

Good morning. I am Laura Manfre, co-Founder and President of Sofia Sees Hope, which has paid for my travel here this morning. We are a nonprofit organization that receives grants from many companies and Spark Therapeutics is one of them.

Sofia Sees Hope, named for my now 14-year-old daughter with LCA, is an advocacy organization representing patients and families with LCA and other rare inherited retinal diseases, including those affected with blindness caused by the RPE65 genetic mutation.

Founded in 2014, we provide funding for diagnosis and research to treat and cure LCA and provide outreach and education to families, enabling them to share stories, connect, and hopefully provide a little emotional relief from the isolation and devastation that this rare disease causes.

We hear from families whose children cannot make eye contact with their own parents and the devastating impact it has on the child and the entire family. We hear from kids who face social and academic challenges that range from bullying and exclusion to being perceived as less intelligent — when the only difference they struggle with is that they cannot see as well as their sighted peers. Even in the best of circumstances they are growing up with a tremendous pressure that most of us never had to — that they they will someday live in a world of complete darkness.

The emotional, social and educational toll of this vision loss at a young age is tremendous.

While certainly there is an urgency to approve Voretigene Neparvovec for our children as we know the benefits are greater when the retina is healthier, I want to share today how important it is to improve

- ANY amount of vision for
- ANY amount of time for

- ANYONE with RPE65 retinal dystrophy.

To this end, I have a letter here from Tami Morehouse, who is the oldest patient in the trial conducted at CHOP.

Tami was diagnosed with LCA almost 20 years ago. Her first treatment was done in March 2009, at age 44, and her second procedure was done in November 2010, at age 46. At the time of her trial she had lost so much vision that there were days she could only see the brightest of light.

Here are some excerpts from Tami's letter that she asked me to share with you this morning:

After my procedures, I no longer lived in fear. A huge weight was lifted from my shoulders. This became especially true when I began to see much more light, differences in color, movement, and more of everything around me in general. I was once again able to see such things as the faces of family and friends, some letters on the eye chart and the beautiful colors of a sunset over Lake Erie.

One of the most important experiences that I have had since my procedures happened on my last visit with my Dad. When I stopped to see him that day, I knew that this would be our last time together as he was terribly ill. Before I left at the end of our visit, I put my arms around him, looked into his tired eyes, that I could actually see, and told him how glad I've always been to be his daughter and how much I loved him. When I got to the door, I turned and waved goodbye.

He raised his hand to give me a small wave and smile.

I actually saw him do that, and he knew that I saw him.

That is a wonderful memory that I'm sure my Dad took with him when he left and I will carry with me for the rest of my life. I'm so thankful that we were able to share something so wonderful during our last time together.

The only regret that I have involving my treatment is that I wasn't able to receive it sooner. I try not to focus on this too much but I definitely would have benefited more from treatment if I had been able to receive it earlier in life when my retina was healthier. Nonetheless, I am so appreciative of the benefits that I did receive. I am truly blessed.

Tami's letter, which was also sent directly to you, is much longer, but that is all I have time to share today.

The story of her father is just one experience that this life-changing treatment made possible for her.

I hope that as you consider approving this therapy you will remember Tami's story and that it helps you to understand just how very, very important it is for our disease community to be able to retain or restore any amount of vision for any amount of time.

On behalf of Tami, and all of the LCA families that Sofia Sees Hope represents, I would like to thank you for your time and thoughtful consideration.