50/50 raffle, and a steak dinner!


Thomas' Golf for a Cure

Upcoming Events

August 1, 2015 – The Carter Lackey memorial celebration fun run will be held at the Waverly Glen park in Waverly, NY. The event will be a carnival style party with 5k, 10k, and half marathon walk/run/bike options. All proceeds benefit the Carter Lackey Research fund through the UMDF. For more information, contact Brittany Lackey at *brittanylackey23@gmail.com*.

August 1, 2015 – The Annual Baylee's Ball Bash softball tournament will be held in Cannelville, OH, at the Cannelville Softball Field. The entry fee per team is $120. For more information,please email Jody Thompson at *buff2506@hotmail.com*.

August 2, 2015 – Andrea Emery will be participating in a Leap for Life Sky Dive! benefitting her EFL Walkathon: Minnesota team Quinn's Quest.

August 3, 2015 - The Expressway BP and Dairy Queen are donating all proceeds from Dairy Queen and $.10 of every gallon of gas to the UMDF's Energy for Life Walkathon: Minnesota team Leo's Lions! For more information, contact Anne Simonsen at *anne.simonsen@umdf.org*.

August 12, 2015 – The Chuck E. Cheese in Troy, Michigan will be hosting Blake's Super Powered UMDF Fundraiser to benefit Blake's Super Powered Team of EFL Detroit! 15% of all sales from 3pm-9pm will benefit the UMDF and Blake's team! For more information, contact Danielle Diaz at *danidiaz98@yahoo.com*

August 30, 2015- ZANEBASH will be held at Jack's Beagle NoDa in Charlotte, NC. The event will have live music and a silent auction. For more information, contact *John Dungan at* *johndungan@gmail.com*.

September 18, 2015 – The second Annual Carlos Alberto Memorial Golf Outing will be held at the PipeStone Golf Club. There will be a golf outing and dinner. For more information, please contact Cristina Rue at *cristinarueg@gmail.com*.

September 19, 2015 – The Bryn Athyn 5k for the UMDF will take place at Bryn Athyn, PA. For more information, contact Weston Wadsworth at *Weston.wadsworth@ancss.org*.

September 26, 2015 – The second annual Jaxon's Warriors 5k, 10k, and little warrior fun run will take place at Highland Church of Christ. All proceeds will benefit the Jaxon Sharma Research Fund through the UMDF. For more information, please contact Amanda Sharma at *amandacole525@yahoo.com*.

October 9, 2015 – The Emma Frances Dalton Memorial Golf Tournament will be held at the Juniper Hill Golf Club in Northborough, MA. All proceeds will benefit the Emma Frances Dalton Research Fund through the UMDF. For more information, please contact William Dalton at *whd73@yahoo.com*.

October 11, 2015 – The UMDF is partnering with the Chicago Marathon to raise funds and awareness for mitochondrial disease. For more information, please contact Gary Moberly at *gmoberly@ymail.com*.

November 7, 2015 – The Mito Disc Golf Fundraiser will be held in Knoxville, TN as a part of the Knoxville Disc Golf Association. Contact Justin Forbes for more information at *justinforbez@yahoo.com*.

November 2015 – Coming soon! The annual "Fall into a Cure" wine-tasting event and auction! For updates and more information, visit *www.umdf.org/fallintoacure*

Central Regional Symposium

UT Health-The University Texas/ Children's Memorial Hermann Hospital, Houston, TX

February 12-13, 2016

Full CME day on Friday for medical professionals and morning meetings on Saturday for patients/families.

Course Chair: Mary Kay Koenig, MD
CME Chair: Bruce H. Cohen, MD

Southeast Regional Symposium

Duke University School of Medicine, Durham, NC

February 19-20, 2016

Full CME day on Friday for medical professionals and morning meetings on Saturday for patients/families.

Course Chairs: Dwight Koeberl, MD and Edward Smith, MD
CME Chair: Bruce H. Cohen, MD

Great Lakes Regional Symposium

Mayo Clinic, Rochester, MN

April/May 2016 – Dates TBA

Full CME day on Friday for medical professionals and morning meetings on Saturday for patients/families.

Course Chair: Linda Hasadsri, MD, PhD
CME Chair: Bruce H. Cohen, MD

Mitochondrial Medicine 2016: Seattle – National Symposium

DoubleTree by Hilton, Seattle, WA

Scientific Program - **June 15-18**
Family & LHON Program – **June 17-18**
Course Chair: Russell Saneto, DO, PhD
CME Chair: Bruce H. Cohen, MD

For details on all UMDF Symposia visit www.umdf.org/symposium in the coming months.

Upcoming EFL Walkathons

Our Spring 2015 Walk Season was amazing! THANK YOU to our 10 Energy for Life Walks for raising an outstanding $395,000!

From March through May, the walks experienced, wind, rain, sun and even SNOW! But through it all, we shared our stories, our love, laughs and our time for the cure.

THANK YOU TO:

Energy for Life: Atlanta
www.energyforlifewalk.org/atlanta
Energy for Life: Tampa Bay
www.energyforlifewalk.org/tampabay
Energy for Life: Cincinnati
www.energyforlifewalk.org/cincinnati
Energy for Life: St. Louis
www.energyforlifewalk.org/stlouis
Energy for Life: Nashville
www.energyforlifewalk.org/nashville
Energy for Life: Evansville
www.energyforlifewalk.org/evansville
Energy for Life: Pittsburgh
www.energyforlifewalk.org/pittsburgh
Energy for Life: Dallas
www.energyforlifewalk.org/dallasfortworth
Energy for Life: Shreveport/Bossier City
www.energyforlifewalk.org/sbc
Energy for Life: San Francisco Bay
www.energyforlifewalk.org/sanfrancisco

P.S. It's not too late to make a donation or to double your donation with a matching gift! Ask your employer if they match gifts!

We are looking forward to our Fall Walks! Our 17 fall walks have a goal of $932,625! Please help us reach those goals by supporting one of these amazing cities!

Saturday, Aug 8 – Minnesota
www.energyforlifewalk.org/minnesota
Saturday, Sep 12 – Indianapolis
www.energyforlifewalk.org/indianapolis
Saturday, Sep 12 - Western New York
www.energyforlifewalk.org/westernnewyork
Saturday, Sep 19 – Central Texas
www.energyforlifewalk.org/centraltexas
Saturday, Sep 19 – Detroit
www.energyforlifewalk.org/detroit
Saturday, Sep 19 – Del/Val
www.energyforlifewalk.org/delval
Saturday, Sep 19 – Kansas City
www.energyforlifewalk.org/kansascity
Saturday, Sep 19 – New Orleans
www.energyforlifewalk.org/neworleans
Sunday, Sep 20 – Chicago
www.energyforlifewalk.org/chicago
Saturday, Sep 26 – Omaha
www.energyforlifewalk.org/omaha
Saturday, Sep 26 – S. Wisconsin
www.energyforlifewalk.org/southernwisconsin
Saturday, Oct 3 – Akron
www.energyforlifewalk.org/akron
Sunday, Oct 4 – Seattle
www.energyforlifewalk.org/seattle
Saturday, Oct 17 – Birmingham
www.energyforlifewalk.org/birmingham
Saturday, Oct 17 – Charlotte
www.energyforlifewalk.org/charlotte
Saturday, Oct 24 – Columbus, GA
www.energyforlifewalk.org/columbusga
Saturday, Nov 14 – Fort Myers
www.energyforlifewalk.org/southwestflorida

UMDF national

News from the national office.

Are you looking for someone to connect with? Connect with a UMDF Ambassador, an affected individual/family member who would be happy to network with you. To get started, complete our contact form at http://secure.umdf.org/RegionalContact or call us toll-free at 1-888-317-8633.

Are you interested in volunteering? If yes, please visit www.umdf.org/volunteeropps!

WHAT UMDF REGION DO YOU LIVE IN?

Below are the UMDF's current regional coordinators and their email addresses.

We also encourage you to stay up to date in your region by visiting the regional webpages listed below!

Northeast Region 2
Nicole Shanter
Nicole@umdf.org
www.umdf.org/northeast

Southeast Region 3
Margaret Moore
Margaret.Moore@umdf.org
www.umdf.org/southeast

Great Lakes Region 4
Anne Simonsen
anne.simonsen@umdf.org
www.umdf.org/greatlakes

NETWORK WITH OTHERS

Inspire℠ together we're better℠

Inspire is a free online patient/caregiver community organized by discussion topics. Visit us at: *www.inspire.com/groups/united-mitochondrial-disease-foundation*

FOLLOW US ON

The UMDF Facebook Group is intended for networking with affected individuals and families. Join us at *https://www.facebook.com/#!/groupsumdfgroup.*

"Like" our page to stay updated! Simply type United Mitochondrial Disease Foundation in the search box on Facebook to find our page.

UNITED MITOCHONDRIAL DISEASE FOUNDATION STAFF

Executive Staff

Charles A. Mohan Jr.
CEO/Executive Director

Janet Owens
Executive Administrative Asst.

Philip Yeske, PhD
Science and Alliance Officer

UMDF Symposia

Kara Strittmatter
Meeting Event Director

Finance

Mark Campbell
Chief Financial Officer

Donna Nameth
Data Entry Manager

Barbara Cullaj
Administrative Assistant

Communications

Clifford Gorski
Director of Communications

Jeff Gamza
Multimedia Coordinator

Development & Member Services

Beth Whitehouse
Director of Development

Tania Hanscom
National Walk Manager

Cassie Franklin
Gifts Officer

Julie Hughes
Development Associate/ Grants

Tara Maziarz
Development Associate/ Social Media

Nicole Shanter
Regional Coordinator - Northeast

Margaret Moore
Regional Coordinator - Southeast

Anne Simonsen
Regional Coordinator - Great Lakes

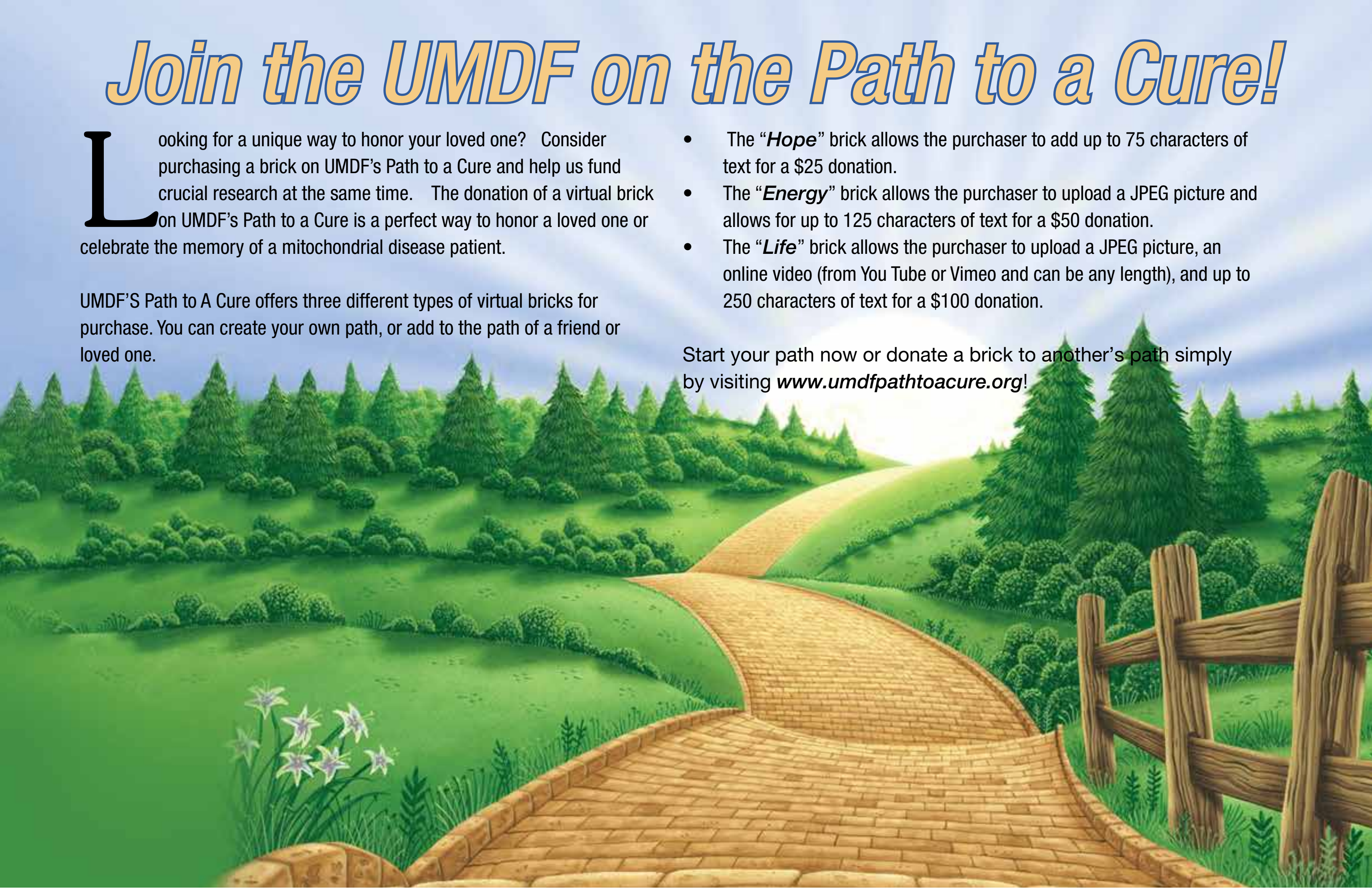
Leslie Heilman, JD
Development Associate/ Matching Gifts

UMDF MISSION

To promote research and education for the diagnosis, treatment and cure of mitochondrial disorders and to provide support to affected individuals and families.

The UMDF focuses on coordination, communication and collaboration.

We bring people and resources together to make an impact on diagnoses, treatments and a cure for mitochondrial disease.



Join the UMDF on the Path to a Cure!

Looking for a unique way to honor your loved one? Consider purchasing a brick on UMDF's Path to a Cure and help us fund crucial research at the same time. The donation of a virtual brick on UMDF's Path to a Cure is a perfect way to honor a loved one or celebrate the memory of a mitochondrial disease patient.

UMDF'S Path to A Cure offers three different types of virtual bricks for purchase. You can create your own path, or add to the path of a friend or loved one.

- The "***Hope***" brick allows the purchaser to add up to 75 characters of text for a $25 donation.
- The "***Energy***" brick allows the purchaser to upload a JPEG picture and allows for up to 125 characters of text for a $50 donation.
- The "***Life***" brick allows the purchaser to upload a JPEG picture, an online video (from You Tube or Vimeo and can be any length), and up to 250 characters of text for a $100 donation.

Start your path now or donate a brick to another's path simply by visiting ***www.umdfpathtoacure.org***!

UNITED
MITOCHONDRIAL
DISEASE
FOUNDATION®
HOPE. ENERGY. LIFE.
8085 Saltsburg Road, Suite 201
Pittsburgh, PA 15239

Donate today!
Every dollar counts.
Visit www.umdf.org.