

## **Summary – 2014 Town Meeting**

**A great year ahead, Cristy Balcells RN MSN Executive Director**



For MitoAction, 2013 was an important year. We learned that our Mito community is growing and that we are ready to work together to bring awareness and treatments to light. During 2014 we will continue to build, expand, and broaden our scope with even more opportunities to make a difference and educate people about Mito. For more information, email us at [info@mitoaction.org](mailto:info@mitoaction.org).

### **Camp Korey Summer Camp: Emma Thier, Camper Recruiter**



**a seriousfun camp** This summer, Camp Korey in Seattle, WA will offer three sessions for campers with mitochondrial disease. Camp Korey gives children ages 7-16 with serious medical conditions a summer camp experience in a medically sound environment. The Mito sessions will be held June 30-July 4, July 7-11, and July 21-25. Applications are available online and will be accepted on a first-come, first-served basis. Go to [www.campkorey.org/apply](http://www.campkorey.org/apply) for the application and more information. We are so excited to continue our partnership with Camp Korey. If you have questions, please email Emma at [etheir@campkorey.org](mailto:etheir@campkorey.org) or call 425-844-3226. Email Emma if you are interested in being a health care volunteer.

## MitoAction Events



You should be a part of MitoAction's awareness event lineup for 2014! Join us for a special screening of "The Magic Bracelet" in Boston. Details to come!

On Feb. 8, doctors and nurses will gather in Los Angeles for the [LA Mitochondrial Disease Clinical Conference](#).



On May 3, join us at the fifth annual [Sandra K. Russell Derby Day Benefit for Mito](#) at Mandarin Oriental in Boston and for the Boston Mitochondrial Disease Clinical Conference. Tickets for Derby Day go on sale on Feb. 1.



Don't miss our largest community gathering, the annual MitoAction Boston Energy Walk & 5K on Sept. 14, 2014 at DCR's Mother's Rest. Registration opens in April for our 10th annual event! This event kicks off awareness week.

In 2013, four families hosted their own awareness events, and we want to help you create such an event in your community! For more information on all these events, email us at [events@mitoaction.org](mailto:events@mitoaction.org) or [Sara@mitoaction.org](mailto:Sara@mitoaction.org).

## EPI-743 Clinical Trial: Dr. Matthew Klein, Edison Pharma



Progress of trials with EPI-743 around the world was truly a highlight of 2013. Three hundred patients, with 30 different diseases, were treated with EPI-743 as of December 2013. "The data suggests that it's safe and well tolerated," said Dr. Klein. There are eight ongoing 743 trials in the US, Asia, and Europe. For more information, go to [clinicaltrials.gov](#). In the next six to eight months, Edison will determine next steps. Phase 1A trials are completed on EPI-589 with favorable results so Edison will be doing two trials with 589. For more information, visit [www.mitoaction.org/epi743](http://www.mitoaction.org/epi743) or

[www.edisonpharma.com](http://www.edisonpharma.com), email [info@edisonpharma.com](mailto:info@edisonpharma.com), or call the EPI-743 hotline at 1-800-243-0254. To get updates on 743 or 589, visit [www.edisonpharma.com](http://www.edisonpharma.com), go the contact page, and sign up for updates.

### Courtagen Life Sciences, inc., John Linnan, Vice President of Sales and Marketing



In 2013, Courtagen continued to build its internal database. Data was built looking for patterns in Mito and trends in overall treatment modalities. In 2014, the hope is to expand the base and include patient histories both prior to treatment and post treatment. An effort will be made to capture more clinical data; genetic counselors will be reaching out to more doctors to see what post-clinical treatment data can be gathered. And new assays for co-morbidity panels will be explored for Autistic Spectrum disorders, trying to sort out treatments and diagnoses.

### Stealth Peptides, Travis Wilson, CEO



A report will be ready at the end of 2014 on Phase 2 on a multinational study of 260 patients in the US & Europe. Stealth will be initiating new clinical studies later in 2014 to look more closely at potential treatments/therapies for Mito. For a discussion of Bendavia, a Stealth Peptide drug, go to <http://www.mitoaction.org/blog/introducing-bendavia>.

### Light a Light for Mito



As a community, we continue to honor the memories of our dearest children, friends, and family we lost to mitochondrial disease. Join us on Wednesday, Sept. 17 to "Light a Light for Mito." Our webpage, [www.mitoaction.org/memories](http://www.mitoaction.org/memories), offers a list of resources, a link to share your photos and memories, a link to our forum, and more. Please contact us at [memories@mitoaction.org](mailto:memories@mitoaction.org).

### Marcel's Way Family Fund



The Marcel's Way Family Fund committee invites you to apply for support or donate in order to give a gift to those in need. The Marcel's Way Family Fund

offers one-time grants of up to \$500 for expenses related to mitochondrial disease. To donate, apply, or learn more, please visit [www.mitoaction.org/marcels-way-family-fund](http://www.mitoaction.org/marcels-way-family-fund).

### Mito 411 Support Hotline



1-888-MITO-411 is a support group for patients and families provided by families. Anyone is welcome to call the hotline and speak with a volunteer who is either an adult patient or a parent of an affected child. In addition, we are seeking volunteers to help provide vital one-on-one support. Mito 411 allows a person to talk to someone who understands what they are going through. It's not about medical advice; it's about helping, supporting, and offering a shoulder to lean on. For more information or for support, call us (1-888-MITO-411) or email [mito411@mitoaction.org](mailto:mito411@mitoaction.org).

### ThriveRx iThrive: Abby Brogan, Outreach Coordinator



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. Upcoming topics include GI Anatomy and Dysmotility Overview. For more information, follow Thrive RX on Facebook at <https://www.facebook.com/ThriveRx>, visit <http://www.thriverx.net>, or email [info@thriverx.net](mailto:info@thriverx.net).

### UMDF Conference: Cliff Gorski



This year's UMDF Mitochondrial Medicine conference will be held in Pittsburgh, PA. The four-day scientific and clinical program runs from June 4-7; the two-day family session is June 6-7. Registration is now open. Scholarships are available to attend the meeting. For more information, email [info@umdf.org](mailto:info@umdf.org) or [connect@umdf.org](mailto:connect@umdf.org), call 888-317-UMDF, or click [here](#). If you are holding an educational meeting, please let UMDF know at [info@umdf.org](mailto:info@umdf.org) and they will put it on the calendar.

### Mito Socials & Awareness Kits: Ginger DeShaney, Support Coordinator



Mito Socials bring families together in the same community for support, friendship, and camaraderie. We are always looking for volunteer hosts. It's easy and super fun. For more information, check out <http://www.mitoaction.org/mito-patient-and-family-socials>. If you'd like to raise awareness, please email us for a kit that

includes materials to hand out. If you're interested in an awareness kit or in hosting a Social, please email Ginger at [support@mitoaction.org](mailto:support@mitoaction.org).

### **Hole in the Wall Gang Camp, Cristina Sapoval**



**a seriousfun camp** This camp in Ashford, CT, serves kids ages 7-15 with mitochondrial disease and metabolic disorders in the Northeast. The seven-day residential camp is free of charge. The medical and nursing team, along with a dietary specialist, focus on the campers. The camp also has special programs for siblings of patients. A metabolic family weekend, a whole family camp experience, is scheduled for April 11-13. Find the application on the website at [www.holeinthewallgang.org](http://www.holeinthewallgang.org). For more information, email [ashford@holeinthewallgang.org](mailto:ashford@holeinthewallgang.org) or call 860-429-3444.

### **Miracles for Mito, Maria Hopfgarten and Heather Schichtel**



Miracles **for** Mito™ This nonprofit was established 3 years ago in Colorado by two mothers of children with Mito to ensure that no one was alone. It provides support to those in the Rocky Mountain area and so far has reached 100 families. Events are held every quarter; sessions are about 3-4 hours long and bring awareness, education, and support. The next meeting is April 5, with meetings also in June and September. Its annual walk is in May. Miracles for Mito also sponsors a family grant program launched last year to help purchase equipment or help with other costs as well as to bring food to families who have someone in hospital. The group works with Children's Hospital of Colorado, giving free of charge Cristy's "Living Well with Mitochondrial Disease" book to all families who use the Mito Clinic. Through their fundraising efforts, Miracles for Mito has earmarked funds for the Research Clinic for Mito in Colorado and to provide Ubiquinol free to all families who use the clinic.

### **Foundation for Mitochondrial Disease Medicine, Laura Stanley, Executive Director**



Located in Atlanta, this group supports the development of the most promising mitochondrial disease research and treatments of the many forms of mitochondrial disease. They will be awarding a large grant in 2014. More collaborative partnerships are planned for 2014 as well as regional awareness events and fundraisers. For more information, go to [www.mitochondrialdiseases.org](http://www.mitochondrialdiseases.org) or [www.hopeflies.org](http://www.hopeflies.org).

## **Research updates**

### **Dr. Richard Boles, Courtagen Life Sciences and Children's Hospital LA**

Energy metabolism studies are well underway. Studies are looking at components of cells that have not been looked at before that may be treatable so that clinicians can get an exact diagnosis and pinpoint a treatment.

### **Dr. Amy Goldstein, Children's Hospital Pittsburgh**

Dr. Goldstein is secretary-treasurer of the Mitochondrial Medicine Society ([www.mitosoc.org](http://www.mitosoc.org)). The group has been able to publish two surveys for all Mito Physicians in North America; this will provide consensus statements since so many physicians were involved. Later this year, one survey will be published dealing with topics like exercise, diet, pulmonary issues, etc. The North American Mito Consortium is enrolling patients in its registry – it will be the largest database of patients with Mito thus far. Research studies will be open to all those registered all over North America. Regarding the Common Data Elements Project: particular outcomes are scientifically selected so that when research is done, outcome measures from one study to the next can be compared. For more information Google “common data elements.”

### **Northwest Mitochondrial Disease Research Guild, Jill Herczog**



The guild started with just one doctor, but now has a team of specialists & researchers. The 2014 Family Grant Program offers funds for expenses not covered by insurance. The guild supports Camp Korey, as well as research through grants to fund postdoctoral positions. The second annual Northwest Mito Summit will be held Sept. 11-13 and brings together experts. For more information, visit [nwmito-research.org](http://nwmito-research.org) or email Jill at [j.herczog@nwmito-research.org](mailto:j.herczog@nwmito-research.org).

### **MitoCanada**



MitoCanada has obtained office space. They are offering a new program, Mito MailPal, that allows children ages 3 - 12 to connect with each other. The first support network meeting was held in November and had a great turnout. [www.mitocanada.org](http://www.mitocanada.org).

### **Mito Quilts of Hope, Christine Knox**



In August 2013, Christine Knox launched a new project -- **Mito Quilts of Hope** -- which raises awareness about Mito. This project educates quilters about Mito and encourages them to make quilts for Mito patients. Quilters can register to make quilts, and Mito patients can sign up to receive a quilt. [www.mitoquiltsofhope.org](http://www.mitoquiltsofhope.org).

## **Advocacy Task Force**



MitoAction's Advocacy Task Force was initiated in response to the publicity surrounding the Justina Pelletier court case, which demonstrated the need for advocacy in order to help families handle allegations of medical abuse, etc. Applications will be available for anyone who would like to volunteer to work on this task force. People with professional backgrounds and/or personal experience are needed. Work will include networking and guidance, and require collaborative efforts. <http://www.mitoaction.org/advocacy>