UMDF
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UNITED MITOCHONDRIAL DISEASE FOUNDATION®
HOPE. ENERGY. LIFE.

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Jack Black Dances for UMDF

It is not unusual for longtime UMDF supporter Jack Black to make a splash and raise awareness for mitochondrial disease and donations for UMDF.

On Wednesday, January 27, 2016, Jack appeared on *The Ellen DeGeneres Show* to promote his upcoming film, *Kung Fu Panda 3*. During the course of the interview, Ellen told Jack he could raise up to $10,000 for a charity of his choice. Jack was instructed by Ellen that he would have to perform a dance based on the card that she held up. Jack chose the United Mitochondrial Disease Foundation as his charity of choice and did some really creative dancing to earn the money.

The donation was made by Chideo, through their partnership with *The Ellen DeGeneres Show*. Chideo is a content-driven fundraising platform that offers exclusive and original videos across a broad spectrum of entertainment, sports, business, music, fashion, food, design and culture in an effort to modernize our approach to charitable giving. Chideo's primary goal is to give celebrities, non-profits and brands an easy way to quickly drive awareness and raise funds for important causes through the creation of unique and entertaining content.

On behalf of all patients and families, UMDF extends our sincere thanks Jack Black, Ellen DeGeneres and Chideo.

UMDF education

Bringing the experts to your community.

With generous support from

UNITED MITOCHONDRIAL DISEASE FOUNDATION®

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From the Chairman

Raising awareness is not an easy thing to do. No matter how many times an "Energy for Life Walkathon" gets media coverage on your local television news to the mere mention of mitochondrial disease in the newspaper, getting valuable exposure usually takes weeks of planning, phone calls, and cajoling. Members and their families have tried for years to get mitochondrial disease mentioned on daytime and prime time television. In some cases, it has worked and worked well. Many have told us that they've written to television shows hoping to highlight mitochondrial disease in their family or child. They are often disappointed when they receive no response from the show or the network. But, in late January, it happened. And, it happened quickly.

We first learned about it through an alert on a newsfeed we monitor. The alert tipped us off that Jack Black danced on *The Ellen DeGeneres Show* for the United Mitochondrial Disease Foundation. Upon further investigation, we were able to track down the video of Ellen showing Jack a card depicting several dances. Jack got $1,000 for each dance done correctly, as judged by Ellen. It was very funny. In the end, Jack mentioned UMDF as his charity of choice and was presented a check in the amount of $10,000. Ellen's show partners with Chideo, a cause-entertainment company that is dedicated to changing the way cause and entertainment come together. Chideo provided the $10,000 donation to the UMDF. This isn't the first time Jack has named UMDF his charity of choice. He participated in *Who Wants to be a Millionaire?* and the "Moet & Chandon Red Carper Champagne Toast" for UMDF. We are grateful for his support, not only financially, but to our patients and families.

So how does a movie star's appearance on a daytime television show raise awareness? Before the show aired, we took a look at the traffic on the UMDF website. It was fairly normal at 1,000 visits per hour. When the show started airing in towns across the United States, website traffic to *umdf.org* increased to **10,000 hits an hour.** Website visitors were Googling the same question --"What is Mitochondrial Disease?" Think of all of the people who learned about our disease and our community because of Jack's appearance on *The Ellen DeGeneres Show*.

UMDF works to raise awareness daily. Think of the importance of a UMDF Grand Rounds presentation in your local hospital. Mitochondrial medicine experts, working with UMDF, have educated more than 400 physicians and clinicians in the past year through this program.

Imagine the awareness created when an Energy for Life Walk is held in your community. Think of the families served and the education and information shared by our families and with the general public. And finally, think about the awareness and education that is provided when our families gather in their hometown for a UMDF family meeting. For many, it is the first time that they are able to ask and have answered their questions by respected mitochondrial medical experts.

Jack's appearance was a great milestone for us on the road to awareness. But as you well know, we have a long path to travel before mitochondrial medicine is a household word.

Patrick Kelley, UMDF Chairman

Clinical Study on RTA 408 Capsules

Reata Pharmaceuticals is actively enrolling the MOTOR study, a placebo- controlled, multicenter clinical study of RTA 408 capsules in Mitochondrial Myopathy. For more information, go to **clinicaltrials.gov** and search "RTA 408 MOTOR".

MOTOR
a study in mitochondrial myopathy

UMDF Responds to NBC's *Chicago Med*

Like you, we were very disappointed in the NBC television program *Chicago Med* portrayed mitochondrial disease. We know the medical advisor for the program reached out to a top mitochondrial medical expert while the script for the show was in development. We believe the writers and producers had an opportunity to educate the public about mitochondrial disease and its difficult diagnosis. Instead, they chose to use mitochondrial disease as a basis for a medical child abuse storyline, portraying it as a "wastebasket diagnosis."

We want you to know that the UMDF reached out to NBC and the producers of the show to let them know how we all feel. We have provided the text of the letter below and hope that you reach out to NBC as well.

February 3, 2016

Mr. Robert Greenblatt
President
NBC Entertainment
30 Rockefeller Plaza
New York, NY., 10112

Mr. Dick Wolf
Wolf Films
100 Universal City Plaza
Universal City, CA 91608

Dear Mr. Greenblatt and Mr. Wolf:

This letter is being written on behalf of the parents and families impacted by mitochondrial disease to express our disappointment over how the episode "Reunion" on *Chicago Med*, televised on February 2, 2016, handled the unique issues that face those affected with this debilitating and often fatal disease.

While we understand your story line was "ripped from the headlines," the perception portrayed to any viewer of this show is that mitochondrial disease is not real and that every parent whose child needs emergency medical care for mitochondrial disease should be suspected of medical child abuse. That is false, totally disrespectful and potentially damaging to the thousands of patients and families who deal with mitochondrial disease on a daily basis. This is even more disappointing knowing that writers and producers of the program were briefed by a well-respected mitochondrial disease expert on the symptoms and diagnosis of mitochondrial disease prior to the taping of the episode.

While we are frustrated that you chose mitochondrial disease as the basis for an episode dealing with medical child abuse, we were particularly disturbed with the dialogue between the characters Dr. Natalie Manning and Dr. Will Halstead:

Manning: "She has mitochondrial disease…"
Halstead: "Mito? We really are talking fiction here."
Manning: "You don't think it exists?"
Halstead: "Always seemed like a wastebasket diagnosis to me. Doctor can't figure out what's really going wrong, says it's mito and calls it a day."

Mitochondrial disease is a real disease, with real symptoms, affecting real people, both children and adults. Mitochondrial disease is recognized by the medical profession and the National Institutes of Health (NIH). One in 4,000 people are born with mitochondrial disease. For many, the disease is incredibly debilitating and can be terminal.

We invite you to learn more about mitochondrial disease at www.umdf.org and www.mitoaction.org. As a public service, we urge you to add a disclaimer to the end of the episode identifying the fact that mitochondrial disease and its symptoms are real. In the disclaimer, we would like you to direct viewers to visit our websites for more information about this disease. This disclaimer will help alleviate any misconceptions and misinformation when the Reunion episode is rerun, syndicated or streamed online. We also hope that you will communicate this information on your website and social media channels.

We have worked hard for decades with physicians all over the world and families who are suffering to achieve greater understanding of mitochondrial disease and to fund research for a cure. Please consider using your program for good and helping the mitochondrial disease community educate the public and mitigate the damage that your program may have unintentionally caused.

Sincerely,

Charles A. Mohan, Jr.
CEO/Executive Director
UMDF

Patrick Kelley
Chairman of the Board
UMDF

Cristy Balcells, RN MSN
Executive Director
MitoAction

Sumit Parikh, MD
Chairman
UMDF Scientific and Medical Advisory Board

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UMDF

in the fight against Mitochondrial Disease.

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Mito Listed with DoD Again

The United Mitochondrial Disease Foundation is pleased to announce that mitochondrial disease have been listed again in the Department of Defense Peer-Reviewed Medical Research Program. Thanks to everyone who advocated for this critical inclusion during UMDF's 2015 Day on the Hill or by contacting your Congressman!

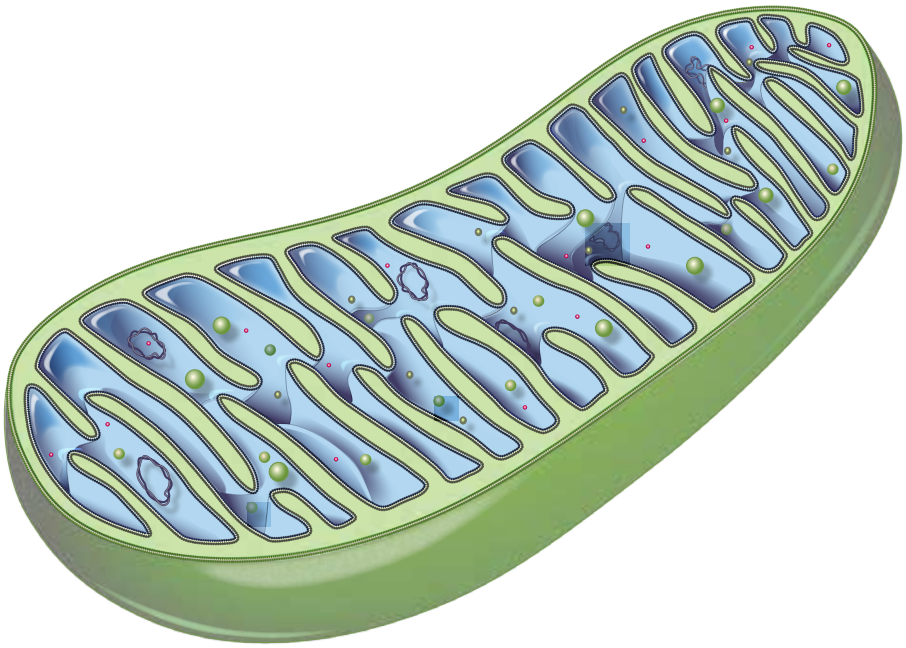
We asked for the inclusion of mitochondrial disease in this important appropriations measure, and they listened! Mitochondrial disease is listed along with more than 40 other diseases and illnesses eligible for research funding. This is the second year in a row that UMDF has been able to help the entire community by having mitochondrial disease included.

In addition to the inclusion, UMDF worked with the Congressional Directed Medical Research Program to identify affected individuals, family members, and care givers who would consider participating on a review panel for research projects. While participation is anonymous, we know that dozens of members from our community applied for the program and many were selected. UMDF also helped CDMRP recruit researchers to sit on the scientific review panel.

You are the power behind advocacy. Four diseases/conditions were eliminated from research funding this year. In order for mitochondrial disease to stay on the list for this important research funding, we need you to thank your Congressman for including mitochondrial disease and ask him/her to continue this important inclusion.

Stealth BioTherapeutics

is committed to the development of therapies for mitochondrial disease and proudly supports the advocacy efforts of the UMDF



To learn more about our work, please visit StealthBT.com or follow us on social media:

@StealthBT Stealth BioTherapeutics

Stealth BIOTHERAPEUTICS | Leading Mitochondrial Medicine

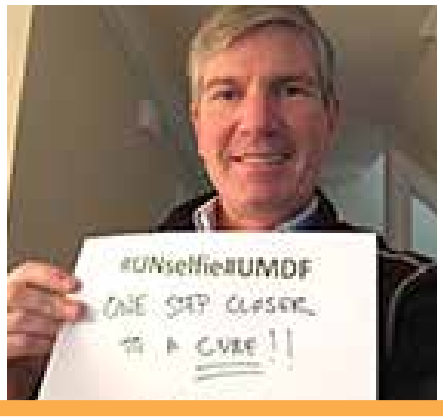
Success on Giving Tuesday

To the many who participated in giving and spreading the word on Giving Tuesday, December 1, 2015: **You're Amazing!**

Through over 175 gifts, you surpassed our Giving Tuesday goal of $30,000.

In one day, you raised $49,743 toward a cure!

Patrick Kelley *(right),* Chairman of UMDF's Board of Trustees, was proud to match your gifts with a commitment of $30,000, bringing our **Giving Tuesday 2015 total to $79,743**. One incredible day--one step closer to a cure. You made it happen. Thank you!



UMDF

members

Uplifting stories about our members

Kimberley's Story

Kimberley Anne Yochum was born on Easter Sunday, April 6, 1980 in Melbourne, Australia. Her parents, Inga and Terry Yochum, were living in Melbourne at the time while Terry (a chiropractic physician and radiologist) was helping to build a radiology department at the Royal Melbourne Institute of Technology.

In 1983, Kimberley had two large life changes, she became a big sister to Philip and then the entire Yochum family moved to Denver, Colorado. Kimberley was healthy as can be, only going to the doctor for a yearly physical. When the family moved to Denver in September of 1983, her parents noticed that Kimberley's skin was increasingly pale and that she had dark circles under her eyes. They assumed it was the harsh winters, which having lived in Australia her entire life, she was not used to. In February of 1984, her skin had not improved, so they took her to a doctor for bloodwork, with nothing showing on any test results. She was always pale and had dark circles, but she still did all the normal things children do. When she was 4, she became a big sister for the second time to Alisha and enrolled in dance classes. She could participate but would always come home complaining of soreness in her legs. She started going on field trips with the girl scouts and would come home complaining of extreme headaches. It seemed that her physical symptoms only came about after some form of exertion.

Kimberley's symptoms only progressed over time. When she was seven, she had a really bad case of exhaustion after a field trip. She threw up for days and had headaches. Six months passed by without another episode, and then it happened again. Dr. and Mrs. Yochum took their daughter to a neurological specialist who performed a brain scan, which showed no abnormalities. Kimberley went another six months after that episode with no problems. Later, it happened again, this time, much worse. She was hospitalized at the Children's Hospital where they performed another MRI which was abnormal. Kimberley had fluid on her brain similar to that of a stroke victim, however, she did not exhibit any other symptoms that accompany a stroke. Dr. Alan Seay ordered a spinal tap for Kimberley, and, after seeing the results, consulted with Dr. Carol Greene. Greene and Seay realized it was a metabolic diagnosis. Dr. Greene then consulted with Dr. DeVivo at Columbia, and they figured out what was causing her so many physical ailments. Kimberley was nine years old when she was diagnosed with MELAS. At the time, she was one of only 70 patients in the world who were known to have this specific mitochondrial dysfunction.

Dr. and Mrs. Yochum received some of the worst news parents can hear. They heard that their daughter will probably only live 5 more years. Thankfully, Kimberley defied the odds and now, at 35, continues to surpass them with her MELAS not progressing as fast as most. Between the ages of 9 and 25, Kimberley had about 50 seizures, went through periods of blindness and/or deafness, only to have her eyesight and hearing return days later. She was hospitalized multiple times. After one of her hospitalizations, where she had been in a coma for five days, her parents took her to see their friend, Craig Reese in Boulder, who specialized in chiropractic medicine and naturopathy. Mrs. Yochum took Kimberley to see Dr. Reese every week, where she received adjustments and was put on a specific nutrition regimen that seemed to help her.

Kimberley successfully graduated from Ranum High School with above average grades. Her school district provided an in-home tutor who taught Kimberley in the family kitchen. In her spare time, Kimberley enjoys old classic films and reading. Her favorite authors are Mary Higgins Clark and Jane Austen. She also loves music, especially ballad singers. She attends church once a week with her parents and lives just ten minutes away in an amazing care home.

The Yochum family spent a lot of time wondering, "Why us?", but their faith made them realize, "Why not us?" They realized they had all the capacities to not only help Kimberley, but for Kimberley and their family to help others. They are a family full of hope and want people to know that not every instance has to be a terminal diagnosis. With the right amount of support and understanding, there is always some hope.

Meet your UMDF Ambassador

The UMDF is about *coordination, communication and collaboration*; coordinating our efforts and resources, communicating our needs and abilities and forming collaborations to bring information, patients, medical professionals and resources together to enhance progress for treatments and cures.

UMDF Ambassadors are an essential part to those efforts. They are a resource for members seeking to gather information. They coordinate efforts with other members to make a difference in their local areas, and they are key collaborators with the UMDF regional and national staff.

If you would like more information about the UMDF Ambassador program, please contact us at connect@umdf.org.

Hello! My name is Becky Yu, and I live in the Tampa Bay Area. I serve as a UMDF Support Ambassador.

I was first suspected of having CPEO (Chronic Progressive External Ophthalmoplegia) in 2011. It is one of the more common mitochondrial myopathies. Since that time, my life has most certainly become bittersweet. In 2013, I was officially diagnosed with genetic testing via eyelid muscle biopsy. While it causes a number of struggles for me, I have made it a goal to learn and understand all that I can about mitochondrial diseases. My hope is that this knowledge will allow me to help others with their journey.

Due to living life with a mitochondrial disease, I also have grown to have more compassion and wisdom in dealing with others. I have met and spoken with (via email, phone, Skype and Facebook), the most incredible people from all over the world. Mito is about making adjustments in our daily lives. My purpose is to help others to begin or continue navigating through the mysterious mito maze and listen to their struggles and questions. I only am able to do this because of the wonderful contacts I have and all the people who listened to me and helped me in my journey. I'm so thankful for this wonderful opportunity.

The UMDF Staff says:

"Becky always makes herself available to chat with others and has been a great asset for those wanting to share experiences. Becky once shared with me that she feels so much energy listening to others and helping them in their situation. That filled my heart to know our ambassadors find purpose and their own support connecting to families and affected individuals. Becky is a true gift to our families."

Anne Simonsen, UMDF Regional Coordinator - Great Lakes

"Volunteers don't necessarily have the time, but they have the heart. Becky Yu is a great example of this as she is a great resource and always available for others questions and concerns that are affected by mitochondrial disease."

Julie Hughes, UMDF Development Associate

"I feel blessed to have a support Ambassador like Becky Yu in the Southeast Region. While she joined us 'officially' as a support ambassador focusing on adult support in September of 2014, she was already helping people in our community that were in need for at least a year! Becky is the first person I think of when I meet a newly diagnosed adult. Becky is so supportive, so kind and honest with these folks, focusing on positive next steps. She talks to several newly diagnosed members a month, and helps out some of the veterans in our community as well.

You would think that would be all one person could do... but not for Becky! She actually holds a committee position on our EFL Tampa Bay Committee, helping to make phone calls to our affected individuals and families in the Tampa Bay Community. Thank you, Becky!!

Our Volunteers are such an important part of the work we do here at the UMDF!! As I have told Becky many times (and yes I know it is corny)…There wouldn't be a UMDF without YU;) Our most valuable resource? Our People!"

Margaret Moore, UMDF Regional Coordinator - Southeast

Team Giuliana - A POWERHOUSE for EFL: Delaware Valley

Less than two years ago, Gianna Foglia and husband Enzo D'Aria joined the Energy for Life Walkathon in Del/Val with their team, Team Giuliana. At the time, they walked to connect and meet with other families.

Now, after their second year, they realize it's also about raising awareness and funds for mitochondrial disease research. Every person that Gianna asks to join their team not only learns about mitochondrial diseases, they also show support to Gianna and her family. That support is one of Gianna's favorite things about the Energy for Life Walkathon.

Team Giuliana has had a major impact on funding research. Just after 2 years, their team has raised $45,229! When asked "How do you do it?", Gianna laughs and says, "A lot of support from family and friends". Gianna asks her friends and family to sign up to walk on their team. She also encourages them to ask their friends and family to support their team by walking with them and/or making a donation. Gianna's dad, Michael, not only sponsors the walk through his company, Rahway Steel Drum, but also asks his friends and colleagues to support his granddaughter and her team. Team Giuliana also raises funds for their team through company matches. Just this past year alone, almost $6,000 was added to their total in matching gifts! Amazing!

One of the best ways they ask their friends and family is through the Email Center on the Energy for Life Walkathon website. This past year, they sent over 900 emails. Team Giuliana, you're a great inspiration to so many teams! Keep up the great work!

There are so many amazing "powerhouse" teams throughout our 29 walkathons. Each and every team is amazing, and we honor each of you!

Our challenge to you, as we enter the Spring Walk Season, is to have fun, ask as many people as possible to join your team and don't forget about those matching gifts!

Double Your Gift to the UMDF!

Be a matching champion! The UMDF makes it easy for our families and donors to access matching dollars from their employers. Simply visit www.doublethedonation.com/umdf to start the process. Requesting an employer matching gift is an easy process and can make a significant impact to your UMDF donations. We'd like to introduce and share examples from two "Matching Champions" who have made a difference through coordinating, communicating and collaborating with their co-workers.

KIM MULLINS
EVANSVILLE, INDIANA

As the Team Captain for Team Homie at the Energy for Life Walkathon-Indianapolis, Kim Mullins rallied her co-workers at Springleaf Financial Services to donate and submit employee matching gift requests. Kim championed matching by helping her co-workers.

"I sent my co-workers all the information they needed to fill out for the matching funds from our company. By providing them the necessary information, it almost guarantees UMDF will receive the additional donation," she said.

Kim's advice is to ask your HR or community relations representative about your companies matching program. Many companies have a matching fund policy that isn't always publicized or their employees aren't aware of it. It doesn't hurt to ask your employer, and it's like receiving free money! Kim and her co-workers were able to raise an additional $755 for Team Homie, in honor of Kim's niece Abigail Goerges, and were the top fundraising team for Indianapolis raising $6,865!

THE DIAZ FAMILY
WARREN, MICHIGAN

2015 was the first year the Diaz family participated in the Energy for Life Walkathon-Detroit. With their focus to access The GE Foundation's Matching Gift program, Blake's Super Powered Team raised $8,209, which included over $2,000 in matching funds. Blake's father, Will, scheduled a meeting with the top executive in his department.

"I was able to explain the UMDF, Energy for Life Walkathon, and Blake's story," Will said. "I was also very fortunate to have this executive sponsor to present to her peers at an executive conference which generated large donations from other business units."

Will posted information on the company intranet and break rooms, including a QR scan code to Blake's team page and how to use the company matching site. Donors to Blake's team were immediately sent a thank you and instructions for the GE matching site. For friends and family outside of GE, a link was sent to help them find out if their company matched funds. The Diaz family provided a second thank you to GE matching fund donors that increased awareness throughout the organization.

"A UMDF pin was given to each person who would forward their completed Foundation matching email," he said.

Double Your Donation!

Double the Donation's *Top 10 Companies with Matching Gift Programs*:

- **Boeing**
- **British Petroleum**
- **CarMax**
- **ExxonMobil**
- **Gap**
- **General Electric**
- **Johnson & Johnson**
- **Microsoft**
- **Soros Management Fund**
- **State Street Corporation**

If you see your company on this list – be sure to check to see if YOUR donation can go the extra mile!

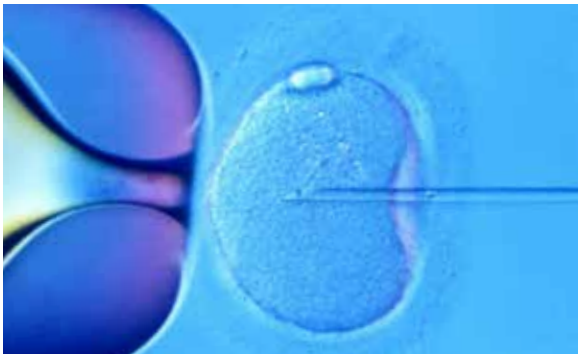
If not, just go to www.doublethedonation.com/umdf and check to see if your company is on the list!

Mitochondrial Replacement Therapy- We've come so far, but...

by Phil Yeske, PhD, UMDF Science & Alliance Officer

Most likely you have heard of mitochondrial replacement therapy (MRT), but perhaps under media-adopted names like "3-Parent Babies" or "Designer Babies." These misnomers are poor representations of scientific advances that afford a subset of our disease community the opportunity to have genetically-related children with significantly reduced risk of passing along a mitochondrial DNA mutation from mother to child.

First allow me to share a bit of scientific background, although most of you are probably quite familiar with this information. All humans have two genomes, or repositories, of genetic information stored within DNA molecules. The largest, and most commonly thought of repository is our nuclear genome. The important regions within nuclear DNA make up about 99.8% of all genetic material and are responsible for encoding proteins that control what makes each of us who we are- hair and eye color, intelligence, personality, etc. The remaining 0.2% or so of human genes are located within mitochondria, and their major role is to ensure our cells have enough energy to function properly, although nuclear DNA helps with that too.



Mitochondrial diseases can result from mutations (errors) in either genome, but MRT is only of potential benefit to women with a mutation in their mitochondrial DNA. Since mitochondrial genomes are passed solely from mother to child, mutations in the mitochondrial DNA are a significant reproductive problem. Using MRT, in vitro fertilization clinicians would replace the faulty mitochondria in a woman's egg or embryo with healthy donor mitochondria from another woman, thus prohibiting the transmission of disease-causing genetic information. Importantly, no changes are made to the nuclear DNA provided by mother and partner.

Sounds great, right? So why is MRT controversial? By replacing even a small bit of the genetic information in a cell we (society) would be introducing something called a germline modification. This means the genetic change made during MRT would be permanent and all future generations would inherit this change. For some people this is ethically troubling, and viewed as opening the door to manipulating genomes for less-altruistic purposes down the road. But just to be clear- MRT in the context of mitochondrial disease is 100% about the prevention of disease transmission from mother to child.

As a society we have wrangled with medical ethics on many topics, and this one will be no different in that there will never be a consensus. There are risks and benefits in any medical procedure. In the United Kingdom a long and substantial public debate took place over the past several years that resulted in legislative approval for clinical usage of MRT. The first licenses are being granted to clinical centers, and it is expected that a first child will be born later this year as the result of MRT. The situation in the United States is less clear.

In 2014 the Food and Drug Administration (FDA) convened a panel of experts to review the status of MRT in response to interest from US-based research centers in beginning clinical trials to test the safety and efficacy of MRT techniques. Clinical trials are the FDA's process for rigorously testing a new medical technique or therapeutic, and so these initial trials would yield critical data for the FDA to consider before ever allowing broad clinical application of MRT. At that time the FDA felt it needed more information, and so asked the Institute of Medicine (IOM, a non-government affiliated non-profit made up of leading researchers and clinicians) to more thoroughly review the ethical, social and policy considerations of MRT. The IOM issued their report in February 2016 and concluded that based on their comprehensive investigation that it is ethically permissible for the FDA to move forward with oversight of MRT-focused clinical trials. The IOM recommended certain initial limitations, including that only women with a proven, severe mitochondrial DNA-based form of mitochondrial disease should be included, and that only male embryos should be utilized (initially) so as to eliminate the risks associated with passing along the germline modifications described above to succeeding generations of offspring.

The UMDF has taken the position that MRT, when proven safe and efficacious via clinical trials, should be an option made available to women in the US with a mitochondrial DNA disease that want to have a genetically-related child. The IOM report seemingly brought our community to the cusp of such trials in the US, but a legislative twist will likely delay the process for at least a year…if not more. In the fall of 2015 Congress passed an omnibus spending bill for the current fiscal year that included language prohibiting the FDA from even accepting applications based on gene editing, and MRT falls under that broad category. Because the restrictive language only covers the current fiscal year it is possible it could be changed or removed altogether in a future spending bill, but no one can say what will happen- especially in a year in which the presidential office will also change.

It is important to remember that other techniques, such as pre-gestational diagnosis or adoption, remain options for women affected by mitochondrial DNA-based mitochondrial disease to consider. There will likely be a long road of advocacy and lobbying ahead in order for MRT to reach approved clinical application in the US, and UMDF will remain an important voice of the patient in this process in which you are encouraged to participate as well. Please visit our webpage dedicated to this topic in order to stay abreast of the latest developments: www.umdf.org/replacementtherapy.

AACT Update

On behalf of the UMDF Adult Advisory Council Team, we want to wish you good health and happiness throughout the New Year, and may it be a year with many blessings.

2016 IS AN EXCITING AND SPECIAL YEAR

UMDF is celebrating its 20th anniversary and AACT's 10th anniversary - what milestones! Below, we have highlighted some of the many special events taking place across the country. Lots happening this year, and we hope you can join us along the way.

UMDF MITOCHONDRIAL MEDICINE 2016

For the first time, UMDF will host its annual national Symposium in the beautiful Pacific Northwest - Seattle, Washington! In addition to a number of Adult and Young Adult Sessions, the Family Program is packed with many wide ranging Sessions, Workshops and networking opportunities.

Please Note… The 10th Annual Adult Gathering will be on **Thursday** evening from 6:30 pm to 8:00 pm. Young Adults are welcome and we look forward to kicking off the Symposium with you!

Registration is now open at *www.umdf.org/symposium.*

Use registration code: FAM1605.

Lastly, UMDF is currently accepting LEAP nominations that will be awarded at the Friday Night Banquet to one special Adult or Young Adult living courageously with mitochondrial disease. To read more about the LEAP award and to submit a nomination by April 30, 2016 please go to: *https://www.surveymonkey.com/s/2016LEAPAward.*

UMDF REGIONAL SYMPOSIA

UMDF is hosting four one-day Regional Symposia! It's a great day to be together to exchange information, to network with some of the top mito doctors in the field, and to meet others fighting this disease.

Central Region: Houston, Texas
Saturday, February 13

Southeast Region: Durham, North Carolina
Saturday, February 20

Great Lakes Region: Rochester, Minnesota
Saturday, May 21

Northeast Region: Philadelphia, PA
Saturday, October 29

Please note: A Full CME Day is available on the Friday preceding the above as part of the Regional Symposium for clinicians – encourage your doctors, nurses, therapists, etc., to attend!

For more information and to register go to: *www.umdf.org/regionalsymposia.*

AACT WEBINARS

In conjunction with UMDF, we are excited to announce we will host two Webinars this year (dates and times TBA)!

The first Webinar will be in July and the topic will be: *New Treatment & Research Updates*.

The second Webinar will be in October. The topic is open and we welcome your suggestions. Please keep in mind, the topic should be for the general Adult and Young Adult population. If you would like to submit a topic recommendation, please send to: *news@umdf.org.*

ENERGY FOR LIFE WALKATHONS

The Energy for Life Walkathon is UMDF's signature fundraising event to help raise awareness and to support vital research grants which, one day, will lead to finding treatments and cures for mitochondrial disease. This year, over two dozen Walkathons will take place across the country in beautiful venues. Please form or join a Team and become a part of the path toward a cure. To view all EFL Walkathons and to form or join a Team, go to the UMDF homepage at: *www.umdf.org* and click on the EFL Walkathon box on the bottom right-side.

Once again, happy New Year and we look forward to seeing you soon!

TO UMDF…

On behalf of AACT and the community we represent and serve, a very happy 20th anniversary and a big "Thank You" for all you have done - and continue to do - for all of us affected by mitochondrial disease!

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 am 57 years of age and was diagnosed with MELAS about 10 years ago. I recently saw my neurologist and got the results of my latest MRI. It showed quite a lot of white brain matter. When I asked what caused it, I was told he wasn't able to tell. Some form lesions, others form scar tissue and yet still possibly dead cells. I don't know the extent of the damage and was not given a percentage when asked. Is this normal? Also, I was told that in all the cases he had ever heard or read about, all the patients went deaf. I am not sure how much he really knows about this disease. He says he is quite educated but I have some doubts. He prescribed me a medication for headaches that warns to not take if you have a metabolic acidosis. It is for migraines and seizures. What am I to do? I distinctly asked him if it would cause my MELAS any problems and he said no. I am obviously concerned. Any help? I believe the medication is topiramate.

Russell Saneto, DO, PhD

A: I am sorry to hear about your disease. MELAS is not a very friendly disease and much to our regret is progressive and treatments are limited. I guess the first question is to ask if you have the common m.3243 mutation and do you know your heteroplasmy. Since metabolic strokes are noted in all cases of MELAS (mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes), have you had many strokes? Also, are you on L-arginine of L-citruliine for prophylaxis? There are some patients that we can control the headaches with the mentioned medications and some we have to add headache medication. White matter changes in the brain are not uncommon and vary from patient to patient. When we wrote our paper on MRI findings and mitochondrial disease, there was no particular pattern that was seen. If you are deficient in central folate, than using folinic acid might be helpful to slow the loss of white matter (this is the material that surrounds the neuron axons to isolate and speed communication between neurons in the brain and muscles outside the brain). The finding of diabetes (usually begins with type II diabetes) and hearing loss is a common finding of the m.3243 mutation. You do need to keep a close watch on the diabetes as this is treatable with medications and if treated, will really go a long way to keep you at your best health. You also should see a cardiologist and make sure your heart is doing okay, often they might request an EKG or ECHO of your heart. Peripheral neuropathy, or loss of feeling in your legs/feet is not uncommon-usually starts as having the feelings of "pins and needles" is also a common finding. If this is happening, then medication might be helpful for this condition.

The use of medications is pretty physician specific. I usually do not use topiramate as I do not like the side effects in my mitochondrial patient, but certainly I know physicians who use it. But, if you find that your having trouble finding the proper words to use, it may not be the best medication for your headaches. Also, topiramate is associated with glaucoma in about 0.5% of patients who take it, so you will need eye check ups. Again, your health is so important, do your homework well and see someone who deals with mitochondrial diseases.
Russell Saneto, DO, PhD

Q: I have read conflicting articles on whether amitryptiline helps or hurts mitochondria. Which is it?

Sumit Parikh, MD

A: There are many medications that can theoretically be toxic to mitochondria (as seen in animal studies or cell research) that are often tolerated in mitochondrial disease patients. Amitriptyline is generally tolerated by mitochondrial patients and does not exacerbate mitochondrial disease as far as we know. The actual list of medications that truly need to be avoided by most mitochondrial patients is pretty short - and there are truly no absolutely contraindicated medications - in that if there are no other treatment options - we may need to use these as well - though with much caution. - *Sumit Parikh, MD*

Q: My daughter has seizures, muscle weakness, tremors, and episodes of high ammonia. She also has lung issues, constant sickness, nausea, and tiredness. We recently had testing done that showed a 15q11.2bp1-bp2 deletion syndrome and a homozygous variant detected on the UQCRC2 gene. We have had a normal muscle biopsy, and skin biopsy. Does this rule out mitochondrial disease? Our doctors are at a loss and have no further answers for us.

Mark Tarnopolsky, MD

A: The UQCRC2 is a mitochondrial gene - it depends on the variant if it is pathogenic or not - there can also be secondary mitochondrial enzyme deficiencies in deletion syndromes.
- Mark Tarnopolsky, MD, PhD, FRCP(C)

Q: Hello. My son is 11 and has been seeing a geneticist for the past year originally for abnormal organic acids and acylcarnitine profiles. We are awaiting gene tests to help us understand what's going on. Meanwhile, his CoQ10 levels are low and we will begin supplementing. From what I read, there are primary deficiencies and secondary for both the disorders my son is showing (the first being fatty acid oxidation). How does that work and does that mean you don't really have mito if you have a secondary form of either of these? Thank you.

Marni Fak, MD

A: Primary fatty acid oxidation disorders are metabolic disorders caused by mutations in nuclear genes that encode enzymes involved in the fatty acid oxidation process that burns fat within mitochondria. These present with a variety of symptoms from sudden death to liver or heart involvement, and require specific dietary and medical management. They can be suspected based on accumulation of specific intermediate metabolites on plasma acyl-carnitine profiles and urine organic acid profiles. While these disorders do involve a key process occurring in mitochondria, they do not directly impair the mitochondrial respiratory chain (or electron transport chain) that are often considered to be "primary mitochondrial diseases". Many mitochondrial respiratory chain diseases do cause secondary deficiencies in the cells ability to burn fat through the fatty acid oxidation pathway, and so abnormalities can be seen on metabolic screening lab investigations in blood or urine that are not caused by a genetic defect within the fatty acid oxidation pathway.

Primary coenzyme Q10 deficiencies result from mutations in nuclear genes that encode one of the biosynthetic enzymes necessary to generate coenzyme Q within the cell. They are very variable in presentation from myopathy to neurologic or renal problems. They are exceedingly rare, although in some cases can be very responsive to coenzyme Q10 supplementation. Much more commonly, individuals with mitochondrial respiratory chain deficiencies due to other genetic disorders can develop a secondary Coenzyme Q10 deficiency in their tissues (mostly tested in muscle), which is why so many patients with respiratory chain diseases are given coenzyme Q10 supplementation. It is not clear that blood (plasma) levels of coenzyme Q10 really correlate to what is happening in a person's high-energy tissues (muscle, nerve, liver, etc), which is why it is recommended to measure white blood cell (leukocyte) coenzyme Q10 levels that are thought to be a better indication of what intracellular levels may be. Metabolic screening lab studies such as organic acid profiles and acylcarnitine profiles would not be useful to specifically detect or confirm coenzyme Q10 deficiency; genetic diagnostic testing would be the first line choice to find the cause for a low tissue or cellular coenzyme Q10 level. Coenzyme Q10 supplementation is fraught with practical challenges, including mainly that it is generally very poorly absorbed within cells and therefore some forms of supplementation do not improve tissue coenzyme Q levels even if it seems the "plasma" levels are higher. A reduced form of coenzyme Q10 known as ubiquinol (not the more commonly available ubquinone) is thought to be more bioavailable in this regard.. - *Marni J. Falk, MD*

Q: In your opinion, what is the relationship, if any, between CFS/ME and Mitochondrial Disease?.

Fran D. Kendall, MD

A: There are a number of papers and studies that indicate that patients with chronic fatigue syndrome can have mitochondrial dysfunction. Similar defects in oxidative metabolism in other disorders such as bipolar disease, multiple sclerosis, Parkinson's disease, schizophrenia, and depression have been reported.

Although the majority of patients with multiple sclerosis appear to have widespread mito dysfunction and impaired ATP production, a recent paper by Morris and Berk in BMC medicine, April 2015, indicate that mitochondrial impairment is inconsistently found in patients with other disorders including chronic fatigue syndrome indicating multiple other pathophysiologic processes (causes) are likely involved in those diseases.

In addition, mitochondrial disease and dysfunction are utilized differently by mitochondrial experts who typically reserve the classification of mitochondrial disease for those patients who are found to have gene mutations in mitochondrial genes or fall neatly into a well described clinical phenotype such as Leigh disease and not for those diseases that may secondarily impact or impair mitochondrial functionality through a mechanism other than an alteration in mitochondrial gene.

In summary, then, chronic fatigue syndrome has been associated with mitochondrial dysfunction based on the number of studies. However, while there may be some impairment in mitochondrial function, most of these patients are not going to fall under the mitochondrial disease diagnostic heading unless their fatigue is a component of a larger clinical picture and is confirmed utilizing standard mitochondrial disease diagnostic tools. - *Fran Kendall, MD*

You can quickly and easily find "Ask the Mito Doc" Q/A's 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.

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Fundraisers Benefitting the UMDF

October 2015 Leslie Dacosta and Kean University in New Jersey hosted a successful Lightbulb Campaign! They raised over $50 bringing awareness to their campus community.

October 28, 2015 Mount Pleasant Elementary School (the Livingston School) hosted a fundraising walk at their school in Mount Pleasant, NJ,raising over $3,000!

October 29, 2015 Brady's Bunch held a fun and creative paint night to fundraise for their research fund and upcoming 5k walk. They raised over $200.

October 30, 2015 BNY Mellon hosted a jeans day, and the UMDF was their charity of choice! BNY Mellon matched all donations and raised over $1000.

November 7, 2015 Carly Kindbom hosted an exciting and unique event, a Bar Crawl, in Philadelphia, PA that raised over $400!

November 7, 2015 The annual Fall into a Cure wine tasting and auction was held in Lovetsville, VA. The event was beautiful with a record number of auction items this year. Fun was had all around and they raised a whopping $31,070!

November 14, 2015 Meghan Shields, Justin Forbes, and the Knoxville Disc Golf Association hosted a disc golf tournament in Knoxville, TN. The first time event was wildly successful, raising over $1300!

December 1, 2015 Ryan and Gaby Duncan held a fundraiser in Texas in honor of their friend Derek Bair. Ryan's co-workers donated money to have him cut his hair! They raised over $5000!

December 1, 2015 Francesca Esquenazi held a bake sale at the Riot Games workplace in San Francisco on Giving Tuesday and raised over $1000.

December 2, 2015 Stonehedge Elementary school in Camillus, NY, held a walk in honor of their friend and classmate, Molly. The elementary school started a Path to a Cure in Molly's honor and raised over $1200.

December 5, 2015 The second annual Carter's Christmas vendor sale was held once again at the Sayre Elks Club in Sayre, PA. The benefit hosted a number of craft vendors, breakfast with Santa, and fun games for the kids! This year they raised over $3,000!

December 6, 2015 The annual Bowling for Mito in Delaware was held in Dover, DE. A pleasant time was had by all who attended and raised over $1000!

The UMDF will offer a regional symposium at the Mayo Clinic in Rochester, Minnesota on May 20-21, 2016.

Upcoming Events

February 14, 2016 The New Jersey Devils hockey team will partner with UMDF as their charity for this game. For more information, or for tickets, contact Tara at *TaraM@umdf.org*

March 19, 2016 Madison's March Mile and Pup Parade will be held at Gregory Hill Park in Smyrna, TN. For more information, please contact Kelly Russell at *russellk@rcschools.net*

April 2, 2016 The annual Jackson Culley Mito What? 5k will be held in Millington, TN. For more information, contact Angie Nunn at *angienunn73@gmail.com*

April 9, 2016 The first Brady's Bunch 5k and Fun Run will be held in Vincennes, IN, to benefit the Brady Sterchi research fund through the UMDF. For more information, please contact Jamie Sterchi at *yamie00@hotmail.com*.

April 9 , 2016 The Mito Dawgs of Athens, GA, will host a Fun Run and Silent auction! For more information, please contact Emily Lyons at *mitodawgs@gmail.com*.

April 23, 2016 The 13th Annual Bet on Baylee Casino and Auction Night will be held at the Perry County Fairgrounds in New Lexington, OH! The event promises to be even more fun (if possible) than last year! For more information, please contact Jody Thompson at *buff2506@hotmail.com*

May 7, 2016 A Dinner in the Dark will be held in Northfield, MN to benefit the LHON Project through the UMDF. For more information, contact Tara at *TaraM@umdf.org*

June 4, 2016 The annual Greater Mito Open (Birdies for Brianna) will be held at the Broadlands Golf Club in North Prairie, WI. For more information, please contact Dave Dobke or Karen Loftus at *ddobke@ball.com* or *Milwaukee_mitoans@yahoo.com*.

June 11, 2016 The annual Nicholas J. Torpey Butterfly Classic will be held at the Sycamore Hills Golf Course in Macomb, MI. For more information, please contact Jennifer Ruhana-Smerek at *jruhana31@hotmail.com.*

Upcoming Symposia

Central Regional Symposium
UT Health/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
www.umdf.org/symposium/central

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
www.umdf.org/symposium/southeast

Great Lakes Regional Symposium
Mayo Clinic, Rochester, MN
May 20-21, 2016
Full CME day on Friday for medical professionals and morning meetings on Saturday for patients/families.
Course Co-Chairs: Ralitza Gavrilova, MD and Marc Patterson, MD
CME Chair: Linda Hasadsri, MD, PhD
www.umdf.org/symposium/greatlakes

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
Reserve your hotel room now!
www.umdf.org/symposium

For details on all UMDF Symposia visit www.umdf.org/symposium.

Upcoming EFL Walkathons

Spring is coming upon us – which brings perfect weather for an Energy for Life Walkathon! Our amazing volunteer planning committees are busy planning 11 Energy for Life Walkathons in April and May! We have a goal to raise $512,160 this spring, and we need YOU to help us hit that goal! Join us in one of these amazing cities:

Saturday, April 9 – San Francisco Bay Area
www.energyforlifewalk.org/sanfrancisco
Saturday, April 16 – Atlanta
www.energyforlifewalk.org/atlanta
Sunday, April 17 – Houston
www.energyforlifewalk.org/houston
Saturday, April 23 - Tampa Bay
www.energyforlifewalk.org/tampabay
Saturday, April 30 - Dallas/Fort Worth
www.energyforlifewalk.org/dallasforthworth
Saturday, April 30 – St. Louis
www.energyforlifewalk.org/stlouis
Saturday, May 1 – New England
www.energyforlifewalk.org/newengland
Saturday, May 7 – Evansville
www.energyforlifewalk.org/evansville
Saturday, May 14 – Nashville
www.energyforlifewalk.org/nashville
Saturday, May 14 – Cincinnati
www.energyforlifewalk.org/cincinnati
Saturday, May 21 – Pittsburgh
www.energyforlifewalk.org/pittsburgh
TBD – Shreveport/Bossier City
www.energyforlifewalk.org/sbc

SAVE THE DATE!:

Fall will be here before you know it! We've started to get some dates on the books for the Fall! Save these dates and keep checking the website for updates!!

Saturday, Aug 20 - Minnesota
Saturday, Sep 3 - Omaha
Saturday, Sep 10 - Indianapolis
Saturday, Sep 17 – Detroit
Saturday, Sep 17 – Kansas City
Saturday, Sep 17 - Western New York
Sunday, Sep 18 – Chicago
Saturday, Sep 24 – Southern Wisconsin
Sunday, Oct 2 – Seattle
Saturday, Oct 15 – Charlotte
TBD - Central Texas
TBD - New Orleans
TBD - Delaware Valley
TBD - Akron
TBD - Birmingham
TBD - Columbus, GA
TBD - Southwest Florida

YOU ARE OUR ENERGY!

Want to join one of our planning committees? We want to talk to you! Call 888-317-8633 or email events@umdf.org!

More Ways to Give

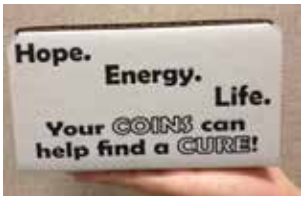
Activate Your Mitochondria!

Run a marathon, skydive, compete in a triathlon! Run, walk, swim, ski, jump! Get going, get moving, get ACTIVE - and support those who cannot! Participate in your favorite physical activity, or challenge yourself to find a new favorite, all while raising funds for a cure! *www.umdf.org/activate*

Path to a Cure

UMDF's Path to a Cure symbolizes our commitment to fund research that will bring us closer to a potential cure. A donation of a brick is a perfect way to honor a loved one or celebrate the memory of a mitochondrial disease patient. Your brick could commemorate a significant life event, like a wedding, birthday, engagement, anniversary, birth and more. Visit *www.umdfpathtoacure.org*

Coins for a Cure



Place coin collection containers in your school, church or local business for a designated period of time. No amount is too small to propel us down the path towards a cure.

Light Bulb Campaign

The UMDF will provide you with light bulb paper cutouts that can be sold for a minimum donation of one dollar at schools, local restaurants or businesses and placed on a designated wall or window to raise awareness.

Family Research Funds

UMDF Research Funds are established by families as a way to honor or memorialize a loved one affected by mitochondrial disease. Your donation to any of our existing funds ensures that the world's top mitochondrial scientists are receiving the support they need to perform breakthrough research. UMDF would be happy to work with you on establishing a research fund in honor/memory of your loved one.

Do you want to host a fundraiser for the UMDF?
Contact UMDF Special Events at
events@umdf.org for more information!

Online Shopping and Search Options

AmazonSmile
AmazonSmile is a simple and automatic way for you to support the UMDF every time you shop, at no cost to you. You'll find the exact same low prices, vast selection and convenient shopping experience as amazon.com with the added bonus that Amazon will donate a 0.5% of the purchase price to the UMDF! Visit *smile.amazon.com* and select UMDF to receive donations from eligible purchases before you begin shopping.

GoodSearch.com
Set your default browser to GoodSearch.com and every time you search the internet at GoodSearch.com, the UMDF earns $ .01. The more you search, the more dollars we earn to find better treatments and cures. Type *http://www.goodsearch.com/ ?charityid=806412* into your browser to contribute to the UMDF.

iGive.com
The *www.iGive.com* feature on the UMDF website allows you to shop online and have part of your purchases donated to the UMDF. Register at *iGive.com* and support the UMDF at the same time.

FundPhotos
FundPhotos is an online photo printing and personalized photo merchandise retailer dedicated to supporting community groups and non-profit organizations just like the UMDF. FundPhotos will donate 10% of the purchase of all prints and personalized photo merchandise to the UMDF. Join for free at *www.FundPhotos.com*. Create photo galleries that you can share with friends and family. Select the UMDF as your photo gallery's contribution recipient and when you or anyone you share your photos with, makes a purchase, the UMDF will receive your 10% donation!

AWARDS

Katie Parsons (right) accepts the 2015 Heartstrings Award from Patrick Kelly at the 2015 UMDF Symposium

Every year, the UMDF honors the accomplishments of our constituency. A number of awards are invited for nominations from the public. The UMDF invites you to nominate candidates for the three awards listed below. The deadline for nominations is April 30, 2016.

The awards will be presented at the UMDF's annual symposium on June 17, 2016. The winners will be honored with a plaque and will be featured in the UMDF Newsletter.

Energy Award

The purpose of the UMDF Energy Award is to recognize an individual who embodies the spirit of the UMDF and its Mission of "promoting research and education for the diagnosis, treatment, and cure of mitochondrial disorders and providing support to affected individuals."

There is no age restriction for the nominee. You may nominate an individual for the UMDF's Energy Award by filling out the form online with a 100 word explanation as to how this individual has exemplified the UMDF Mission.

You may also mail or e-mail supportive documents of your nomination to info@umdf.org (please put Energy Award Nominee and their name in the subject line) -- listing projects, activities, or other information that highlights their efforts in supporting the UMDF mission.

Submit your nominations online at https://www.surveymonkey.com/s/2016EnergyAward

Heartstrings Award

The Heartstrings Award recognizes a child or teen that has donated or raised funds for the UMDF, enabling the UMDF to continue its mission. The individual recognized must be under 18 years of age at the time of the donation or fundraising activity.

As part of the criteria for the award, the nominee must implement a fund raising project, demonstrate how the project was communicated to the community for awareness, and show the time invested in the project. The amount raised in comparison to the age of the individual will be considered. For nominees who donate funds, the judges will consider the generous spirit shown, communication, and amount donated in relation to the age of the individual.

You may nominate an individual for the Heartstrings Award by filling out the form online with a 100 word explanation as to how this individual has "tugged at your heartstrings" through fundraising for or donation to the UMDF. Identify important features of the nominee's activity, such as the time invested, creativity, communication skills, determination, effectiveness, and generosity.

Submit your nominations online at https://www.surveymonkey.com/s/2016HeartstringsAward

LEAP Award

The LEAP Award recognizes an individual who is living positively with mitochondrial disease (Living, Encouraging, Achieving, & Persisting).

In order to be nominated, the nominee must be at least 14 years of age or older. The nominee must have a confirmed or suspected mitochondrial disease and has demonstrated how they overcome the daily challenges to achieve their goals in career, family or volunteer service. The nominee must demonstrate a positive attitude, hope for a brighter future, and have enthusiasm that inspires others.

You may nominate an individual for the LEAP Award by filling out the form online with a 100 word explanation as to how the nominee demonstrates a positive attitude, hope for a bright future, and inspires others. If you wish, please provide copies of articles about the nominee, lists of projects, activities, or clubs to which the nominee belongs.

Submit your nominations online at https://www.surveymonkey.com/s/2016LEAPAward

MITOCHONDRIAL MEDICINE 2016: SEATTLE

Scientific Program
June 15-18, 2016

Experts from around the world join together to better understand mitochondrial disease. The Scientific Program will deliver the following learning objectives:

- Recognize the ways in which animal models of mitochondrial disease can help us better understand mitochondrial disease mechanisms in humans, and develop new concepts in the pathophysiology of human mitochondrial disease as well as therapeutic interventions.
- Illustrate how mtDNA replication and stability is a key factor in mitochondrial health and how impaired mtDNA replication and translation can lead to mitochondrial disease in humans.
- Explain the current knowledge of mitochondrial diseases of the eye and eye movement, focusing on those diseases that lead to visual loss and optic atrophy as well as ophthalmoplegia.
- Describe the new therapeutic techniques in altering mitochondrial function including mitochondrial replacement therapy and the use of zinc-finger nucleases, mitoTALENs and CRISPER technology.
- Compare the lessons learned with other rare diseases in terms of therapeutic drug development, and how to partner with the FDA for clinical trial design.
- Describe the ways in which cellular nutrients and nutritional interventions influence mitochondrial function and clinical trial design.
- Summarize the other functions of the mitochondria including the cell-danger response and how external toxins affect mitochondrial function.
- Distinguish how patient priorities, new drugs, and personalized clinical trials are being aligned to bring effective treatments to mitochondrial disease patients.

Patient/Family Program
June 17-18, 2016

Topics include:

- Study Design and Other Regulatory Issues Involving Clinical Trials for Mitochondrial Diseases
- What is Mitochondrial Disease and What to Expect
- Managing your Child's Individual Education Plan (IEP)
- Gastrointestinal Motility & the Mitochondrial Disease Child
- Transitioning to Higher Education and/or Jobs for the Young Adult Living with Mitochondrial Disease
- Behavioral Issues in the Child and Adolescent with Mitochondrial Disease
- Where does Dysautonomia Fit in the Mitochondrial Disease Patient?
- Medical and Genetic Testing in Individuals with Mitochondrial Disease
- Anesthesia and the Mitochondrial Disease Patient

Reserve your hotel room now! Visit *www.umdf.org/symposium/hotel2016* or call 206-246-8600.

BOOK EARLY - OUR ROOM BLOCK FILLS UP QUICKLY!

SCIENTIFIC & FAMILY PROGRAM REGISTRATION FORM
Mitochondrial Medicine 2016: Seattle
DoubleTree by Hilton Seattle Airport
June 15 - 18, 2016

REGISTER NOW TO GUARANTEE YOUR ATTENDANCE!

To Register:
1. Complete the registration form below and mail it back to the UMDF.
2. Complete the registration form below and fax it to UMDF at 412-793-6477.
3. Register online at www.umdf.org/symposium/registration. Use the registration code FAM1605 or SCI1605.

Registration Code: SCI1605

SCIENTIFIC PROGRAM FEES (Full four-day registration)

- ☐ $825 Physician/Researcher
- ☐ $775 UMDF/MMS/MRS/MIP Members (discount rate)
- ☐ $450 RN's/Allied Health Professionals/Residents/GCs*/Fellows**/Students

(GCs - Register online for CEU credit)*
*(** Letter from program director must be received in our office before May 29, 2016, in order to receive this fee.)*

Full registration entitles you to a hard copy syllabus, daily continental breakfast, refreshment breaks, lunch and the Friday night UMDF banquet (does not apply to daily rates). Payment must be received prior to attendance at symposium.

Registration Code: FAM1605

FAMILY PROGRAM REGISTRATION FEES

- ☐ $225 Individual Registration
- ☐ $450 Family Registration *(2 adults/same household)*
- ☐ $85 Daily Rate / LHON Only *(Banquet and Syllabus not included)*
- ☐ $65 Additional Friday Night Banquet tickets *(per ticket)*

Individual and Family registration entitles you to a hard copy syllabus, daily continental breakfast, refreshment breaks, lunch and the Friday night UMDF banquet (does not apply to daily rates).

TEEN REGISTRATION *(Sessions are free but registration is required!)*

- ☐ FREE Teen Registration *(Lunches and banquet not included)*
- ☐ $10 Friday Lunch *(per teen)*
- ☐ $25 Friday Night Banquet *(Special pricing for teens)*
- ☐ $10 Saturday Lunch *(per teen)*

Please make all checks payable to: **The United Mitochondrial Disease Foundation**

Please charge this registration to the following: Visa MasterCard Discover American Express

Card Number _____ Expiration Date _____

Name as listed on card (please print) _____

Signature _____ (invalid without signature)

PLEASE PRINT CLEARLY

Last Name _____ First Name _____ MI _____

Degree/Suffix _____ Specialty _____

Address _____

City _____ State/Province _____

Country _____ Zip/Postal _____

Email: _____

Phone: _____ Fax _____

Mail to UMDF, 8085 Saltsburg Road, Suite 201, Pittsburgh, PA 15239 or fax to 412-793-6477

Daily Rates
Registration for single days of symposium is available online.

Banquet Tickets
Additional tickets for the Friday night banquet are available online.

Scooters
UMDF has arranged for scooters for those in need of this type of assistance. In order to ensure that we can meet your needs, please be sure to request a scooter on your registration form.

Cancellation Policy
All cancellations will be subject to a service charge based on registration fee amounts – a small percentage must be deducted to cover processing charges to the foundation. A written notification of cancellation must be made to UMDF in order to process any refunds. No refunds will be issued after Friday, June 26, 2016..

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.

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!

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Tara Maziarz
Development Associate/ Social Media

Nicole McCaslin
Regional Coordinator - Northeast

Margaret Moore
Regional Coordinator - Southeast

Anne Simonsen
Regional Coordinator - Great Lakes

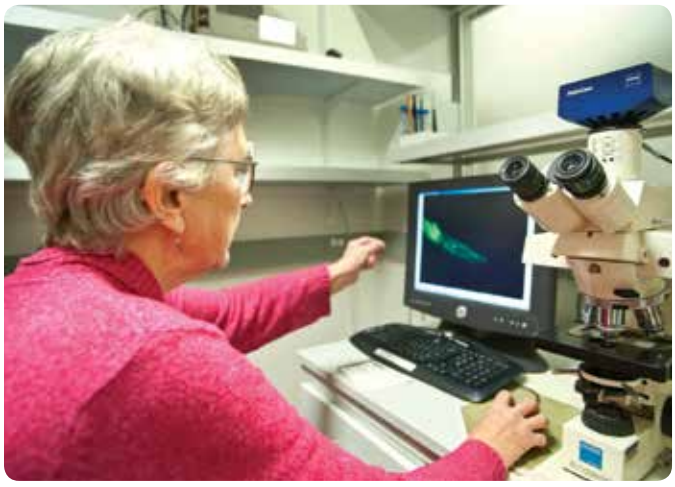
UMDF Mission

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.

A leader in studying mitochondrial disease, Seattle Children's research is being applied to state-of-the-art patient care, diagnosis and treatments.



Visit www.seattlechildrens.org/mito-research to learn more about how you can help advance mitochondrial research at Seattle Children's.

Seattle Children's®
HOSPITAL • RESEARCH • FOUNDATION

Research Institute