An Open Letter to FDA Commissioner Margaret Hamburg, MD

Dear Dr. Hamburg,

I am writing first to thank you and your team at the Center for Drug Evaluation and Research (CDER) for your willingness to meet with – and listen to – me and the other mothers and families dealing with the horror that is Duchenne Muscular Dystrophy . We have made real progress and, for the first time, we have reason to be hopeful. But time is running out for all of us. And now, with less than a week before Dr. Russell "Rusty" Katz, the director of the Neurology Division, retires, we are worried that this unprecedented opportunity may slip through our fingers. And we can't let that happen.

My son Jett was diagnosed with Duchenne when he was five years old. Since then, he has steadily lost muscle function, moving to a power-chair at age 14. Jett is now 18 years old and has become a quadriplegic - time is running out for him, his heart and lungs will fail around the age of 20.

Each year, about one in every 3,500 boys is born with Duchenne Muscular Dystrophy. Most will die before their 20th birthday. Until now, Duchenne was a certain death sentence. However, a new drug – eteplirsen – has been shown to be effective in boys like Jett. You've seen the results: ten year-old boys who could barely walk have started to run; bodies that were failing to produce dystrophin – the vital muscle protein missing in Duchenne patients – are now demonstrating dystrophin production and functional benefit. It's unbelievable.

We have now had five groundbreaking discussions with Dr. Janet Woodcock and the CDER leadership, the result of the FDA Safety and Innovation Act (FDASIA). Throughout these conversations, Dr. Katz has been extremely sympathetic. Two weeks ago, when we showed him and his FDA colleagues video testimony – patient reported outcomes – to the progress these boys have made, we heard his encouraging words. **I may be wrong, but I am hoping that making eteplirsen available to my son and the thousands of other boys who need it now will be a fitting legacy for Dr. Katz, a wonderful end to an honorable 30 year career.**

We are well aware that accelerated approval requires more than stories of heartbreak and hope. We understand that science – not sympathy – must drive your decisions. But this drug is working in the 12 boys in the US who were lucky enough to be part of the trial. Now, nearly two-years into the trial, there have been no side effects. Boys with Duchenne are stabilizing…some are getting better. The risk of not doing anything, of not providing eteplirsen to those who would be amenable, is incomprehensible. Waiting for additional studies, while watching those with Duchenne lose function and life, is simply not acceptable.

Please, do not let this moment – this unbelievable chance of lifetime – pass.

Sincerely,

Christine McSherry

The writer leads the Jett Foundation and is a founding member of the Duchenne Alliance, an effort by over 20 organizations working together to find a cure for Duchenne Muscular Dystrophy. ***www.duchennealliance.org***