

**Date:** 2/17/2015

**To:** Connecticut General Assembly's Public Health Committee

**Hearing Date:** 2/23/2015

**From:** Debra A. Gove

**Subject:** Support for House Proposed Bill No. 6709, AN ACT CONCERNING THE RIGHT TO TRY EXPERIMENTAL DRUGS.

The world was our oyster! We had the plan. We had the boat. We got married. All we needed was to sell the house and wait for Tim's pension to start in September 2016. Then everything imploded! Life is what happens while you are busy making plans.

First the plan. In December of 2009, we signed a contract to have our retirement / dream boat built, a 43 foot pilothouse trawler. We took delivery of Ningbo Cruiser, in August of 2010 and started on a series of planned upgrades and refinements to get her ready for use as a live aboard and full time cruiser. Our plan was to sell the house and head down the coast of the US and continue on to the islands for an adventure of a lifetime!

During our winter layup in February 2014, while we were doing another upgrade, I was moving a box when my left arm just stopped working! Our first stop was our primary care provider (PCP), where I was immediately referred to a neurologist, Specialist #1.

Specialist #1 prescribed blood work, an MRI, and electromyography (EMG). For those not familiar with an EMG, it is a VERY invasive test of the electrical activity generated by the skeletal muscles. It uses a long needle, inserted into every muscle group in the body, from head to toe. Even the tongue! It took two visits to complete the entire first test. The blood work and MRI showed nothing remarkable.

Almost a year ago, March 2014. Specialist #1, believe it or not, called me on the phone at work, an elementary school in Durham, to tell me I may have a degenerative neuromuscular disease and should go to the Hospital for Special Care (HFSC) in New Britain for a second opinion. The doctor also told me someone would call to set up an appointment. We waited! We worried! We hoped it was a mistake! No call. Specialist #1 had dropped the ball, so we arranged for our own appointment for a second opinion. We should note that there is a neurologists' handbook which states that a patient should NEVER be told over the phone. It should be done face to face in an office setting. We filed a formal complaint.

On April 10, 2014 we went to the HFSC for an initial consultation with Specialist # 2. Specialist #2 reviewed all of the materials from the PCP and Specialist #1. Another EMG was scheduled for visit two. At visit three, the diagnosis of a degenerative neuromuscular disease was confirmed, but never named. It wasn't until we gave the doctor a list of various diseases that it could be that we were told, "from now

on I will refer to it as Amyotrophic Lateral Sclerosis, ALS, Lou Gehrig's disease!". A death sentence! The three of us cried.

We were told there was only one FDA approved medication for the treatment of ALS, a drug called Riluzole. Specialist #2 said, "If you can afford it (\$2,000/month) it will only extend your life by a couple of months, and it is not the months you want". I was told to take Tylenol for the pain and given a prescription for antidepressants.

Needless to say, we have not gone quiet into this dark night, nor have I taken the antidepressants! On to Mass General Hospital and Specialist #3. Again the diagnosis was confirmed, but we were told Riluzole was recommended for all of her patients, "but not if you have to sell the house to afford it". Luckily we have excellent medical insurance and can afford it.

In the meantime, Specialist #3 referred us to a number of different studies. I feel a responsibility to participate in every study I can so I can help researchers develop an understanding of this disease and maybe help others. So after numerous studies, which involved: another MRI, two hours; more blood tests, I am now up to fifty-two vials; DNA tests; saliva samples; urine samples; and countless questionnaires, strength tests, and specialists we didn't even bother to number, I found information on an actual FDA approved clinical trial being conducted in the US that may actually be a treatment that could help.

BrainStorm Cell Therapeutics, an Israeli company, is conducting an FDA Approved Phase 2 Safety Trial of forty-eight subjects that involved collecting a subject's bone marrow, extracting the stem cells, using a proprietary process to grow and treat the stem cells, and then transplant the subject's own stem cells back into their spines and arm muscles (**more info can be found at <http://www.brainstorm-cell.com/>**). These trials were being conducted at three US locations. Two of which were close enough for me to qualify as a subject. This generated a heartfelt letter to Specialist # 4 asking for a chance to "make history" with him. Sadly, Specialist #4 transferred off of the trial, but not before referring me to Specialist #5.

This brings us to September 2014, we went to UMass Memorial Hospital in Worcester, MA for an initial consultation with Specialist #5. After his initial assessment, he asked me, "are you here for a second opinion, or are you interested in the trial". We assured him we had plenty of opinions, I wanted into the trial! At this point, I was told I looked like an excellent subject, healthy in every regard, except for the ALS, at which point Specialist #6 was called in to do her initial evaluation.

Now it is less than 12 months since my diagnosis. I can't work and have started collecting Social Security Disability. ALS is one of only two diseases that qualify for immediate approval, no questions asked. Tim has taken an early retirement in order to spend time with me. Sadly, a lot of that time is spent in hospitals, clinics, and doctors' offices. We have replaced all of the door knobs in the house with levers so I don't get trapped in the bathroom or bedroom. Everything needed for my daily activities, plates, glasses, cups, microwave etc. are placed on the counters or the lowest cabinet shelves because I can no longer lift anything above shoulder height. I have an ankle brace and cane so I don't trip and fall again. I

can no longer go grocery shopping alone because if I manage to get something into the cart, I can't always lift it back out. Our plans are on indefinite hold.

Last week I spent three days in the UMass Memorial Hospital having 125 million stems cells injected into my spine and 24 places on my right arm. Now we wait, we worry, and sometimes we cry. Another concern, there is a 25% chance I have received a placebo. Nobody knows, not even the doctors, which is why this was called a double blind trial. As a result, we continue tilting at windmills and pursuing every possible form of treatment we can find. To quote Neil Young, "It is better to burn out, than fade away".

Although I will probably never benefit from a Connecticut Right to Try Law, it makes no sense that a terminally ill patient can't avail themselves of any drug or treatment with even the slightest chance of helping. Connecticut already requires insurance companies to pay for experimental drugs, why shouldn't it make it easier for those patients to receive those drugs?

Thank you for your consideration in this matter,  
Debra A. Gove