News from CureGN

March 2016

Sponsored by the National Institutes of Health (NIH)
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)

Thank you for your time and contributions to CureGN. As you may be already aware, the Cure GN study is a huge effort aimed to further the understanding of rare forms of kidney diseases, including minimal change disease (MCD), focal segmental glomeruloslerosis (FSGS), membranous nephropathy (MN) and IgA Nephropathy. Please find below some updates about this important, one-of-a-kind study.

The CureGN study is being performed jointly by 4 coordinating centers: The Midwest Pediatric Nephrology Consortium (MWPNC), Columbia University, University of North Carolina, and the University of Pennsylvania. Each center may have several enrolling sites. We would like to introduce you to each of our centers over the next several newsletters.

Midwest Pediatric Nephrology Consortium (MWPNC) Profile

The MWPNC is a group of more than 50 pediatric kidney centers throughout the USA and Canada with more than 200 members, including physicians, nurses, scientists, and research coordinators, working together to further research in science and medicine. 32 separate sites are enrolling children in CureGN through the MWPNC center. Since our founding in 2004, the MWPNC has published over 27 papers, its members have been awarded 19 federal or regional grants, and it currently has over 30 clinical trials and other research projects in progress. Our mission is to improve and promote high quality care for children with kidney disease by helping researchers work together to find the best treatments for our patients. Collaboration is the key to the success of our mission and is required to perform research on very rare childhood kidney diseases.

Enrollment

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Clinical research studies like CureGN depend on you!

As of 2/29/2016:

Total Enrolled: 1062

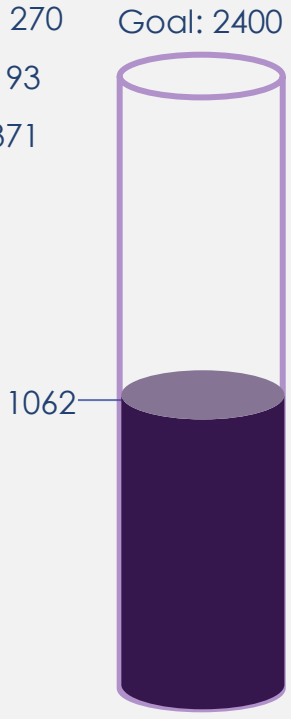
Totals by disease:

MCD: 228

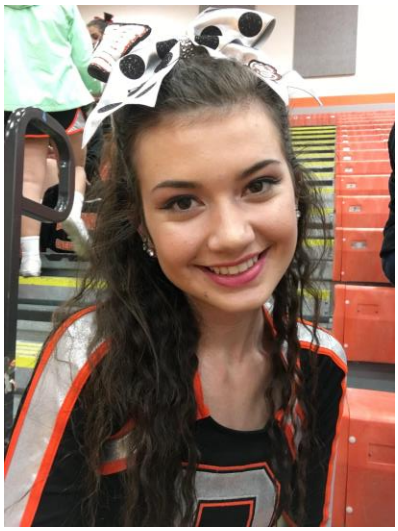
FSGS: 270

MN: 193

IgA: 371



Patient Profile - MWPNC



My name is Katherine and I am a sophomore in high school. I am a JV cheerleader for my high school football and basketball teams. When I was 11 years old, I was diagnosed with FSGS. Having FSGS has made things very difficult at times. It's hard to always stay positive when I don't know if I may lose a kidney in the future. I am fortunate to be in remission right now, but I don't know how long that will last. I try not to think or worry about it, but sometimes I do. I joined this study in hopes that a cure will be found for FSGS, or at least some information that helps me know how or why I got this disease. My wish is that a cure will be found for FSGS because being 11 and finding out that you have a disease is scary, especially when you don't get all your questions answered. I would hate for another young kid to go through this and I want them to get the answers they deserve.

CureGN Disease Spotlight:
Focal Segmental Glomerulosclerosis (FSGS)

What does FSGS stand for?

If you are unclear about what Focal Segmental Glomerulosclerosis (FSGS) is, you are not alone. It is a complicated disease for both patients and health care professionals to understand. First we have to break down the name:

- **Focal**: Some, but not all, of the kidney's 2 million filters are damaged
- **Segmental**: The damage only affects a part of each affected filter
- **Glomerulo**-: The injury is to the filters (glomeruli) of the kidney
- **Sclerosis**: scarring (-***sclerosis***)

FSGS leads to scarring of the kidney filters (glomeruli) and leaking of too much protein into the urine.

What causes FSGS?

FSGS is the name for a number of different conditions that cause the same pattern of injury to the kidney. For example, it may be caused by:

- genetic mutations
- infections
- certain medications

- conditions that cause the filters to overwork, like obesity or being born with relatively few filters

Many times the exact cause of FSGS is not known, but suspected to be caused by abnormalities in the immune system. This is called 'primary' or 'idiopathic' FSGS, and it is often treatable with drugs that affect the immune system.

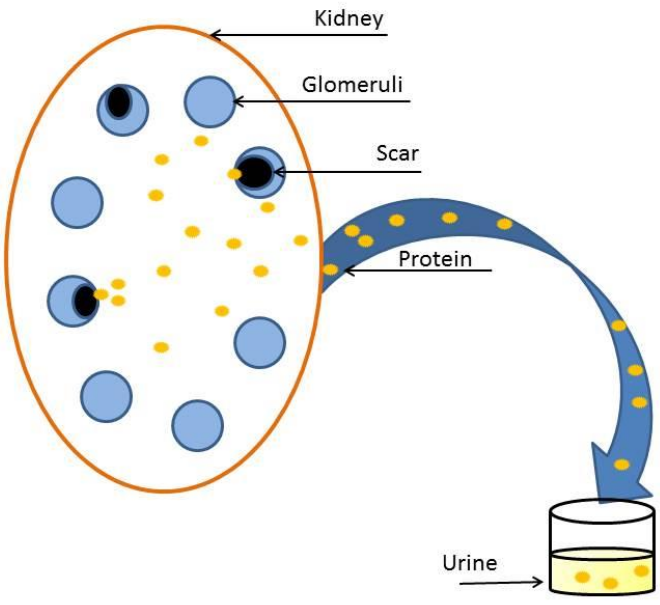
Do all patients with FSGS have the same future?

Research has shown us that lowering the urine protein level to normal or near normal levels in patients with idiopathic FSGS results in much healthier kidneys in the future compared with patients whose urine proteins remain too high. Doctors usually prescribe blood pressure medications and often medications that suppress the immune system to lower urine protein levels and protect future kidney health.

Why is FSGS research important?

Patients with FSGS who do not improve with treatment have a high risk of developing kidney failure (end stage kidney disease) requiring a kidney transplant or dialysis. With the CureGN study leading the way, we aim to achieve a better understanding of the different types of FSGS in order to find new and more effective treatments for the disease.

This cartoon shows FSGS in the kidney. The filters are called glomeruli (blue). Some of these filters are partially scarred (black) and too much protein (yellow) is lost in the urine.



Additional content can be found on our website CureGN.org or at Nephcure.org.