Quilters Dream Batting®

"Quilts Like A Dream"
589 Central Drive
Virginia Beach, VA 23454

Hopes & Dreams Finding a Cure for ALS (Lou Gehrig's Disease)

Testimony of Kathy Thompson on 2/25/2013

Food and Drug Administration (FDA)
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Considerations Regarding Food and Drug Administration Review and Regulation of Drugs for the Amyotrophic Lateral Sclerosis; Public Hearing

My name is Kathy Thompson and in early 2007 my wonderful 32 year old son Josh Thompson was told to get his affairs in order. Josh was diagnosed with ALS on his one year wedding anniversary and two weeks before the birth of his first child, Wyatt. To say we were devastated is a true understatement. We were even more shocked to learn that in this day and age there was not a single effective treatment available.

Faced with the hopelessness and horrible realities of ALS, while Josh was being tested to eliminate possible disease mimics, we found ourselves praying - please let it be cancer, AIDS, MS - anything other than ALS that has a treatment, that gives us a fighting chance. We would rather have faced any of these awful diseases than have a disease with no treatment.

We were then given a glimmer of hope when we were told by doctors at both Hopkins and Duke that the Arimoclomol trial is the trial for Josh. But we waited and waited for the trial that never was.

We then fought for Iplex, but our applications for compassionate use were denied. We filed a legal appeal and waited several months, all while Josh's' physical state was severally declining. We finally were granted a limited supply of Iplex, which by the way helped Josh in so many ways. Sadly, only other patients showed interest in our results with Iplex, not the FDA, not the ALS medical community.

Josh is now on a ventilator and feeding tube. With Josh by our side, our family has raised millions of dollars for ALS. However, by the current standards of the FDA, the Pharmaceutical companies, and many in the medical community my amazing kind intelligent son is considered a "throw away patient."

He, like thousands of ALS survivors and many of these incredible patients here today, is no longer eligible for most ALS clinical trials designs. So, we are left to our own devices to fight and survive - relying on other "throw-away patients" as sources of inspiration, shared experiences and our own attempts at research and self- medicating. The ALS forums are an incredible source of information for us, and SHOULD be for YOU also.

I am here to respectfully request the following:

1. That the FDA and ALS community quickly devise a disease stage scale for limb and bulbar onset in both sporadic and familial ALS patients similar to the way cancer and other diseases are divided into types and stages for better more relevant studies. Define, recognize and respond to the patient subsets that experience benefits from trials.

(The current ALSFRS is vague and woefully inadequate and does not measure patients' reports of positive improvements or feeling better.)

Quilters Dream Batting®

"Quilts Like A Dream"
589 Central Drive
Virginia Beach, VA 23454

Hopes & Dreams Finding a Cure for ALS (Lou Gehrig's Disease)

2. Allow patients in advanced stages of ALS to be included in clinical trials - and monitor the trials accordingly. There could be a lot to be learned if a drug does not work in the faster paced progression of stage 1 and stage 2 of ALS, but extends survival in the slower paced progression of stage 4 ALS patients.

3. Expand the criteria of the trials to include patients who have taken life saving measures such as feeding tube and mechanical ventilation.

4. In ALS the cause of death is usually lack of air, nutrition or aspiration pneumonia from poor bulbar function. We first and foremost need drugs, devices, and or stem cells that allow PALS (persons with ALS) to sustain breathing, swallowing, speech and nutrition. This is what sustains life. Paralyzed patients can live a long time, but suffocating, starving, non- communicative people wither and die.

5. When a new investigational drug trial is not targeting the diaphragm, breathing and bulbar muscles - no matter how good the investigational drug may be - trial patients may not survive a possibly effective trial if life sustaining measures remain exclusionary in the trials

I am requesting that participating trial patients be allowed, even encouraged to get a feeding tube, diaphragm pacing or be tracheid & vented if and when necessary to sustain life. It is the only way the current line-up of drug trials can meet the survival criteria and for the drug to possibly eventually be part of the 'treatment cocktail' that we are all hoping and praying and working for.

6. Put the original intent of 'compassion' back in the compassionate use emergency IND approval process for the terminally ill. Understand and make exceptions for the extreme limitations caused by this disease. The current application process is biased towards the physically able and financially well off. As trial criteria's stand now, most patients here today may only be able to access investigational drugs and legitimate stem cells through compassionate use. I am requesting that the FDA add a special disability clause offering a simplified application process, delete the expensive and laborious and ineffective IRB process, and provide a patient representative to expedite, assist and follow the patient in the process for compassionate and emergency use treatment applications. The urgency in emergency is no truer than in ALS.

In closing - this young man, my son, lays there paralyzed and bed bound - everyday facing helplessness, fear, daily indignations, physical pain and discomfort. But he fights on, hoping beyond hope for a treatment with an eye stem cells.

When I read to Josh about Neurastem, Brainstorm, etc. he smiles. When I asked Josh what is the first thing he wants to do when he gets better, expecting that he would say something grand like go 'surfing in Hawaii', he spelled, "I want to hug my boys."

We need the FDA's urgent help to make that happen. In truth, our lives lie in your hands. We need you to trust these esteemed ALS researchers and doctors. We need you to trust us. We are all here to implore you to expedite the approval process and allow all ALS patients to have a chance at life.

Thank you,
Kathy Thompson

(757) 463-3264 • (757) 463-3569 Fax • (888) 268-8664 Toll Free • (800) 626-8866 24 Hour Fax
QuiltersDreamBatting@juno.com • QuiltersDreamBatting.com

Quilters Dream Batting®

"Quilts Like A Dream"
589 Central Drive
Virginia Beach, VA 23454

Hopes & Dreams Finding a Cure for ALS (Lou Gehrig's Disease)

EDITORS NOTE:

I had seven (7) recommendations listed, but let this one out of my presentation because it was covered by several other presenters.

7. Use historical controls from previous trials and/or from non-participating PALS. Placebo trials are too cruel and too expensive in dollars and human treasure in the face of ALS.

(757) 463-3264 • (757) 463-3569 Fax • (888) 268-8664 Toll Free • (800) 626-8866 24 Hour Fax
QuiltersDreamBatting@juno.com • QuiltersDreamBatting.com