CellR4 2013; 1(2): E403

LACK OF PROVEN EFFICACY: A LEGITIMATE REASON TO DENY ACCESS TO CELLULAR THERAPIES OR A BARRIER TO INNOVATIVE TRIALS AND THE DEVELOPMENT OF CURES?

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Keywords: Stem cells, Cell therapy, Multiple sclerosis, FDA, Regulatory, Patient advocacy.

Our first mission at PFSC[1] is to change the negative drumbeat in the press that all stem cell therapy is quackery. We quickly did that, just by telling our own stem cell patient stories, with interviews in BusinessWeek[2], Forbes[3] and NPR[4].

A similarly complex situation over autologous stem cell therapy is unfolding in Italy, and the CellR4 journal is wisely pointing out the biased journalism tactics that are clouding the debate[5,6].

Poor journalism leads to confused public opinion and lack of public input into developing regulations. This permits the FDA to operate behind a shroud, which has led to flawed, overzealous regulations that limits access to this lifesaving therapy. We explored this regulatory overreach in our post "The History of FDA Control of Your Body"[7], which covers the midnight change to 21 C.F.R. 1271.3(d), classifying our own autologous stem cells as investigational new drugs, and forcing patients with no other medical options back into stem cell medical tourism.

The FDA accomplished this without the mandatory public commentary in 2006. Consequently, when we found out there was a comment period on a draft guidance that covered this issue, we put out the call, Take Action by July 8th: Tell The FDA Your Stem Cells Are Not A Drug![8] Initially, there were only two comments in the Federal Register; in five days patients added another fifty comments, focusing on the remedy put forth by Mary Ann Chirba[9], who is a Professor of Healthcare Law at BC College.

I helped form Patients For Stem Cells, along with other no-option patients, because I feel driven to help correct this egregious regulation because I now know firsthand that this therapy can help chronically ill people. In May of 2012, I was treated in an IRB monitored study sponsored by Celltex Therapeutics, a company that multiplies and stores adult stem cells. The infusions of my own adipose derived mesenchymal stem cells, expanded to a therapeutic dose, was to treat multiple sclerosis, which I've had since 1995, which had become progressive and disabling since 2002. None of the FDA approved drugs helped my condition. My legs were so stiff I was on the verge of complete paraplegia, and I knew a nursing home move was looming. My stem cell treatment has yielded a sustained recovery in thirteen of my twenty-eight symptoms, including heat intolerance, fatigue, constipation, urinary incontinence, numbness, spasms and spasticity. I've now thrown away my nursing home brochure collection. "Recovery" is not even in the medical lexicon when it comes to progressive MS, so this is a significant finding that can't be ignored.

While this study was in progress, the FDA issued a warning letter to Celltex, based on the cells = drugs policy, causing termination of the study, and leaving patients that were already scheduled for treatment stranded. Many of them were MS patients like myself, failed by all other FDA approved drugs and now out of options.

To define what "no options" means, there is no efficacious pharmaceutical treatment for progressive multiple sclerosis. Novantrone, the single treatment approved for progressive MS, has a black box warning that it could cause possible heart damage and Leukemia. This drug does not stop the disease[10]. I had this treatment in 2002, until my heart function decreased to the point where I had to stop. I derived no benefits from this drug, only detriments, and started using a cane for the first time while I was on the drug.

Every other pharma treatment has been tested and approved for relapsing-remitting MS (RRMS) using invalidated surrogate markers[11]. Hence, any drug

other than Novantrone is by definition "experimental" and of unproven effectiveness for the prevention of further progression for those with progressive MS.

Researchers have been studying treating MS patients with expanded numbers of stem cells in MS, including progressive MS, for years with some effectiveness, even with non-repeated and relatively small doses (a single dose of 63 million MSC in one study)[12].

Dr. Slavin is one of the leading researchers on MS. He is very influential and has over 500 papers to his credit in his field, and has established an independent clinic to treat people in Israel with different cell therapies including expanded mesenchymal stem cells (MSC) for MS[13]. This demonstrates how promising this treatment option is for those of us with progressive MS.

I have already been waiting years for something like this to be available; it is unethical to tell me I must wait with no therapy, or leave no other option but to travel in my wheelchair to overseas clinics like Dr Slavin's. People with MS are competent and can understand the nature of "experimental" treatment; they already decide with their doctors to take drugs approved for RRMS, some with lethal side effects, on the chance it might help their progressive disease. As with all clinical medicine, the proof is in the eyes of the patient and the doctor.

To assume an MS patient cannot make this decision with one's own doctor is to push progressive MS patients towards dangerous pharmaceuticals that lack proof of efficacy, as if lack of proven efficacy is inconsequential for pharma drugs, but somehow a legitimate reason to deny a patient the right to a empirical trial of expanded MSC.

Leaving progressive MS untreated is not "safe." It's time for people to accept that in some diseases the consequences of denying access to therapy and claiming it is for their own good is a cruel joke.

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