

April 23, 2014

Committee: FY15: Driving Innovation through Federal Investments

TESTIMONY FROM A PARENT

Madam Chairwoman, Vice-Chairman Shelby and Members of the committee;

Thank you for allowing us to present testimony for your hearing; Driving Innovation through Federal Investments. I am submitting this testimony to support the worthy work of the NIH (National Institutes of Health). Our family is a live testimony of the reputable work that is accomplished at the NIH.

Kayla, my 10 year old daughter, has a rare syndrome called NOMID (Neonatal Onset Multisystem Inflammatory Disease). Affected individuals often have headaches, seizures, and vomiting resulting from chronic meningitis, which is inflammation of the tissue that covers and protects the brain and spinal cord (meninges). Intellectual disability may occur in some people with this disorder. Hearing and vision problems may result from nerve damage and inflammation in various tissues of the eyes.

People with NOMID also experience joint inflammation, swelling, and cartilage overgrowth, causing characteristic prominent knees and other skeletal abnormalities that worsen over time. Abnormal deposits of a protein called amyloid (amyloidosis) may cause progressive kidney damage.

In her first year of life we went to see 22 different specialists, many testing appointments, etc. As a mother, there is no greater hurt than seeing your child in physical pain this entire time and not being able to help them, even if you're trying your best.

At 15 months, she finally got the correct diagnosis of NOMID **at the NIH**; and we've been coming ever since. Before being diagnosed we were two weeks away from a feeding tube and/or chemotherapy before coming to see Dr. Goldbach-Mansky (who heads the research team at NIAMS) (National Institute for Arthritis and Musculoskeletal and Skin Diseases) for NOMID and other CAPS (Cryopyrin Associated Periodic Syndromes) patients.

Fast forward 10 years. Kayla is in 4th grade. She attends a small Catholic School outside of Chicago. Makes me very proud to say she is a straight A student. She loves to dance, play piano, swim, read, practice yoga and just be a 'typical' 10 year old. I am forever indebted to the NIH/NIAMS and their team for being her lifeline.

The medicine she takes daily, (KINERET) is taken by daily injection was originally purposed for other conditions. This shot is her saving grace. Although we had unfortunate hurdles in the system to get it FDA approved and dealing with our insurance companies to get the medicine has not always been easy, the struggle (as you can see) is worth it every day I get to see my daughter pain free.

Lastly, it is so key to keep that line of communication open with the pharmaceutical companies who, at the end of the day are our partners and we want to be able to streamline and work more collaboratively with them.

Again, thank you to the committee for having this hearing and for allowing us to submit testimony.

Respectfully submitted,

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