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Legislative Agenda 115th Congress, 2nd Session

NephCure Kidney International is the only organization committed exclusively to support research seeking the cause of the potentially debilitating kidney disease Focal Segmental Glomerulosclerosis (FSGS) and the diseases that cause Nephrotic Syndrome (NS), improve treatment, and find a cure. NephCure is driven by a panel of respected medical experts and a dedicated band of patients and families.

FSGS occurs when the kidney's glomeruli become scarred. The damaged filters allow protein to leak into the urine, and prolonged leakage can lead to kidney damage and failure. Patients with kidney failure must undergo dialysis or receive a kidney transplant. Unfortunately, FSGS returns in 30-40% of patients who receive a transplant. FSGS is the most common cause of steroid resistant NS in children, and it is a leading cause of kidney failure in children. In addition, African Americans are impacted at a five times greater rate than the general population. The causes of FSGS are unknown and there is currently no cure.

Patient Stories

Noah



I was diagnosed when I was 7 years old with Nephrotic Syndrome. I am almost 9 years old now. When I get very sick it is called a relapse and then I have to start taking higher doses of steroids (prednisolone). Taking steroids doesn't seem like a big deal but it makes me really hungry all the time. I started a new medicine (tacrolimus) on August 10th that is working, so January 6th, 2018 was my last day of taking steroids, hopefully forever. I didn't like being on steroids. Besides being hungry all the time, it made my face really big and I gained a lot of weight, and I stopped growing taller, so my twin brother is now way taller than me. They say I could still catch up, but I have to wait and see. I'm 2 minutes older and was always bigger but I'm being patient. I still have to take a blood pressure medicine. I take the same

pills as my 90 year old great grandfather! This is from both the steroids and the disease. I check my urine and blood pressure every day and have to take the tacro at 8am and 8pm. We have alarms to remind us all. My Mom was worried when school started because steroids can make you act crazy she says. I love school so I never let it get me in trouble. I was even invited into the ALPS program which is Advanced Learning Program for Students because I did so well! I play baseball and basketball because my parents won't let my disease define me, so as long as the doctors say it is okay and I want to do it, they let me do it. I wish a cure could be found for Nephrotic Syndrome. I don't like having to explain it to my friends, and I don't like how worried my parents always are.

Paige

Paige was diagnosed at the age of three, over eight years ago. Her body responds to steroids so we rely solely on that drug to maintain her health. Paige's current treatment plan is identical to someone who was diagnosed with this condition in the 1970's. Can you imagine being diagnosed with a chronic condition and the Dr. using case studies from over forty years ago to develop your initial treatment plan? It is a devastating feeling to know there remains no known cause or cure to the condition that affects your child daily.



The side effects of steroid use are numerous, the list is very long. Research is needed to find alternative and better treatment methods. Paige relapses when her immune system is tested and yet the treatment method we have to rely on causes her immune system to weaken. Nephrotic Syndrome and steroids have changed the way we live our lives, we have worry and stress over her health instead of joy.

Nephrotic Syndrome changed Paige's life, but she does not allow Nephrotic Syndrome to ruin it. She is a smart, determined, kind young person who is a scholar and a competitive swimmer and has the best giggle around. She makes a positive difference in this world. Our family supports the need for additional research organizing annual running teams to raise vital funds to support research. Paige may not remember how life was like without Nephrotic Syndrome but we certainly do. We ask for your support in funding additional, vital research to help find a cure for these devastating kidney conditions. Thank you.

Kimberly



My name is Kimberly Queen and I was diagnosed with Focal Segmental Glomerulosclerosis (FSGS) in 2012 at the age of 25. At that time, I was fulfilling my passion teaching Georgia State Pre-k when I received the news; it was only my third-year teaching. After only two months of being diagnosed and being prescribed 60mg of Prednisone, I went into septic shock. Thankfully I was surrounded by amazing doctors who saved my life. It was then that I realized it was time to fight this disease. However, just as I was starting my fight, my kidneys failed in the first nine months.

I am forever grateful to my brother who donated his kidney to me on November 7, 2014, but with FSGS there is always a chance of reoccurrence, which I saw firsthand shortly after when I began spilling protein. During the two weeks I spent in the hospital, we started putting together a game plan for how to put this awful disease into remission. I began daily plasmapheresis along with taking a blood pressure medication.

We saw a little change but not enough. It's now been three years since my reoccurrence. In that time, I have done over three hundred plasmapheresis treatments, experimented with different dosages of Prednisone, tried different blood pressure medications, started using Acthar Gel and started Rituximab. I have attained partial remission using the Acthar Gel, and we are hoping to reach full remission with the Rituximab. More research is needed with this disease so that myself, and others do not feel like "test subjects" trying different medications and so there can be a higher success rate. I would love to be able to live my life not focused around doctor appointments, treatments and long infusions. Luckily, I am surrounded by a family who understands how FSGS has impacted my life, as well as friends who support me and encourage me to stay strong daily fighting a disease with no cure.

FY 2019 Funding Priorities

- **Please provide the National Institutes of Health (NIH) with at least \$38.1 billion in FY 2019, a \$2 billion funding increase.** There is no known cause or cure for FSGS and scientists tell us that much more research needs to be done on the basic science behind FSGS/NS. More research could lead to fewer patients undergoing ESRD and tremendous savings in health care costs in the United States. NephCure works closely with NIH and has partnered with NIH on two large studies that will advance the pace of clinical research and support precision medicine. These studies are the Nephrotic Syndrome Study Network and the Cure Glomerulonephropathy Network.
 - **Provide a proportional increase for the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)** and support FSGS/NS research opportunities. Cure Glomerulonephropathy (CureGN) is an important study with the goal of supporting precision medicine development for NS.
 - **Provide a proportional increase for the National Institute on Minority Health and Health Disparities (NIMHD)** and support FSGS/NS research opportunities through grant and training programs.
 - **Support rare disease research at the National Center for Advancing Translational Sciences (NCATS).** The NEPTUNE Study, part of the Rare Diseases Clinical Research Network, is a collaboration of NIH, NephCure and academic partners to pool resources and find a cure.
- **Please continue to include “Focal Segmental Glomerulosclerosis (FSGS)” as a condition eligible for study through the Department of Defense Peer-Reviewed Medical Research Program (PRMRP) for FY 2019.**

On average, veterans have a greater burden of chronic kidney disease (CKD) than the general population. Nearly 30,000 veterans suffer from ESRD and an additional 3,000 veterans are expected to reach ESRD each year. Almost 40% of people with ESRD are African American. This health disparity is particularly striking in young African American men of the age typical for active duty service, as rates of kidney disease in men 20-40 years old are as much as 20-fold higher in African Americans than the general population.

Patient Access Issues

- Please co-sponsor *Chronic Kidney Disease Improvement in Research and Treatment Act of 2017* (H.R. 2644). This bill is aimed at improving the lives of those suffering with kidney disease by identifying barriers for transplantation and improving donation rates. It also allows individuals with kidney failure the ability to retain access to private insurance and promotes access to home dialysis treatments. Additionally, H.R. 2644 seeks to understand the progression of kidney disease and the treatment of kidney failure in minority populations and improve access to kidney disease treatment for those in underserved rural and urban areas.