

September 19, 2011

To the members of the Committee, regarding Bill H320, An Act Providing for Care and Treatment of Patients with Mitochondrial Disease:

Thank you for the opportunity to share my experience as a mother of an affected child, a nurse, and an advocate for thousands of patients and families who suffer from mitochondrial disorders.

When I asked our clinician, patient and family community to show their support of legislation which would provide for care and treatment of patients with mitochondrial disease, the response was overwhelming.

Hundreds of families in Massachusetts are paying hundreds of dollars in monthly out-of-pocket expenses for the vitamins, supplements and medical foods which are the cornerstone of treatment for their child or family member's mitochondrial disease. In many cases, insurance denies part of all of the related monthly cost.

Would we deny insulin to a person with diabetes? Would we ignore the potential to add ten years of quality life to a child with cystic fibrosis, even if the mechanism of action for the therapeutic agent wasn't fully understood?

Vitamins, supplements, co-factors and medical foods for mitochondrial disease are not "optional". The words "vitamin" and "supplement" are misleading, implying that use of these compounds are in some way navigated by the patient and are not clinically useful or acknowledged. In fact, the opposite is true. The combination of these vitamins and supplements is known as the "Mito cocktail" and is definitively life-changing for a great majority of patients and families. Further, research in the field of mitochondrial medicine is almost exclusively focused on further development of these compounds as well as improvements in testing and diagnosis.

While taking the Mito cocktail, patients report *marked improvements* in their most troublesome symptoms, including muscle pain, extreme fatigue, seizures, vomiting, profound weakness, and stroke-like episodes. Several clinical studies and trials have demonstrated clinical improvement in children and adults with mitochondrial disease. It is thought that use of these compounds can prolong life, stave off disease progression, and offer patients with mitochondrial disease the opportunity to have improved and prolonged quality of life.

Many patients are forced to choose between the mortgage payment and their "Mito cocktail". The compounds used in the Mito cocktail are taken daily, and can cost families anywhere from \$300 to \$3000/month. Too many families are literally losing their homes

in order to keep their children alive because insurance has denied coverage due to a lack of recognition and understanding of mitochondrial disease.

Further, the potential cost-benefit of providing coverage for the vitamins and supplements for mitochondrial disease is clear. Without the opportunity to stabilize symptoms and disease progression using the only recognized treatment approach for mitochondrial disease, patients will cascade more quickly into a metabolic crisis, requiring more frequent and prolonged hospitalization, invasive procedures and testing (MRI, feeding tube, etc.). It is not uncommon for people with mitochondrial disease to “crash” and require extensive and expensive hospitalizations and ICU stays. For the children and adults who are responsive to the vitamins, supplements and medical foods recommended for mitochondrial disease, such hospitalizations and symptom progression can be dramatically improved or altogether avoided by use of the “Mito cocktail”. Compounding pharmacists in MA calculate that the cost to insurance providers of the Mito cocktail is, on average, \$400/month. On the other hand, the cost of more frequent clinic visits, prolonged hospitalizations, IV fluids, ER visits, and support for patients with multiple organ failure begins at \$20,000/month. Many families in MA have MassHealth as a secondary insurance.

Finally, Massachusetts has the opportunity to once again lead the nation by approving H320. Families with mitochondrial disease across the nation are struggling. They are financially devastated by the cost of the only treatment available to them. These families recognize the benefit of the supplements, but are forced into a difficult position due to the overwhelming out-of-pocket expense. *“When forced to choose between medicines that are saving my wife and my child’s life or Christmas presents, of course I choose the medicines. But how do I explain that to our children?”*

Thank you for your support,

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