

David L. Brown, MD  
Harry C. Weinerman, MD  
Felicia M. Wilion, MD  
Viviann M. Rubin, MD  
Maury R. Luxemburg, MD

Margot H. Dayton, MD  
Catherine Worthley, APRN  
Lois E. Heinemann, APRN  
Nirupa Sekaran, APRN  
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To Whom It May Concern:

*I have been asked to offer a letter in support of proposed bill # 6580 concerning the establishment of an advisory council to coordinate the study of the impact of rare diseases on our population. I have cared for the Harwood children since their births. The eldest Maren was diagnosed as having MCAD, a rare disorder of metabolism that if untreated is usually fatal. Her diagnosis was suspected due to our state's excellent program of neonatal screening which alerted us to her diagnosis. Despite our ability to establish the diagnosis and save her life, her parents and caregivers were faced with innumerable barriers to care due to the lack of resources, and knowledge of her disease and the need for comprehensive care. Happily, Maren has done wonderfully and is a beautiful, happy and accomplished child. I fear that without diligence and love provided by her parents and caregivers this would not have been the outcome for her.*

*As providence would have it, Maren's younger sibs did not have the same disease as she does, but an even more difficult genetic disorder affecting the function of their mitochondria which are elements of all cells that regulate the production of the energy that is needed to sustain a normal life. Their diagnosis was even more difficult as there was and still is not a simple test to identify this disease nor are their clear guidelines as how to treat this lifelong affliction which affects virtually every cell and organ system in the human body.*

*The Harwood children have suffered many emergency room visits, hospitalizations, blood tests and surgeries to biopsy skin, and muscle as well as placement and of feeding tubes inserted through their abdominal wall into their stomachs. Otherwise competent and well-meaning physicians unfortunately made errors in diagnosis and care of these children due to lack understanding and training concerning this class of rare diseases. The childrens' care required expertise not available in our state and their parents were forced to arrange care out of state and advocate for insurance coverage for these visits.*

*Although the children are now doing well, they may still have medical challenges that remain poorly understood and for which we may not have the adequate resources available.. At this point in time, the out of state specialist is no longer able to provide the costly consultations that have been so important for these children and we may not be able to easily replace that element of care. Unfortunately, there are probably many children in Connecticut who suffer from rare diseases similar to MCAD and mitochondrial disease that have not had the good fortune to have the family and medical support that the Harwoods have had. These families and their care givers would greatly benefit by the establishment of an advisory council to coordinate the study status of our abilities to care for our citizens with rare diseases.*

Sincerely,



David L. Brown, MD, FAAP  
Clinical Professor of Pediatrics, University of Connecticut School of Medicine

6 Northwestern Drive  
Bloomfield, CT 06002  
860-242-8330

546 Cromwell Avenue  
Rocky Hill, CT 06067  
860-529-6124