

Mito Cocktail Treatment Comparison – March 2013 Survey Results

This survey was designed to assess patients' current experience with and perception of effectiveness of vitamins and supplements known as the "Mito Cocktail" used in the clinical management of Mitochondrial Disease. MitoAction gathered anonymous information from our patient and family community for use in understanding what benefits, side effects and obstacles these treatment recommendations provide. MitoAction intends to repeat this survey again in 2015 in order to track changes in the patient experience over time.

1. Different physicians utilize different mitochondrial vitamin/cofactor/anti-oxidant "cocktails" and even different ones for their different patients, depending on the diagnosis. What does your cocktail consist of?

Total Number of Respondents: 337

Value	Count	Percent
Coenzyme Q10	299	88.7%
Carnitine	269	79.8%
Riboflavin (B2)	210	62.3%
Vitamin C	138	40.9%
Lipoate (Lipoic Acid)	120	35.6%
Other	116	34.4%
Thiamine-HCl (vitamin B1)	115	34.1%
Creatine	107	31.8%
Vitamin E,	97	28.8%
Pyridoxine (B6)	77	22.8%
Selenium (metal cofactor)	24	7.1%
D-Ribose	22	6.5%
Pantothenate	19	5.6%
Nicotinamide	14	4.2%
None- I do not take any of these things	12	3.6%

2. Please rate the level of compliance you or your children, spouse, or patients have when consuming their individual "Mito Cocktail" component(s).

Total Number of Respondents: 337

Value	Count	Percent
Excellent (never misses)	171	50.7%
Good (almost every day)	103	30.6%
Medium (4 out of 7 days)	28	8.3%
Poor (always a struggle)	25	7.4%
User Provided No Response	10	3.0%

3. If you answered "Poor" or "Medium" in the answer above, check which statements apply.

Total Number of Respondents: 337

Value	Count	Percent
User Provided No Response	272	80.7%
Poor - medium compliance due to daily volume	33	9.8%
Poor-medium compliance due to limited noticeable symptom improvement	24	7.1%
Poor - medium compliance due to taste	15	4.5%
Poor medium compliance due to product form (powder, liquid, pill)	13	3.9%

4. In your opinion, how helpful is the "Mito Cocktail" in improving energy and symptoms for you, your child, your loved one, or your patient? (Note: please answer based on your experience using the components as a whole, regardless of cost or side effects)

Total Number of Respondents: 337

Value	Count	Percent
Very helpful - a noticable difference	169	50.1%
Somewhat helpful	100	29.7%
Not very helpful	50	14.8%

I stopped taking the Mito Cocktail because I felt it was not helping	17	5.0%
User Provided No Response	12	3.6%

5. If you have found the "Mito Cocktail" helpful, what specific changes have you noticed? What are the outcome measures (ways you feel that it is helpful, such as increased energy, improved child development, increased concentration, overall stamina, etc.) that you find most important? Please be as specific as you can, thinking about what's important to you.

Representative Responses:

- Sick less often and able to recover more easily from illness, definite change in energy, big gains in cognitive development, improved stamina so my child can actually go to school for the whole day without a nap or a breakdown. Before the cocktail this wasn't even possible.
- Slightly increased energy, tremendous improvement in gut motility (went from almost no stomach motility to being able to eat by mouth during waking hours, g-tube fed at night instead of j-fed around the clock).
- We believe the mito cocktail helped to stop our daughter's seizures. After being on the cocktail for 6 months we were able to reduce her seizure meds from 3 different types to just 1. She also began to gain weight and have more energy.
- Before I took the cocktail I could not function at all. Now I have some good days. My endurance is limited but I can at least do some activities. I think I would be homebound without the cocktail.
- The carnitine has helped with the ketotic hypoglycemia greatly. The CoQ10 GREATLY reduces fatigue, and increases energy to keep body systems running smoothly. We are not sure if the Riboflavin plays a significant part, but use it to help with headaches.
- Increased energy, slowing of disease progression, decreased headaches, stopped tremors, decreased muscle pain.
- It's not that there have been changes that are noticeable. Instead, I feel that her condition isn't progressing as rapidly as we'd feared when she was diagnosed.
- I wasn't sure if the Mito cocktail was helping with any of my symptoms until I had to discontinue it for 2 weeks due to a study I was participating in. Once I resumed the cocktail I noticed I was able to walk farther, concentrate more (I could focus on conversations again), and had overall improvement in stamina.
- My neurologist at the MD clinic refuses to prescribe the cocktail due to research not supporting its benefits. :(
- I cannot take the Mito cocktail because my disability income is barely enough to keep the bills paid and my insurance will not cover it no matter what I try.

6. Based on your experience, which of the following do you feel helps you the most?

Total Number of Respondents: 337

Value	Count	Percent
Coenzyme Q10,	193	57.3%
Carnitine	149	44.2%
Other	51	15.1%
Riboflavin(B2),	42	12.5%
User Provided No Response	36	10.7%
Creatine	28	8.3%
None of these	24	7.1%
Lipoate, (Lipoic Acid)	24	7.1%
Thiamine-HCl (vitamin B1),	17	5.0%
Pyridoxine (B6),	15	4.5%
Vitamin C,	11	3.3%
Vitamin E,	6	1.8%
D-Ribose	3	0.9%
Selenium (metal cofactor)	2	0.6%

7. Based on your experience, which of these side effects do you find troublesome due to ingredients included in the "Mito Cocktail"?

Total Number of Respondents: 337

Value	Count	Percent
Stomach upset, nausea, vomiting	107	31.8%
User Provided No Response	104	30.9%
Body odor	90	26.7%
Disrupted sleep	61	18.1%
Diarrhea	46	13.6%
Agitation, hyperactivity	32	9.5%
Headache	24	7.1%
Staining of the hands, teeth, or skin	16	4.7%

Dizziness	12	3.6%
Skin rash, allergic symptoms	7	2.1%

8. What product form do you prefer?

Total Number of Respondents: 337

Value	Count	Percent
Capsule	168	49.9%
Liquid	162	48.1%
Tablet	80	23.7%
Powder	33	9.8%
User Provided No Response	10	3.0%

9. When discussing the "Mito Cocktail" with your physician, were you given an option or opinionated prescription (a recommendation of supplements and vitamins to try based on your physician's expertise)?

Total Number of Respondents: 337

Value	Count	Percent
I was given a prescription	207	61.4%
I was given a few different options	93	27.6%
A doctor did not prescribe the supplements; we decided to try it on our own	29	8.6%
User Provided No Response	8	2.4%

10. How do you obtain the vitamins and supplements in your or your child's "Mito Cocktail" each month? Please check all that apply.

Total Number of Respondents: 337

Value	Count	Percent
I get them through my local pharmacy	152	45.1%
I order them online from a specialized vendor	113	33.5%

They are compounded by a compounding pharmacy	93	27.6%
I buy them separately and crush or mix them myself	88	26.1%
User Provided No Response	16	4.7%
I am not sure where they come from	2	0.6%

11. If you were taking vitamins or supplements as part of a "Mito Cocktail" at some point and then discontinued, please explain why. (Choose all that apply).

Total Number of Respondents: 337

Value	Count	Percent
User Provided No Response	187	55.5%
Cost - I wanted to take it but couldn't afford it	69	20.5%
Cost - Insurance wouldn't pay for it	65	19.3%
Benefit - I couldn't tell a difference	36	10.7%
Benefit- I didn't feel it was working	35	10.4%
Cost - I didn't feel it was worth it	24	7.1%
Benefit - The difference I (my child) felt wasn't worth the cost or side effects	19	5.6%

12. If you or your child were taking the "Mito Cocktail" and stopped because you saw minimal benefit, how many months were you taking it before you stopped?

Total Number of Respondents: 337

Value	Count	Percent
User Provided No Response	250	74.2%
longer than a year	33	9.8%
six months to a year	12	3.6%
one to two months	12	3.6%
four to six months	12	3.6%

two to four months	11	3.3%
less than one month	7	2.1%

13. What aspect of the Mito Cocktail treatment presents the most difficulty in terms of dosing or compliance?

Total Number of Respondents: 337

Value	Count	Percent
Expense	160	47.5%
Taste	41	12.2%
Side effects	25	7.4%
Difficulty finding it	11	3.3%
Other	100	29.7%

Consolidated additional responses:

- Insurance won't cover it
- Size and number of pills
- Difficulty swallowing it
- Concerns regarding inert ingredients, quality and consistency of OTC supplements
- Certainty that it is actually working
- Difficulties in determining and finding the proper dosages
- Problems with sourcing all of the different components
- Remembering to take all of the different pills; feeling that patient is constantly taking something

14. Have you ever participated in a quality of life survey with your or your child's physician? If yes, please choose which one from the list.

Total Number of Respondents: 337

Value	Count	Percent
No, I have not participated in a quality of life survey for my child or myself	294	87.2%
I'm not sure	26	7.7%
Yes The Newcastle Mitochondrial Disease Scale	8	2.4%
Yes, the PedsQL	4	1.2%

User Provided No Response 5 1.5%

15. Do you think it would be helpful if your doctor had a way to measure whether the "Mito Cocktail" was improving your or your child's symptoms?

Total Number of Respondents: 337

Value	Count	Percent
Yes, that would be helpful	279	82.8%
I'm not sure/No opinion	36	10.7%
No, that wouldn't be helpful to me	14	4.2%
User Provided No Response	8	2.4%

16. Have you or your child had blood tests to measure the levels of various components of the Mito Cocktail, such as blood levels of CoQ10 or carnitine?

Total Number of Responses 337

Value	Count	Percent
No, I or my child has not had blood tests specifically to measure levels of components of the Mito Cocktail	152	45.1%
Yes, the tests showed that there was a difference in blood levels	99	29.4%
Yes, but I'm not sure of the results	57	16.9%
Yes, but the tests did not show a difference in blood levels	24	7.1%
User Provided No Response	7	2.1%

17. Do you feel that you have enough information about the risks and benefits of specific vitamins/cofactors/anti-oxidants to make an informed decision about whether or not you or your child should take them?

Value	Count	Percent
No, but I follow my physician's recommendation	115	34.1%
Yes, I researched this information on my own	112	33.2%

Yes, I was given information by my physician	97	28.8%
No, that is why I do not take these supplements	8	2.4%
User Provided No Response	5	1.5%

18. If you answered "No" to the above question, what type of information and resources do you think would be beneficial?

Representative Responses:

- There is a lot of conflicting advice from different Mito doctors. It would be helpful if there was a way to compile "helpful" Mito cocktail components into the NAMDC or some other database that all physicians and patients could access to see "data" on what has helped others and what side effects or benefits were noted (along with percentages of each).
- It is hard to tell how something will affect my child until we try it. Every Mito child is so VERY different. We are always willing to try something new, but we quit if we don't see improvements over the next few months after starting it. I never know what the bad side effects might be, so if we don't see "good" ones - we stop.
- Information on side effects of the supplements, especially when taken in such high doses and for long periods of time. I would like to have that information provided by the compounding pharmacy and discuss it with the doctor as well.
- A full medication & supplement review including supplements taken that were not recommended for compatibility, side effects, possible alternatives, ease of compliance.
- Typical risk/benefit analysis that we do for prescribed medicine should also be for vitamin regimen.
- Any possible negatives for taking the cocktail - do any of the supplements have an overdose level and who knows how to monitor that?
- Studies regarding the percentage of patients who show a benefit -- and how long it took before the benefit kicked in.
- I think it would be beneficial to notify patients what each of the different components of the cocktail does for the mitochondria specifically. This would help us be more knowledgeable and able to explain to other specialists. It is hard when you go see a new doctor and tell them the meds in the cocktail and they want to know what each of them do and you are unable to give an answer to them.
- I have been envisioning a checklist that the doctor could hand out at appointments. In my mind, I see it as having a list of the commonly prescribed supplements, vitamins and prescriptions. There would be a place for him/her to write the dose and a box that shows how to gradually build to that dose (i.e. 100 mg with breakfast for 7 days then add 100 mg with supper on the 8th day, etc.). I would really love to see a list of "look for these negative side effects and do xyz if you notice them." I also think it would be so incredible if this sheet would say things like, "best to take with food in a.m.," "do not take after 5:00 pm because it causes insomnia," "do not take this with that," etc. Right now, I scribble notes at appointments, read the note when it comes, do research on the Internet, talk to as many Mito moms as I can and then e-mail the doc some questions - and then I add the new supplement without every feeling like I am giving her the right things at the right time. I also live with a lot of doubt if I cannot distinguish enough of a difference when I add yet one more thing to her daily pills, powders, liquids and capsules.

- I feel that more information should be provided on sufficient serum levels of Mito cocktail components and also on the side effects and benefits of supplementation.
- When buying off the shelf or online you have no idea what you are buying. It would be great to have a list of vendors who have been proven to have a quality product.
- What is "the cocktail?" What is recommended for adults? If primary care doctor is left with managing care how can they learn what should be in cocktail?
- Something explaining long-term effects of vitamins, etc. Is there a point when it won't work anymore?
- Some concrete information as to how taking these help my child. Also, if buying the expensive stuff is better than the generic. For example, I pay \$130 for a bottle of liquid CoQ10. Knowing whether or not this is worth it would be nice!
- New information is coming out all the time on the long-term effects of things. It worries me that no one but me seems to keep up with this stuff.

19. Many people with Mito have good days and bad days. Do you make any adjustments to your "Mito Cocktail" on "bad/sick days"?

Value	Count	Percent
No	244	72.4%
Yes	82	24.3%
User Provided No Response	11	3.3%

20. If you were asked "What helps you/your child the most with the symptoms of mitochondrial disease?" what would you say? Please elaborate.

- Rest
- Adequate sleep
- Hydration
- Proper nutrition
- Frequent meals
- Mito Cocktail
- Avoiding mito-toxic drugs
- Precautions against other illnesses
- Therapies
- Good routines
- Managing balance of activity and rest
- Exercise
- Decreased stress
- Some combination of above
- Nothing

Here are some representative comments from those who mentioned the Mito Cocktail:

- Rest, hydration, cocktail, taking it easy when not feeling well, homeschool.
- His therapies coupled with the Mito cocktail.
- Hands down the Mito cocktail has made the biggest difference. Before beginning Vitamin E in combination with the CoQ10, my child could only walk about 10 feet. After a month of adding vitamin e she was practically running 30 feet. Even the therapists noticed a difference.
- The "cocktail" it has certainly improved his stamina and over all activity level.
- The Mito cocktail gives her more energy. A regular diet and balance in stress and activities.
- Definitely the Mito cocktail. When it has been stopped temporarily (by medical team for various reasons) the increased symptoms and overall decline has been rapidly apparent.
- The Mito cocktail, a good night's sleep, naps when needed, and feeding him at least every three hours with a feeding pump running all night. When we don't feed him that frequently, he has seizures.
- Good nutrition (elemental enteral formula), medications, Mito cocktail, and rest. When well enough, the use of motorized exercise equipment designed for wheelchair user that allows passive, active, and resistance exercise of arms & legs. Isometric exercise in bed.
- Closely following the directives of our doctors, staying on top of sicknesses, treating as soon as symptoms arise, staying well hydrated, consistently taking Mito cocktail and other medications.
- Sleep, Mito cocktail and overall nutrition.
- The Mito cocktail, creatine when having bad days and exercise when she has the energy.
- The Mito cocktail helps the most -- after taking them, we notice a big difference in energy and not being so sick all the time as before.
- Considering I have not been given the opportunity to try the cocktail, I have to say adapted exercise.
- I noticed a huge difference in my child the first six months on the cocktail. His color changed, his health improved, he was only sick twice this winter. I would say carnitine, avoiding sugar, increase protein and plenty of sleep (10 Hrs. min).
- The Mito cocktail helps to reduce the amount of time of exacerbations as well as the severity of symptoms. Keppra (NOT generic) is great in controlling seizures and does not have to have routine blood levels drawn. The L-Arginine in the Mito cocktail has helped decrease the recovery time of the stroke-like episodes.
- I feel that the combination of the "Mito" cocktail and as much physical activity as tolerated has helped my daughter the most.

21. Do you give permission for your answers and comments to be shared in an anonymous way for the purpose of educating the medical community and other providers about patient's approaches to managing mitochondrial disease?*

Value	Count	Percent
Yes	337	99.1
No	3	0.9

* Note: The survey results published herein exclude all responses for which the participant declined to share information with the medical community and other providers.