News from CureGN

September 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 CureGN study is a huge effort aimed to further the understanding of rare forms of kidney diseases, including minimal change disease (MCD), focal segmental glomerulosclerosis (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.

University of Pennsylvania Profile

The Penn PCC brings together the expertise of 25 adult and pediatric medical centers in the United States and Canada (https://curegn.org/WhoWeAre.aspx), the efforts of which are centralized through the University of Pennsylvania in Philadelphia. Our members include many clinical and scientific leaders in glomerular disease research and treatment. The diversity of Penn PCC members' locations allows for patients from all over the United States and Canada to participate in Cure GN. We also bring decades of expertise in studying and treating patients with glomerular disease. We are excited to play our part in helping improve the lives of patients with glomerular disease through the Cure GN consortium.

Enrollment

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Clinical research studies like CureGN depend on you!

As of 09/26/2016:

Total Enrolled: 1516

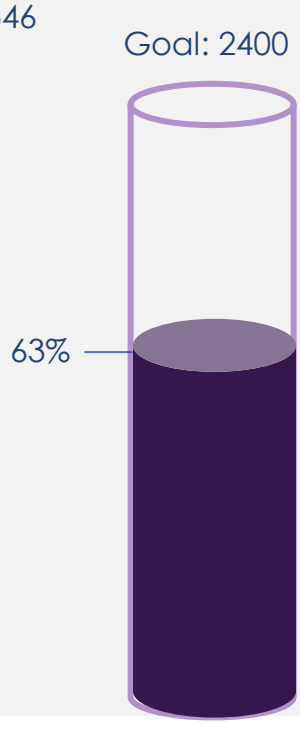
Totals by disease:

MCD: 321

FSGS: 368

MN: 281

IgA: 546



Patient Profile – PENN

Hi, my name is Paul. In late 2015, at the age of 70, I experienced the onset of symptoms including foamy urine and swelling in my feet and legs. I had gained 30 pounds of water weight, which made it very difficult for me to get around and do the things I usually like to do.

My blood work revealed abnormalities so my primary care physician referred me to a nephrologist. I believe that because of her more limited experience and knowledge about the latest research in the field, she recommended a traditional treatment plan. The plan was not incorrect, just very traditional and more aggressive.

Already part of the Hopkins family, as I see another surgeon in the complex, I secured a referral to Dr. Sperati at John Hopkins Hospital. Dr. Sperati was careful to rule out other potential causes for my membranous nephropathy before finalizing a treatment plan for me. He explained 4-5 other treatment protocols that were available, but based on his experience and knowledge of the latest research in the area, he also recommended what he thought was best for me. I believe that he had the expertise because of his research background to craft the most appropriate treatment plan for me.

I take my medication regularly and have blood work done every month. I am in close communication with Dr. Sperati who has been slowly reducing my medication to avoid any toxicity or side effects. I am feeling fine and I am able to play golf again!

It is a privilege to provide this feedback regarding the importance of this clinical study. Without the expertise and passion of physicians, such as Dr. Sperati, people would not have the opportunity for longer lives…free from diseases such as GN. The physician experts need, of course, the resources to conduct the critical research that guides their practice.

CureGN Disease Spotlight:

Membranous nephropathy

Membranous nephropathy (MN) is a disease that affects the glomeruli (filters) of the kidney and results in the loss of protein in the urine (proteinuria). It is called "membranous nephropathy" because kidney biopsies from patients with MN show that the walls (membranes) of their glomeruli are very thick. In adults, MN is one of the most common causes of the *nephrotic syndrome*, where proteinuria results in swelling, high blood pressure, high cholesterol, and, over time, can cause kidney damage. Approximately 1/3 of patients' proteinuria will improve without aggressive treatment, 1/3 will remain stable, and 1/3 will worsen over time.

A major discovery was made in 2009, when it was found that 70-80% of patients with MN have an autoimmune condition caused by an antibody called anti-PLA2R. Autoimmune conditions are those where the immune system attacks one's own organs, and antibodies are part of the immune system used to fight infections. In MN, the anti-PLA2R antibody is produced by the immune system and deposits in glomeruli, causing inflammation, kidney damage, and proteinuria. The anti-PLA2R antibody level can be detected by a routine blood test.

We do not currently know what triggers the production of the anti-PLA2R antibody, but we do know that decreasing the level of anti-PLA2R antibody in the blood has been linked with improving patients' proteinuria. In addition to using blood pressure medications that decrease proteinuria, the treatment of MN uses medicines that suppress the immune system and the production of the anti-PLA2R antibody. These include prednisone, cyclophosphamide, tacrolimus, cyclosporine, and rituximab.

By enrolling 600 patients with MN, the Cure GN consortium strives to improve the understanding and treatment of this disease.

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