

HB 6709

Support "Right To Try"

To the Editor:

One year ago my friend and fellow Durhamite Debra Gove was moving a box when her left arm mysteriously stopped working. A visit to her primary care physician and two specialists later, she got about the worst diagnosis I can imagine: Deb has Amyotrophic Lateral Sclerosis, also known as Lou Gehrig's Disease.

ALS is a neurodegenerative disease that affects nerve cells in the brain and the spinal cord. Motor neurons reach from the brain to the spinal cord to muscles throughout the body; when they die the ability of the brain to initiate and control movement is lost, resulting in muscular atrophy and eventual paralysis. There is no cure for ALS, but the FDA-approved drug riluzole may slow the disease's progression. Several other drugs in clinical trials hold promise.

ALS usually strikes people between the ages of 40 and 70, and as many as 30,000 Americans have the disease at any given time. A short list of famous victims includes Hall of Fame pitcher Jim "Catfish" Hunter, Senator Jacob Javits, actor David Niven, Sesame Street creator Jon Stone, musicians Lead Belly (Huddie Ledbetter) and Charles Mingus, entertainer Dennis Day, former U.S. Vice President Henry A. Wallace and Army General Maxwell Taylor.

My friend Deb is in no rush to join this group! Husband Tim has left his job to support her battle with ALS; sadly, instead of the dream retirement they had planned aboard their hard-earned, custom-made boat, time is now spent in doctors' offices, hospitals and clinics. Doorknobs in the Gove's home have been replaced

with levers, and Deb wears an ankle brace and walks with a cane. She volunteers for every trial and treatment available to her; she is poked and prodded and injected at every turn. Currently she is participating in an FDA-approved Phase 2 Safety trial: forty-eight subjects had their bone marrow collected, their stem cells extracted and treated, then transplanted back into their own bodies. Deb spent three days in UMass Memorial Hospital (in Worcester) having 125 million stem cells injected into her spine and 24 places on her right arm. Now she and Tim wait, worry, and sometimes cry – and it's possible that Deb is in the control group, having gone through all this without actually receiving any treatment.

Which brings me to “Right To Try”: legislation which would allow people diagnosed with incurable conditions to receive experimental drugs that have not completed the Food and Drug Administration's approval process. A lawsuit attempting to force the FDA to expand the availability of investigational drugs failed; a federal judge disagreed that terminally ill people have a right to access to investigational medicine and the U.S. Supreme Court declined to consider an appeal. The FDA does have an “expanded access” program, but only for people who are rejected by clinical trials and can hang on while the bureaucracy processes their request. State-level approaches are an effort to circumvent the federal system.

Clinical trials attempt to establish the safety and efficacy of new medicines compared with placebos; the later-stage studies help scientists determine proper dosages and understand how a drug affects patients who may be taking other medications. However, it takes about 10 years and \$1.2 billion to win FDA approval for a compound, and only 16 percent of treatments that begin clinical trials ultimately hit the market. Pharmaceutical companies are not obliged to release drugs that are still in the approval pipeline, but in some cases a simple doctor's prescription is sufficient to cut

through the red tape. Putting myself in Deb Gove's shoes, I'd certainly like to have that chance!

Our own State Representatives Vincent Candelora and Noreen Kokoruda have introduced Right To Try legislation which is now under consideration by the Committee on Public Health. And – more luck – our State Senator representing the 12th District, Ted Kennedy, Jr., is on that committee (so is Mr. Candelora)! But this is not a “done deal.” Critics argue that patients will avoid clinical trials and that some will die from taking medications that have not been fully vetted. Insurance companies are not obligated to pay for non-FDA approved drugs and may cancel or suspend coverage while such drugs are being used. Certainly a “proceed with caution” approach is called for, but for people like Deb time is of the essence. And look at it this way: every individual who tries an investigational drug makes a contribution to the knowledge base on that drug.

Life takes some unexpected turns, and they aren't all good! Please contact your legislators and urge them to support “Right To Try.” This link will bring you to the right place: <http://www.cga.ct.gov/ph/>, or email phctestimony@cga.ct.gov. You never know when you or a person you care for may want this opportunity.

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