Laura McLinn & Jordan McLinn

Testimony for the HSGAC Hearing – Connecting Patients to New and Potential Life Saving Treatments

Thursday, February 25, 2016 10 a.m.

Hello. My name is Laura McLinn and this is my 6 year old son, Jordan McLinn. I will let Jordan say hello and he might want to say something else into the microphone. His ears are a little more, "mature" now so after he speaks I will let him put his headphones on and play on his iPad probably. ☺

You may recognize Jordan from a special Christmas wish he was granted in 2014. His story went viral after I put together a resume for him to be "hired" at a local fire station. The response was overwhelming as he started getting job offers, patches, shirts, letters and other gifts from fire departments all across the nation. He had a couple of interviews in our home state of Indiana and then found two job offer letters in his stocking on Christmas morning. He started going to "work" on a regular basis…eating meals at the firehouses, washing the firetrucks, fixing taillights and taking part in special trainings. He has even worked with the firefighters at some Nascar races in Charlotte, NC. At just 5 years old he was welcomed into a very special family...the firefighter brotherhood. Jordan is living out this dream as a child instead of at the normal adult age. Let's talk about why…

Jordan was diagnosed with Duchenne Muscular Dystrophy just a few months before his 4th birthday. It came as complete shock to everyone. The disease is 100 percent fatal. To look at Jordan now, you might not even realize he is any different than most energetic, active kindergartners. He is super smart, "runs" and plays with his friends, has more faith than anyone I've ever known and absolutely loves life. The reality, however, is that his muscles are slowly wasting away and without a miracle he will lose his ability to walk very soon. Jordan is missing some exons on his dystrophin gene so his body is unable to produce this very important protein. As a result, he is getting physically weaker every day. Most boys with Duchenne are in wheelchairs before age 10 and do not live past their twenties. Many do not even make it to twenty years old. Duchenne effects 1 in 3,500 boys. Without a miracle Jordan will lose the ability to walk, climb, dress himself, feed himself and he will even lose the ability to hug me. Many of you in this room are parents. I don't have to tell you how heart wrenching this diagnosis is. I also don't have to tell you how fast these childhood years go by for us parents. Jordan is in a race with the clock for his life but there is tangible hope at our fingertips…

For the first time in the history of Muscular Dystrophy there are promising treatments coming up through the pipeline. There are boys who are receiving exon-skipping treatments through clinical trials and it is working to slow the progression of the disease! These boys are walking, playing soccer, riding bikes as teenagers because of the exon-

skipping drugs they are receiving through these trials. This is unprecedented, unheard of in the natural course of this disease. These exon-skipping drugs have no safety issues and they ***are working***. Unfortunately, Jordan and many other boys may not get access to these treatments in time if we have to wait for the standard FDA approval process. Knowing that something exists that is safe and effective gives us hope but it also rips our hearts out because he's not able to get it yet. Jordan is already starting the decline phase of this disease at just 6 years old. He is getting tired faster, he often cries at night because his body hurts, he can't keep up with his friends, he is starting to fall more frequently, he can't do the things they are doing at recess and he doesn't understand why. We do not have time to keep waiting. With muscle disease once you lose function, there is so much damage and it's hard to regain any of that which is lost. This treatment is not just a pill he can take at some point in his future and then be okay all of a sudden. He needs it NOW, before he declines further. I want to see my son grow up. I want to see him be part of the first generation of boys to survive this. And that IS possible. Now, let's talk about the barriers and most importantly some solutions for how to make this happen…

Last spring, Jordan and I helped get the Right to Try Law passed in the State of Indiana. Jordan bravely stood in front of state lawmakers and told them to, "Please say yes". And they did. It passed unanimously in the house and senate. Our family and Jordan's firefighter family was there with Jordan when the governor signed it into law. It was a very special day for us because we felt like we had a new hope, kind of a back-up plan in case Jordan couldn't get the treatment through a trial or even better through accelerated approval. "Right to Try" basically says that if you have a terminal illness and a drug exists that could potentially save your life, you have the right to try it before it gets approval from the FDA. It has to have made it through a couple of important phases with the FDA showing it is safe. You can check out more about "Right to Try" and Jordan's story in Darcy Olsen's book, The Right to Try that was released last November. Jordan is featured in the chapter, "We are the 99 percent." Even though Jordan legally has the "Right to Try" now, the drug company has to be willing to give or sell him the drug. At this point, they are not open to doing that… for reasons that are understandable but not really okay for us at the same time. Maybe the drug companies are afraid to get sideways with the FDA and risk their billion dollar investment. I don't know. Unfortunately people die in the meantime because no company really knows what the FDA will do and the FDA is not really transparent about it. Therefore, the drug companies choose to be cautious. Patients deserve the, "Right to Try" to save their lives though when options are there.

I'd also like to talk about another pathway that can help Jordan. Back in 2012 Congress passed and President Obama signed the Food and Drug Administration Safety and Innovations Acts (FDASIA). FDASIA gave the FDA the backing and support they needed to more broadly grant accelerated approval for safe and efficacious therapies for rare or severe diseases that meet an unmet medical need, just like the exon-skipping drugs I've been talking to you about today… drugs for rare disease like

Duchenne. FDASIA mandates that the FDA include the patient voice in their review process. Sometime in the next few months there will be patients testifying in front of an FDA adcom panel and asking the panel members to endorse the approval of an exon-skipping drug that has shown to be safe and efficacious in clinical trials. Now… we want to see the FDA use the tools in FDASIA to grant accelerated approval to potentially life-saving treatments, starting with eteplirsen, Sarpeta's exon-skipping drug that could potentially be approved by the end of May. We need to hear a YES to this safe and efficacious therapy from the FDA in May. While this mutation-specific therapy will not be able to treat Jordan's mutation, future exon-skipping therapies will. We must start by approving these therapies as soon as possible, because Jordan does NOT have the time to wait.

Please, in order to help Jordan and all other boys living with Duchenne, encourage the FDA to use the tools you have given them to expedite life-saving drugs to patients. FDA has the flexibility and resources needed to approve these life-saving therapies, and they must use them in order to save children's lives. Additionally, if you are willing to stand with us at that adcom meeting and remind the FDA to use the tools you have given them, remind them to listen to the patients on the drug and the patients who want access to the drug, our family and the entire Duchenne community would be eternally grateful for your support.