

Summary – 2015 Mito Town Hall Various Speakers

Welcome message, Philip Borden, President



2014 was a terrific year for MitoAction, one of tremendous growth. A few highlights:

- We held two Clinical Conferences in 2014, one in Los Angeles and one in Boston.
- We established the Advocacy Task Force, an exciting new initiative, in response to Medical Child Abuse situations that Mito families had faced.
- We established the Matthew Harty Camper Fund to send Mito kids to camp. The fund was created in memory of Matthew Harty, who passed away from Mito in 2013. We did a substantial amount of fundraising to seed this fund.
- We built out the team in 2014. “We’re still a small organization but we do a lot with a little,” Philip said. MitoAction brought on new people to expand our scope of services. Those additions are Christine Cox, Director of Outreach and Advocacy, and Shawney Lamm, Event & Program Manager.

In 2015, MitoAction will be increasing our partnerships with companies and organizations and doing additional clinical conferences and support and advocacy services.

Thrive RX, Abby Brogan



ThriveRx is a national nutrition support company providing support for individuals on TPN and/or enteral feeds at home. ThriveRx offers a monthly online educational series of webinars on topics surrounding GI motility especially designed to help patients who rely on tube feedings or TPN. Topics vary and webinars are free. Topics of upcoming webinars include:

- Reviewing inset transition
- Dysmotility, diet, and action
- Advocating for yourself at home, work, and school
- Education system overview, explaining the differences between IHP, 504 and IEP
- GI anatomy overview
- Understanding lab values
- ADA accommodations

For more information: www.thriverx.net

MitoAction Events, Susan Stover, Events & Development Director

Mark your calendars for these upcoming MitoAction events.

- **Feb. 27-28:** Wear Jeans for Genes for World Rare Disease Day. Encourage your coworkers, friends, families, classmates to make a small donation to wear Jeans for Genes and raise awareness. MitoAction will have printable materials on our website.



- **May 2, 2015:** The sixth annual Sandra K. Russell Derby Day Benefit for Mito will be held at Mandarin Oriental, Boston. It's the hottest Kentucky Derby party in Boston!
- **Sept. 12, 2015:** The 11th annual MitoAction Energy Walk & 5K will be held on a Saturday! We'll descend on beautiful Mother's Rest again in South Boston, MA.
- **September 2015, TBD:** The second annual Matthew Harty Camp and Scholarship Fund Golf Tournament will be held.

And as always, volunteers are appreciated. [Click here to sign up to volunteer for Derby Day.](#) [Click here to sign up as a volunteer for the walk.](#)

Susan can be reached at events@mitoaction.org.

Movie Socials & Satellite Events, Shawney Lamm, Event & Program Manager, MitoAction



As MitoAction continues to grow the organization, we'd love to see more satellite events. It is so inspiring when people ask how they can educate the community about Mito. One good way is to host a satellite event, which can include a 5K run, a walk, a golf tournament, a cycling event, and more. And we are always open to your great ideas! These events are a wonderful way to bring people together while raising awareness and having fun!

Another wonderful idea is hosting a movie event with Rina Goldberg's "The Magic Bracelet." Rina, who lost her battle with Mito at the age of 15, asked her mom to take care of her film. Let's help take care of Rina's film, too. Hosting the event is easy and we'll help every step of the way! We'll help you select a location that can show a DVD and seat at least 30 people (local movie theaters, high schools, library meeting rooms, university rooms, etc.). We'll also help with the promotion and compile a package of materials for you to have at your event, including the DVD, coloring sheets, a Magic Bracelet craft, and more. [Click here for more information on hosting a movie showing!](#)

Please email Shawney at shawney@mitoaction.org to get started.

Miracles for Mito, Maria Hopfgarten, Heather Schichtel



Miracles for Mito™ This group provides support to Mito families in the Rocky Mountain region. Its mission is: No one should walk this journey alone.

Among the highlights from 2014, Miracles for Mito:

- Organized its first Mito symposium with UMDF and Children's Hospital Colorado.
- Organized quarterly support meetings for families. The next meeting is Saturday, April 4.
- Provided family grants for things that insurance doesn't cover, such as summer camp, a respite provider, motorized tricycle, and more
- Sponsored two parents to attend the UMDF conference

In 2015, Miracles for Mito is looking to:

- Participate in an ethics conference March 6 at Children's Hospital.
- Host a Mito walk in May
- Hold a silent auction/dinner on Oct. 3.

Miracles for Mito puts a team together to participate in a 156-mile bike ride for Children's Hospital and has raised enough money to earmark funds for Mito research.

Last year, the organization raised close to \$110,000 and has become the fundraising arm for Mito research in Colorado.

For more information: www.miraclesformito.org or on Facebook at <https://www.facebook.com/MiraclesForMito>.

EPI-743 Clinical Trial, Dr. Matt Klein, Edison Pharma



2014 was a very successful year for Edison. In 2014, Edison conducted 13 EPI-743 clinical trials in 10 diseases under three regulatory jurisdictions (United States, European Union, Japan). Four of these trials were randomized double-blind, placebo-controlled trials. In 2014, Edison reported its positive placebo-controlled trial results from the EPI-743 Rhetts Syndrome Study, which demonstrated head growth in kids treated with EPI-743 relative to placebo. It was a very significant finding. By the close of 2014, Edison had treated 300 people – ages 23 days old to 56 years old -- with EPI-743. The company also reached its 1 millionth dose of EPI-743!

On the regulatory front, EPI-743 has secured orphan designation in the United States for three diseases – Leigh syndrome, Friedreich's ataxia, and Rhetts Syndrome; has a

fast-track designation from the FDA for pediatric Friedreich Ataxia; and has received orphan designation in Japan for Leigh syndrome.

The organization has also completed Phase 1 studies on EPI-589. It demonstrated an excellent safety profile and is advancing to Phase 2 clinical trials. Edison has also expanded its collaborative agreement with its partner, Dainippon Sumitomo Pharma, for the development of 10 new redox active compounds.

In 2015, Edison is looking forward to similarly exciting times. In addition to announcing clinical trial results and studies, it will be announcing business initiatives.

For more information: info@edisonpharma.com.

Mitochondrial Medicine Society, Dr. Amy Goldstein



Head to www.mitosoc.org to see the Society's updated active website along with its live Google map of the Mitochondrial Global Networks. Its new mission: Advancing education, research, and global collaboration in clinical mitochondrial medicine. The live Google map highlights clinicians worldwide, support groups, and patient networks. The map is live and the society is anxious to include other clinicians or support groups, so if you know of anyone who should be included, please let Dr. Goldstein know at mitosoc@mitosoc.org.

The website also features recent publications, including Mitochondrial Medicine Society's North American Consensus Criteria for Diagnosis and Treatment of Mitochondrial Disease, which was recently published in Genetics and Medicine. A comprehensive review, the Society looked at every aspect of the diagnosis and treatment of Mito. It will be helpful as a reference for clinicians, and will be useful for insurance approval of supplements and other testing. Parts 1 and 2 of "Practice Patterns" is also on the website, outlining what clinicians are currently doing for diagnosis and treatment for Mito.

The Mitochondrial Medicine Society is looking forward to other projects. Dr. Goldstein said the Society recently completed a statement on genetic testing and noted that families are still getting denied genetic testing from insurance. The group hopes this statement from the Society and other groups will help families get insurance coverage for these tests. The group is also working on projects involving statements for supplement use and advocacy and other concerns regarding Medical Child Abuse. It is also working with New Castle Group in England to update the Standard of Care guidelines on their websites.

Finally, the NIH-initiated Common Data Elements project is completed and going through final review, then will be available for public access. Google Common Data Elements to see what's already out there.

For more information: www.mitosoc.org

Atlanta Clinical Conference, Cristy Balcells, Executive Director, MitoAction



Several years ago, the MitoAction Medical Advisory Board was discussing gaps in care for Mito patients. The board members noted that primary care doctors, pediatricians, and other specialists want to wash their hands of everything having to do with these patients and let the Mito specialists take care of everything. But the specialists can't possibly manage the patients day-in and day-out on everyday issues so the group decided it needed to improve the understanding and practical approaches to clinical management of Mito at the primary care level ... from that discussion, the MitoAction Mitochondrial Disease Clinical Conference was born. It's a clinical meeting to attract healthcare providers who see a few Mito patients on a regular basis to get the nuts and bolts from the experts on mitochondrial medicine on how to care for them.

Our fourth conference will be Saturday, March 21 in Atlanta. Help us spread the word! Tell your doctors they should attend. For more information, go to www.mitoaction.org/clinicalconference. For a printable flyer you can bring to your doctor, [click here](#). And if you need us to reach out to your doctors, email us at info@mitoaction.org.

Mito 411, MaryBeth Hollinger, Volunteer



1-888-MITO-411 is live support offered to families by families. The program doesn't offer medical advice, but rather support, education, and camaraderie. Mitochondrial disease can be scary, overwhelming, and difficult to sort out. The volunteers at Mito411 can help in a crisis, help you feel connected and understood, help a mom not feel so alone or crazy, listen to a dad who lost his job for taking care of his sick wife. It's two people finding common ground and moving forward together instead of alone. When you call the number, you will leave a message and a volunteer will call you back within 24 hours.

Volunteers are the heart of Mito411. Volunteers have been touched by Mito, including adult patients, parents of affected children, social workers, and nurses. They are trained to offer information and support. They rely on Cristy's book, "Living Well with Mitochondrial Disease," the MitoAction website, other websites, and each other for resources. Mito411 is seeking volunteers to help provide this vital one-on-one support. If

you would like to be a volunteer, please email MaryBeth at mito411@mitoaction.org. For more information: www.mitoaction.org/mito-411

Stealth BioTherapeutics (formerly Stealth Peptides), Travis Wilson, CEO



Stealth BioTherapeutics is committed to working on mitochondrial diseases. Stealth has been developing Bendavia and Ocuvia and has been in 15 double-blind, placebo-controlled trials with those drugs and been involved in 500 patients of recruitment.

Stealth is looking forward to an exciting year. A lot of clinical data will be presented. Travis is happy to announce that Stealth will start screening for a Mito myopathy patient study with Bendavia. It currently has a skeletal muscle disorder study that's been enrolling patients. On the ocular front, Ocuvia has started a retinal disease study. For the Mito community, later this year, Stealth will be launching a study in Leber's hereditary optic neuropathy.

For more information: www.stealthbt.com. Learn of their clinical trials on clinicaltrials.gov.

Advocacy Activities, Christine Cox, Director of Outreach & Advocacy, MitoAction



It's been a great year for advocacy at MitoAction!

Christine is completing her first nine months with MitoAction and in that time has rolled out the first wave of Advocacy Task Force materials on www.mitoaction.org/advocacy. The Task Force, made up of professionals and parents with experience with Medical Child Abuse (MCA) issues, has created the Navigator Toolkit to help parents navigate the medical system while keeping triggers in mind that could prompt allegations of MCA. The site has a wonderful piece about responding to adverse allegations written by MaryBeth Hollinger from information she has collected as the Mito411 coordinator. And there's a great article by Kristi Wees called "You know Your Child Best." The Task Force has also been adding podcasts and videos.

Coming in 2015, the Task Force will post patient stories from Mito families who have dealt with allegations of Medical Child Abuse. It's a powerful way for people to understand what can happen and protect themselves. The group is also working on resources for medical professionals as well as attorneys. Christine thanked the 10 volunteers who have worked so hard on this task force.

She also wanted to mention a piece of legislation that was just reintroduced in Massachusetts that MitoAction has been working on for seven years. The Mito cocktail insurance coverage bill, the first introduced in the country, would mandate that insurance companies must cover the Mito cocktail for Mito patients in Massachusetts. If any Massachusetts resident would like to get involved in moving this bill forward, email Christine at outreach@mitoaction.org.

UMDF Events & Updates, Phil Yeske



In 2014, UMDF helped develop and launch the Mitochondrial Disease Community Registry to gather medical information from patients, caregivers and family members with an eye toward facilitating the development of treatments and cures. To sign up, go to www.UMDF.org/registry. A confirmed diagnosis is not necessary to sign up. UMDF is trying to aggregate as much data as possible for those developing drugs and therapies that will lead to treatments and cures down the road. The goal is to try to understand the natural history of Mito from the patient perspective. Privacy is the hallmark of this registry. You have full control over your privacy settings. In the first five months, more than 1,000 people have registered and more than 100,000 questions were answered.

The UMDF Symposium will be held in Washington, DC from June 17-20. The event will also include a Day on the Hill to visit Congress and Senate members and tell them about Mito. [Click here for more information](#).

Partnerships will be big part of 2015, leading to increased grants; increased participation in Grand Rounds; and an increased emphasis on regional symposiums.

Foundation for Mitochondrial Medicine, Laura Stanley



The foundation's purpose is to fund treatments for mitochondrial disease, make connections between related diseases, and raise awareness.

The highlights for 2014 included key research projects the foundation co-funded:

- Alzheimers' Drug Discovery Foundation, New York: It's a \$200,000 project awarded to James Bennett, who is doing gene therapy and research of Alzheimer's and how a Mito protein has been shown to increase Mito function in animal models.
- Michael J. Fox Foundation for Parkinson's research: This was awarded to a Mayo Clinic researcher who is studying the Parkin gene, which impacts the health of the mitochondria.
- Georgia State University: The school is studying cognitive fatigue and its impact on children ages 5-16 with Mito and autism; just Mito; and kids with no diagnoses. The study is still seeking participants in this clinical trial and Laura encourages people to participate: email robinmorris@gsu.org.

Laura mentioned that a local supporter has written a book called "Shannon's Gift," a touching story of how he has managed his grief and the unexpected loss of his wife from Mito. A portion of the book sales supports mitochondrial disease research. For more information: www.shannongift.com. You can reach Laura at Lstanley@mitochondrialdisease.org and the website hopeflies.org.

Camp Opportunities (Camp Korey, Victory Junction, Double H Ranch)



MitoAction is excited to expand our camper network to send as many kids as possible to camp. These camps are medically supervised and equipped to provide kids of all abilities the chance to participate in all camp activities, ranging from zip lines and horseback riding to fishing and swimming.

In addition to Camp Korey, we are now partnered with Victory Junction in Randelman, NC, and Double H Ranch in the Adirondacks of New York.

According to Victory Junction medical director Dr. Haberman, Victory Junction serves 1,000 kids from ages 6-16 each summer. "We are so excited to bring camp to more Mito kids," she said, noting that since the camp is in North Carolina, it has a NASCAR racing theme. Victory Junction's Mito sessions will be held June 28-July 1 and Aug. 2-5. For more information: Victoryjunction.org

Double H Ranch has already held sessions with mitochondrial disease kids. With our partnership, we're going to help bring even more kids with Mito to camp this summer. Double H's Mito sessions will be held June 24-29; Aug. 4-9; Aug. 12-17. For more information: www.DoubleHRanch.org

MitoAction has partnered with Camp Korey for several years. As the camp has grown, so has the program to serve kids with Mito and we're thankful for the opportunity to have that partnership. Kids from all over country have been able to go to Camp Korey. The Carnation, WA, camp has three weeks of Mito sessions this year: June 29-July 3, July 20-24, Aug. 10-14. www.campkorey.org

For more information, check out www.mitoaction.org/mito-camps. All of this is made possible because of the generous donations to the [Matthew Harty Camper Fund](#), which helps offset costs of travel, programming, etc.

Nextgen Sequencing, Dr. Richard Boles, Medical Director, Courtagen Life Sciences



Dr. Boles is enthusiastic about Nextgen Sequencing. It's totally revolutionized his practice to the point where most of his Mito patients have exact diagnoses. He said there is a lot of work ahead about what the data means but it's revolutionizing the field.

Nextgen Sequencing is entering clinical practice in a larger way in which doctors are now ordering it for autism, mental retardation, and much more.

It's an important diagnostic test, Dr. Boles said. But insurance companies don't want to expand their coverage if they don't feel it's necessary. Dr. Boles and others are asking insurance companies to cover this test when it is the most appropriate test. Dr. Boles has been working with physicians around the country and people who work in labs and in academia to draft letters saying that Nextgen is a useful tool and part of routine care and should be covered by insurance. Dr. Boles asked the audience to encourage their doctors to sign the letters. If your doctor wants more information or to sign the letter, have him or her email Cristy at director@mitoaction.org.

Friday/New England Support Groups, Julie Gortze



MitoAction offers call-in support groups on the second, third, and fourth Fridays of the month at noon EST to share information and get support. Just call in 1-866-414-2828, participant code 017921#. [Click here for more information](#). The groups are:

- Second Friday: Support group for newly diagnosed patients and parents
- Third Friday: Support group for parents
- Fourth Friday: Support group for adult patients and spouses
- The first Friday of the month is reserved for a speaker on a topic relevant to the Mito community. These calls are recorded and put on the MitoAction iTunes library.

Mito Support Group of New England: Julie hosted a Mito Patient & Family Social about 1 ½ years ago. There was a great turnout of about 20 people, who all voiced appreciation to have the opportunity to meet others affected by Mito face-to-face. Somehow, that first Social has turned into a regular support group. The Mito Support Group of New England now meets every other month (with a few exceptions if opportunities arise for special events) at Cardi's Furniture in South Attleboro, MA. Some of these meetings are simple – just people affected who come together to share with and support each other. Others feature special guest speakers who talk on topics important to the Mito community. Julie has offered such things as visits from

representatives of Make-A-Wish, Icing Smiles, Hole-in-the-Wall Gang Camp, ThriveRx, and more. She has also organized events featuring an attorney who specializes in Medical Child Abuse cases, a medical ethics talk, and visits from Dr. Amel Karaa, a Mito doc from Massachusetts General Hospital, and a local state representative who spoke about a rare disease bill he is submitting in Massachusetts. The most recent event was an appreciation luncheon to honor Dr. Mark Korson and Dr. Karaa.

In 2015, Julie has some exciting things in store:

- A meeting geared to teens and young adults.
- A visit from a representative from America's Compounding Center who will talk about compounding the medications/supplements used by Mito patients and insurance issues when trying to get them covered.
- A geneticist who specializes in mitochondrial medicine, Dr. Nyazov, will be coming from New Orleans in July to speak about exercise and the Mito patient.
- A workshop on strategies in helping children learn to advocate for their own needs in schools and among their peers.
- A presentation on palliative care.

If you know of any resources or educational opportunities that would benefit the Mito community, or have any ideas to share, email Julie at julie@mitoaction.org.

Awareness, Ginger DeShaney, Membership & Support Coordinator, MitoAction

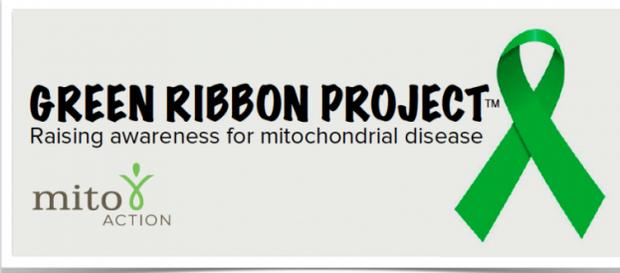


Host a Mito Patient & Family Social! These Socials, held around the country, are laid-back, casual gatherings that allow Mito families to meet in person, give and get support, and realize they are not alone on this Mito journey. We are always seeking people to hosts these Socials. It's super easy to host and Ginger help you every step of the way. For more information or to get started, email her at support@mitoaction.org.



One of the best ways to make an impact in the community is by hosting a Restaurant Night! Eat out, raise awareness, and have fun! Restaurants are excited to support us through these charitable programs in which a portion of the proceeds from your Restaurant Night will be donated to MitoAction. California Pizza Kitchen, Uno, and Not Your Average Joe's are just a few of the restaurants that host these nights. Even if you're super busy, it takes just a phone call or

a visit to the restaurant to set it up. For more information, check out www.mitoaction.org/restaurant-night or email Ginger at support@mitoaction.org.



We count on you, our Mito community, to raise awareness about Mito. New in 2015, MitoAction is launching a global public awareness campaign called the "Green Ribbon Project." The Green Ribbon Project is a way for YOU to make a difference by raising awareness and sharing your story. Part of this campaign involves wearing the brand-new green ribbon pin and finding ways to share how your life has been changed by mitochondrial disease. Just as the pink ribbon has raised so much awareness about breast cancer, we want our Green Ribbon Project to do the same for mitochondrial disease awareness. Email Ginger at support@mitoaction.org, or send us a message on Facebook. You can also read all about it at www.mitoaction.org/green-ribbon-project.

MitoCanada Events & Updates, Vicky Spadoni



MitoCanada has had a really exciting year. The group received its charitable registration status in 2010 and has been operating highly on volunteer support since.

Highlights:

- The support program currently provides one-on-one peer support that matches a volunteer to the client requesting support.
- The support network meetings, which were face-to-face, will rely more on technology to connect Canadians across the country.
- Mito Loves On, for those who have loved and lost someone to Mito, includes a monthly conference call. The organization has set up a [private Facebook page](#), but it's not just for Canadians.
- Mito Mail Pal program, for children 3-12 who have Mito, are a sibling of a Mito patient, or a child of someone with Mito, is a big success.

In regards to awareness, MitoCanada turned Niagara Falls green to promote Mitochondrial Disease Awareness Week. This year, the organization is striving to do that again ... and is looking for other buildings to turn green.

Updates on research front:

MitoCanada has seen significant growth in its capacity to fund research. MitoCanada's research committee, in consultation with leading Canadian Mito researchers, is focusing on supporting research that aims to improve the identification and treatment of Mito. In 2014, MitoCanada funded two projects: Children's Hospital of Eastern Ontario in Ottawa for integrated Mito diagnostics platform combining Nextgen with respiratory studies in

patient cells, and Project Frozen vs. Fresh for muscle biopsies to McMaster University in Hamilton, Ontario. Go to www.mitocanada.org to review the research MitoCanada has funded. [Click here for MitoCanada's Facebook page.](#)

Vicky has resigned as executive director; Maureen Latocki is taking over as ED: Maureen.latocki@mitocanada.org.

Mitochondrial Research Guild, Steve Serax



The Research Guild has developed a new website: www.nwmito-research.org.

The Research Guild provides Seattle Children's Hospital's Mito patients with support by way of annual fundraising events, such as auctions and golf tournaments; partners with local businesses for smaller events; and puts together Mito family gatherings

The Guild provides financial support to its grant program to Seattle Children's Mito patients. It partners with Dr. Russ Saneto from Seattle Research Institute in providing funds to continue to expand its research team and its research. It also provides resources for Dr. Saneto, salaries for post-docs, salaries for a nurse practitioner and administrative support for Dr. Saneto at Children's Hospital; plus funds for research grants and research equipment. Some of the other researchers the guild supports include Dr. Marge Sadensky, Dr. Phil Morgan and Dr. Albert Quintana.