



# NephCure Kidney Network Patient Registry

**March 2015 - March 2016**



ARBOR RESEARCH  
COLLABORATIVE  
FOR HEALTH



**NEPHCURE**  
Kidney International  
Saving Kidneys • Saving Lives



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# Welcome

**I**n the quest to find better treatment options for Nephrotic Syndrome, patients are essential partners.

Prior to having a patient-reported registry, we learned about the patient journey through anecdotal accounts shared on our website or in person. Today, the NephCure Kidney Network (NKN) Patient Registry gives families a platform to share their stories in a structured, more meaningful, data-driven way. Beyond that, participation in the registry process itself sparks a sense of empowerment within patients related to their health and well-being. This benefits NephCure in the long-term by creating a 'research-ready' community to help advance our mission.

In 2015, we were highly successful at growing the registry both in numbers and value. Beyond helping to attract the attention of researchers and the pharmaceutical industry, the NKN provides a forum to educate patients about the myriad of ways they can be part of the research process. Thankfully, these messages have resonated with our community, and excitement is building around the potential of research and clinical trials to find better treatments for Nephrotic Syndrome patients.

None of these achievements would be possible without our prized partnership with Arbor Research Collaborative for Health. Where NephCure excels at engaging patients in the registry, Arbor Research brings an expertise in participatory methods, biostatistical analysis, and database management. Our esteemed collaborators from The University of Michigan, The Children's Hospital of Philadelphia, and The University of Toronto all bring a scientific and clinical expertise to the project, ensuring that meaningful research is done. NephCure and Arbor Research representatives, along with a volunteer-led Steering Committee comprised of patients, clinicians, researchers and industry representatives, are working collaboratively to ensure the success of the NephCure Kidney Network. And, last but certainly not least, thank you to the patients and families who make up the NKN community and whose contribution of data and time is unparalleled in value. We look forward to continued growth and, someday, meeting our mission of improving the quality of life for everyone impacted by Nephrotic Syndrome.



Lauren Lee - NKN Co-PI  
Director of Research and Engagement  
NephCure Kidney International





# The NKN

**T**he NephCure Kidney Network is a patient registry with information provided by individuals with Nephrotic Syndrome or a family member of a patient. It is a systematic, scientific, standardized database with supporting programs to connect patients with cutting edge research and clinical trials. It is a source of information to generate new ideas that accelerate research to find better therapies.

## **The NephCure Kidney Network strives to:**

- Allow people suffering with Nephrotic Syndrome diseases and their caregivers to help accelerate development of new therapies that will improve lives
- Reduce barriers to effective recruitment and retention of participants in clinical research
- Support the development of new, safe, and reliable therapies for primary Nephrotic Syndrome

# Year in Review

## Bringing Patients & Stakeholders Together

Over the past year we have been working hard to build opportunities for patients, clinicians, and researchers to work side-by-side in the development of the NKN as a community sustained by shared value. This culminated in a face-to-face meeting of the NKN Steering Committee hosted by NephCure Kidney International in Philadelphia on January 29, 2016. This interactive meeting launched a critical dialogue across stakeholders and led to plans for the creation of task forces to ensure the conversation continues. Look for opportunities to collaborate with us in the coming year!



## Survey Updates

This year we made some exciting changes to the NephCure Kidney Network survey based on patient and stakeholder feedback. We added new questions, incorporated additional answer choices for old questions, and improved how the questions were organized. These changes ensure we are collecting the information most important to the Nephrotic Syndrome patient community in the way that is most useful for researchers.

## Support for Expansion

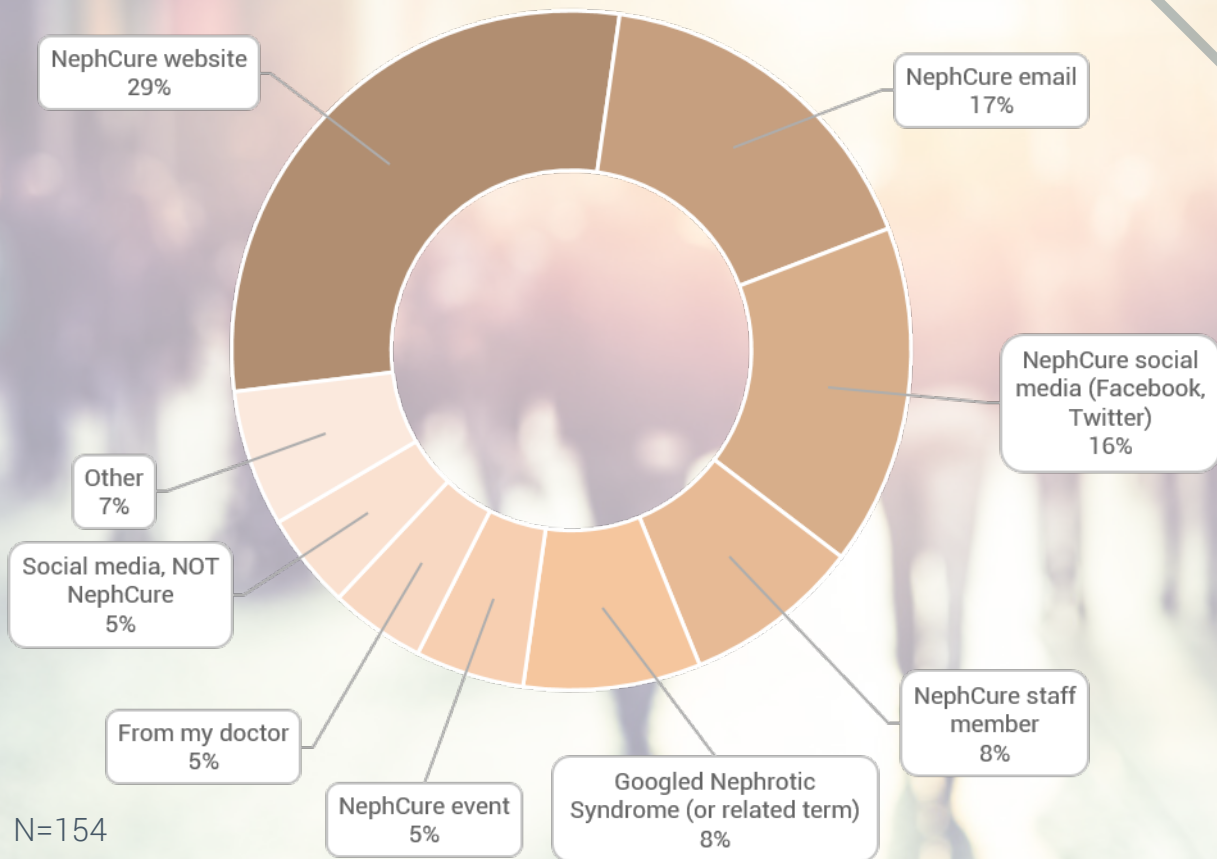
In July 2015, the NephCure Kidney Network was approved for a new three-year \$1.5 million funding award by the Patient-Centered Outcomes Research Institute (PCORI). This funding award continues PCORI's support for the NephCure Kidney Network's participation in the second phase of PCORnet, the National Patient-Centered Clinical Research Network.



## Website Enhancement

In the coming year, we will be reaching out to the patient and research communities for feedback on the NKN portal to learn what we can do better and what new features should be added. To prepare for this exciting work, this year we transitioned the registry to a new data collection and website platform hosted by our partner, Arbor Research Collaborative for Health.

## How did you learn about the NephCure Kidney Network Patient Registry?



# Recruitment

In order to better understand which outreach efforts are most effective, we added a new question to the survey this year which asks participants how they learned about the registry.

### Recruitment efforts for the NKN included:

Emails and mailings to all patients in NKI's contact database, announcements on [www.NephCure.org](http://www.NephCure.org), frequent social media posts, discussions about the NKN at NephCure's educational events, referrals from physicians and other supporters, word of mouth from participants, and flyers and brochures.

As of March 2016, **550** participants have enrolled in the NKN patient registry

# Geography

Participants from **32** countries have enrolled in the NKN



## Participants From Top 5 Countries

**445** USA

**25** Canada

**18** UK

**10** India

**9** Australia

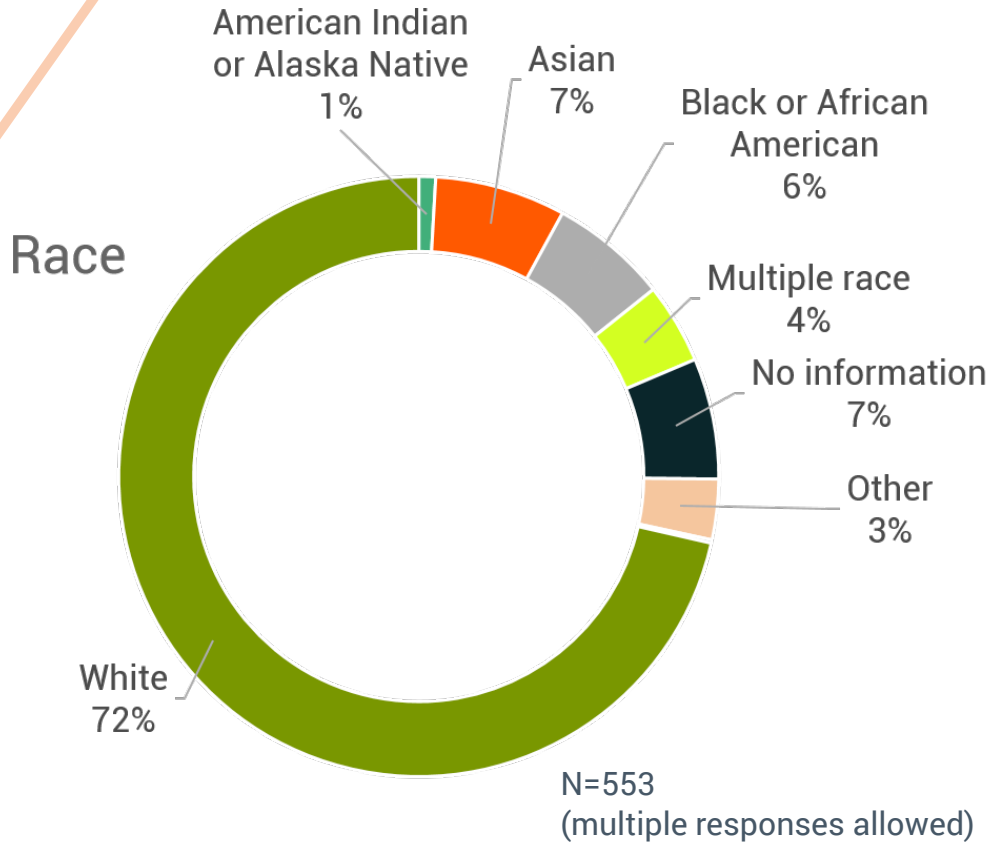
## Other Countries Represented:

Mexico, Netherlands, Ireland, Pakistan, Philippines, South Africa, Spain, Bulgaria, Croatia, Portugal, Trinidad and Tobago, Bahrain, Bangladesh, Brazil, Chile, Colombia, Ecuador, Finland, France, Israel, Republic of Korea, Lithuania, New Zealand, Singapore, Sweden, Turkey, Vietnam



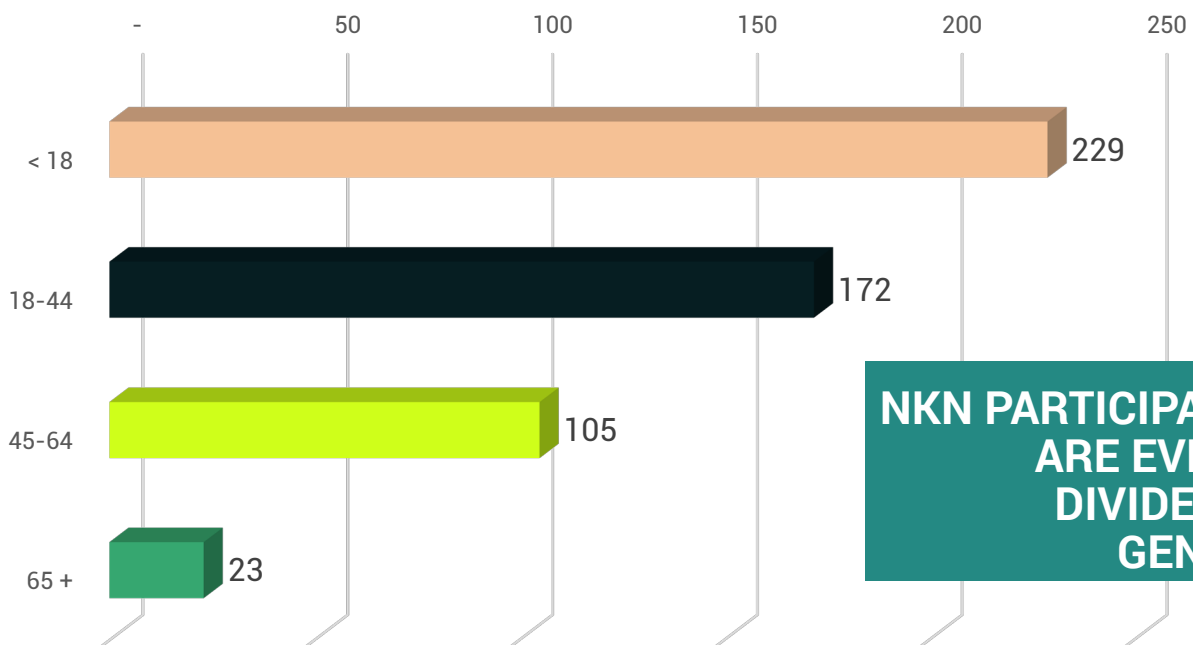
# Demographics

The NephCure Kidney Network seeks to represent the diversity of people living with Nephrotic Syndrome Diseases.



**10%** of participants indicated that they identify as ethnically Hispanic

Number of Participants by Age Group



**NKN PARTICIPANTS ARE EVENLY DIVIDED BY GENDER**



# Diagnosis

**FSGS** FOCAL SEGMENTAL GLOMERULOSCLEROSIS

**HSP** HENOCH-SCHONLEIN PURPURA

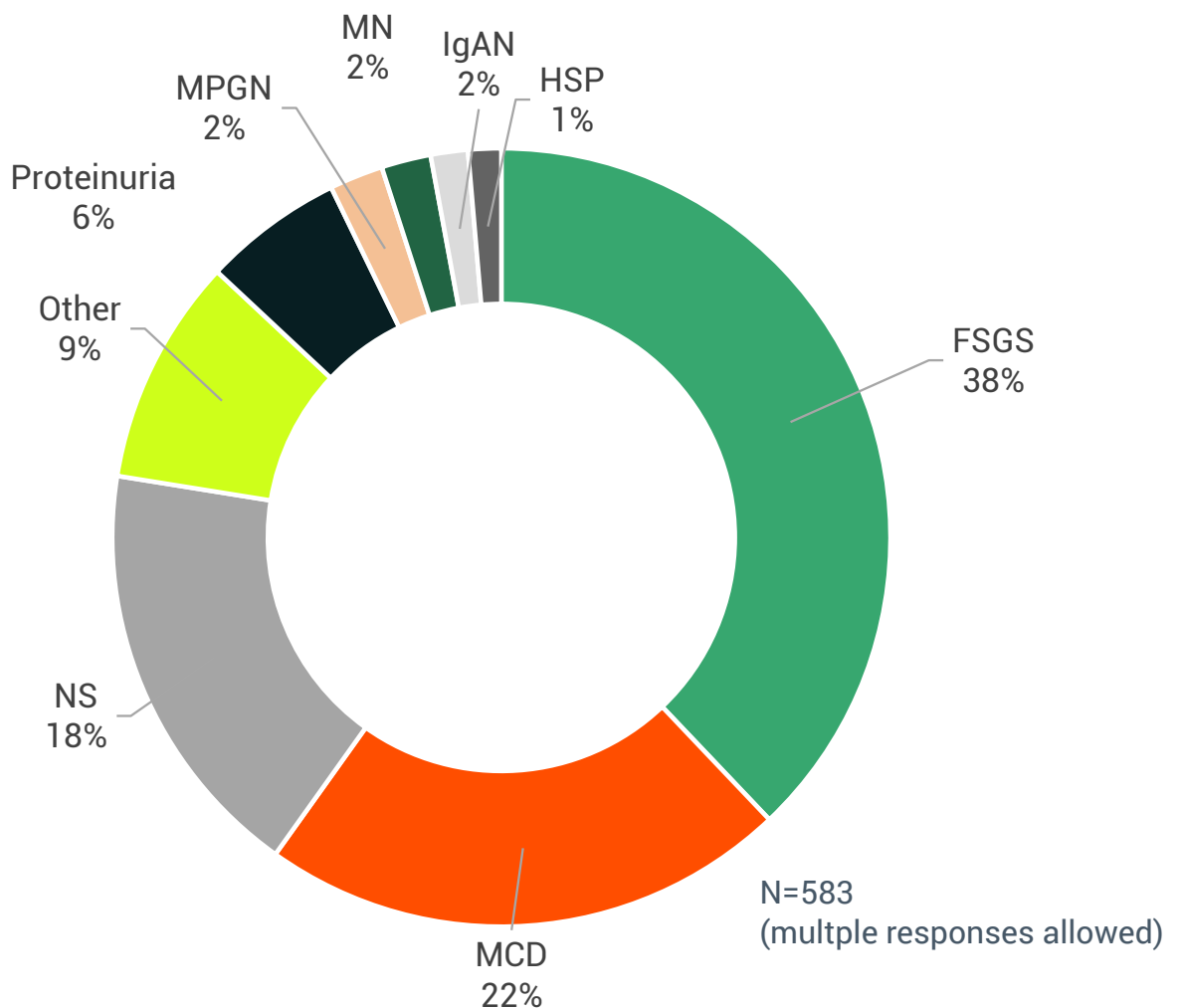
**IGAN** IGA NEPHROPATHY

**MN** MEMBRANOUS NEPHROPATHY

**MPGN** MEMBRANOPROLIFERATIVE GLOMERULONEPHRITIS

**MCD** MINIMAL CHANGE DISEASE

**NS** NEPHROTIC SYNDROME

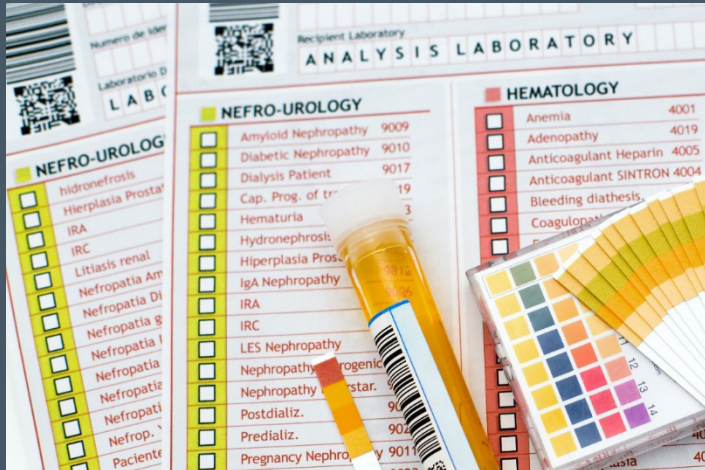


## Diagnosis

The NKN includes patients with a variety of diagnoses that have similar symptoms. Our broad snapshot may provide researchers with information that cannot be found in any other database.

# Lab Values

Lab results are an important element of the data captured in the NKN. These values are important for understanding disease progression. For those who are interested in clinical research, they are often important in determining eligibility for studies.



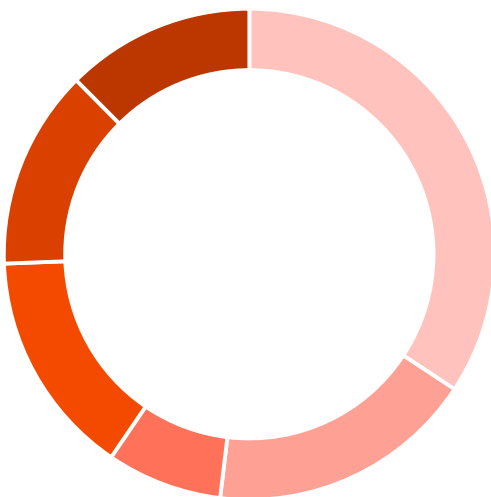
### Most Recent Urine Protein to Urine Creatinine Ratio



- Negative
- 1.0 mg or less
- 1.1 mg/mg to 3.0 mg/mg
- 3.1 mg/mg to 6.0 mg/mg
- 6.1 mg/mg and greater

N=62 participants who provided a value

### Most Recent Urine Dipstick for Urinary Protein



- Negative
- Trace (15mg/dl)
- 1+ (30mg/dl)
- 2+ (100mg/dl)
- 3+ (300mg/dl)
- 4+ (>2000mg/dl)

N=289 participants who provided a value

### Most Recent Serum Creatinine



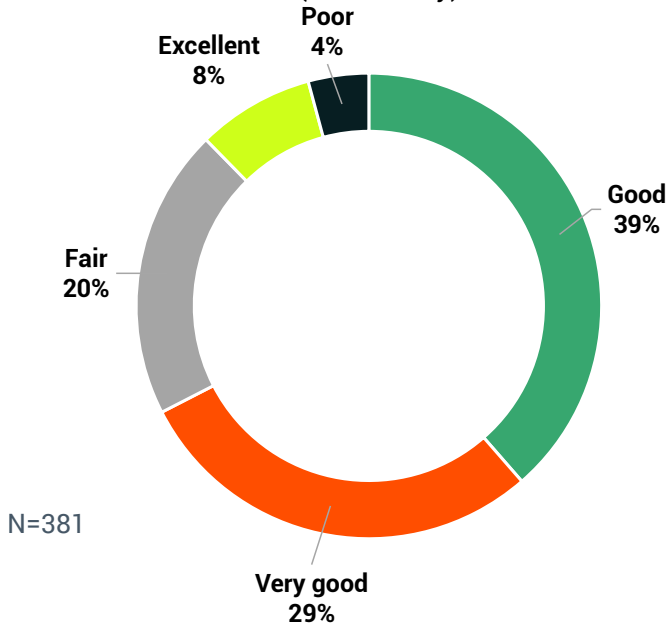
- 1.0 mg/dL or less (88.4 µmol/L or less)
- 1.1 mg/dL to 2.0 mg/dL (97.2 - 174 µmol/L)
- 2.1 mg/dL to 3.0 mg/dL (176.8 - 265.2 µmol/L)
- 3.1 mg/dL to 4.0 mg/dL (274.0 - 353.6 µmol/L)
- 4.1 mg/dL to 5.0 mg/dL (362.4 - 442.0 µmol/L)
- 5.1 mg/dL and Greater (>450.8 µmol/L)

N=89 participants who provided a value

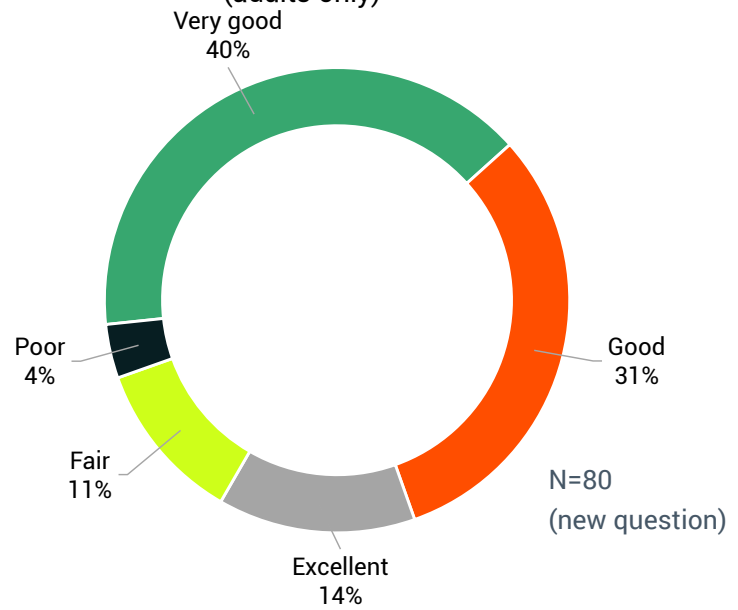
**P**atient-reported Quality of Life measurements are becoming increasingly recognized as a vital component of disease research and treatment. Since only patients can provide this type of information, their involvement in research is increasingly important.

# Quality of Life

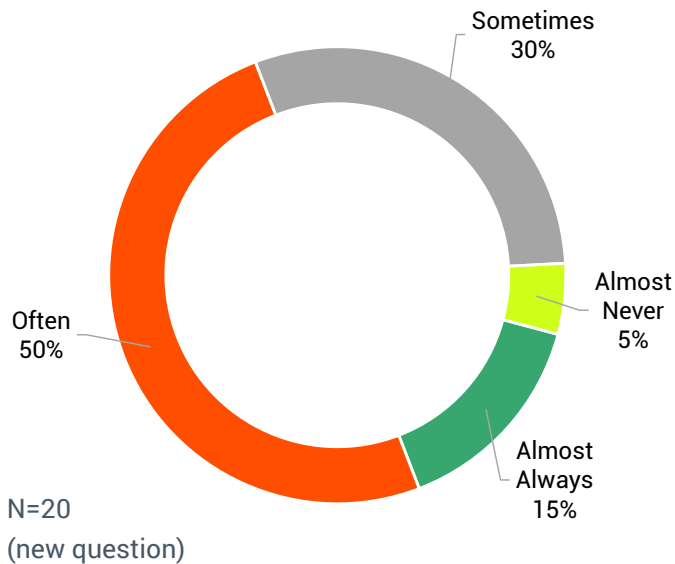
In general would you say your health is...  
(adults only)



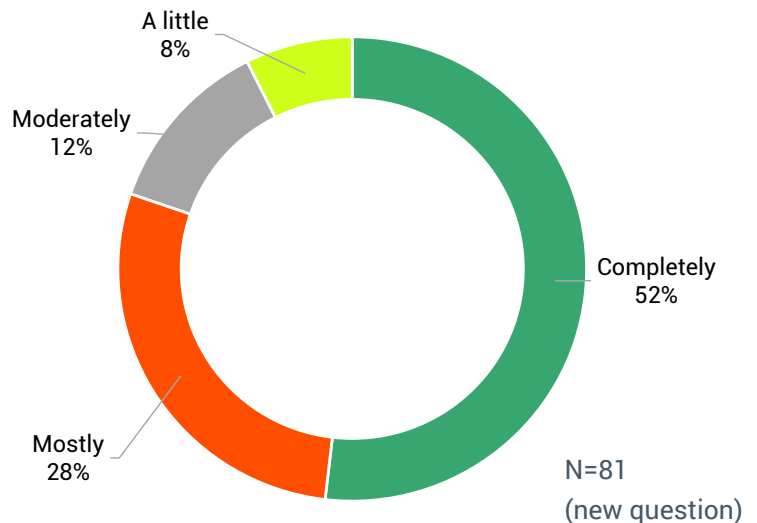
In general, how would you rate your mental health including your mood and your ability to think?  
(adults only)



How often do you have fun with friends?  
(children only)



To what extent are you able to carry out your everyday physical activities such as walking, climbing stairs, carrying groceries, or moving a chair?  
(adults only)



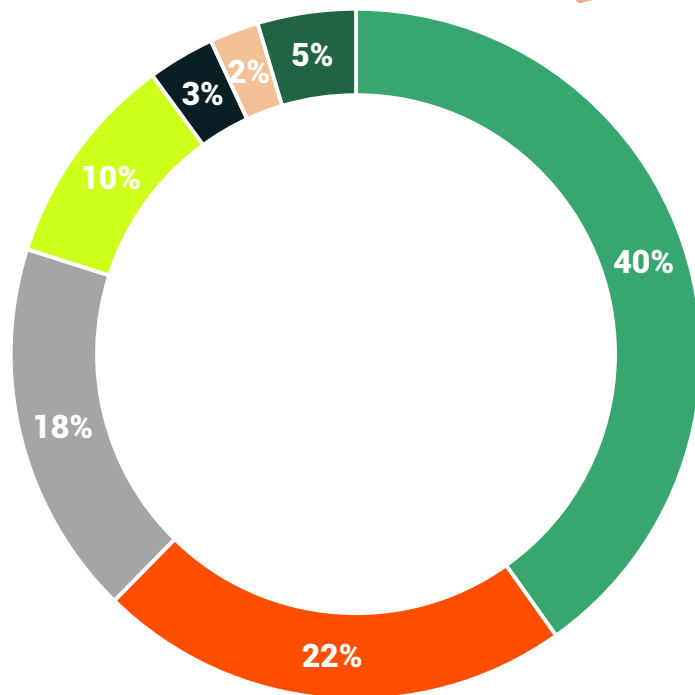


# Treatment

Drugs which suppress the immune system are the leading first-line treatment for Nephrotic Syndrome diseases.

## Immunosuppressants Commonly Taken

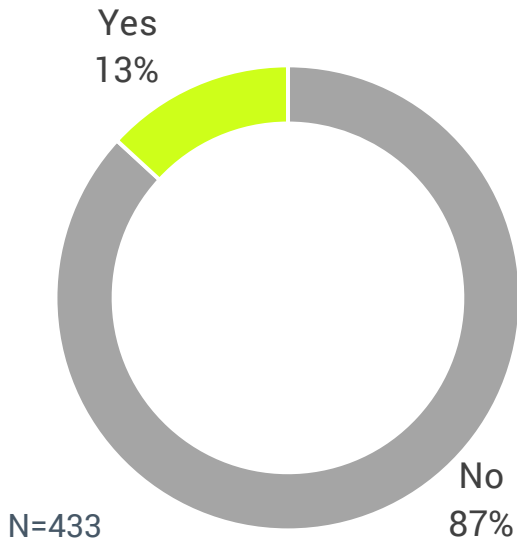
- Prednisone, Prednisolone, Solumedrol (Steroids)
- Prograf (tacrolimus)
- Cellcept or Myfortic (mycophenolate mofetil, mycophenolic acid)
- Neoral, Sandimmune, Gengraf (cyclosporine)
- Rituxan (rituximab)
- Acthar (adrenocorticotrophic hormone, ACTH)
- OTHER



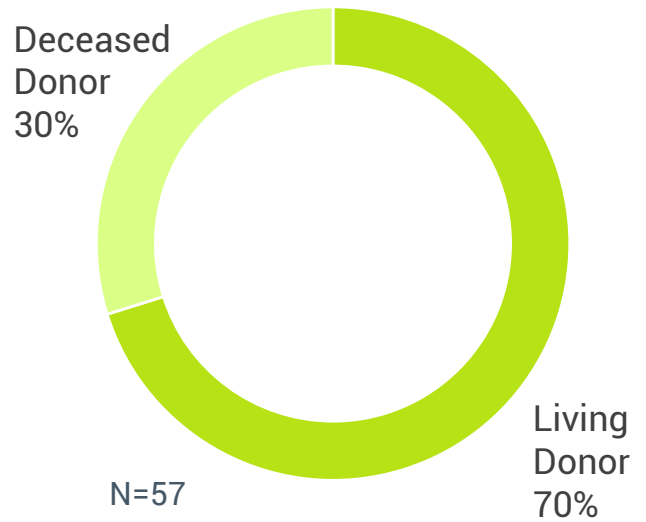
N=500 (multiple responses allowed)

**87%** of participants report having taken some type of immunosuppressant (N=157, new question)

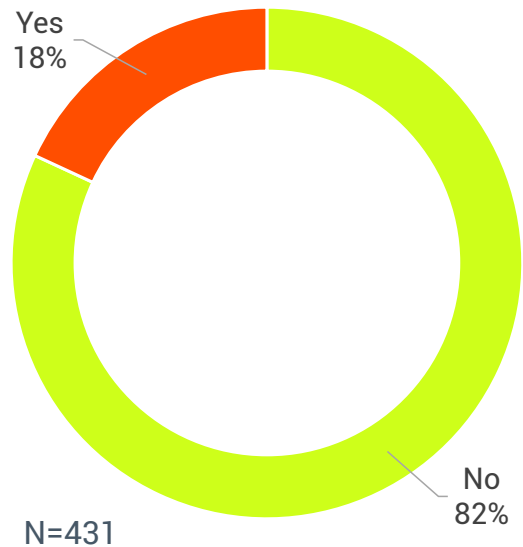
Has the patient had a kidney transplant?



What type of a kidney transplant did the patient most recently receive?

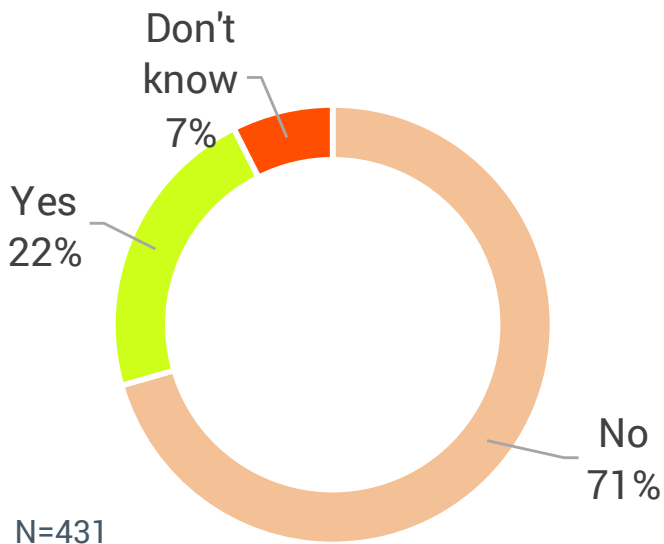


Has the patient ever had dialysis?



The average age at which patients started dialysis is **24**

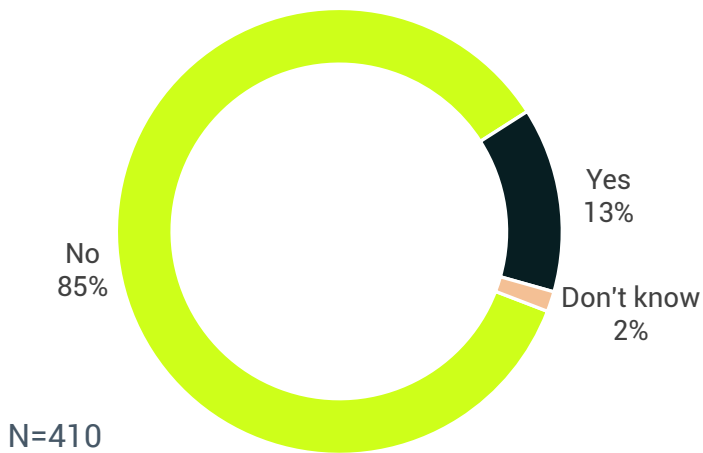
Has the patient had genetic testing?



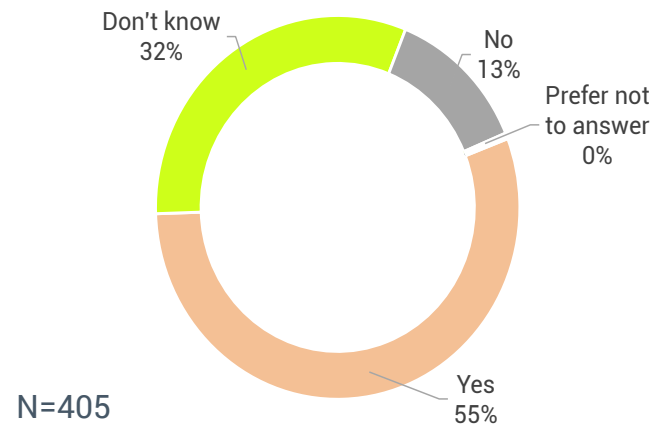
Currently over 30 genes have been identified as possible causes of Nephrotic Syndrome diseases. Continued research into genetic factors will enable a more personalized approach to treating Nephrotic Syndrome patients.

# Research

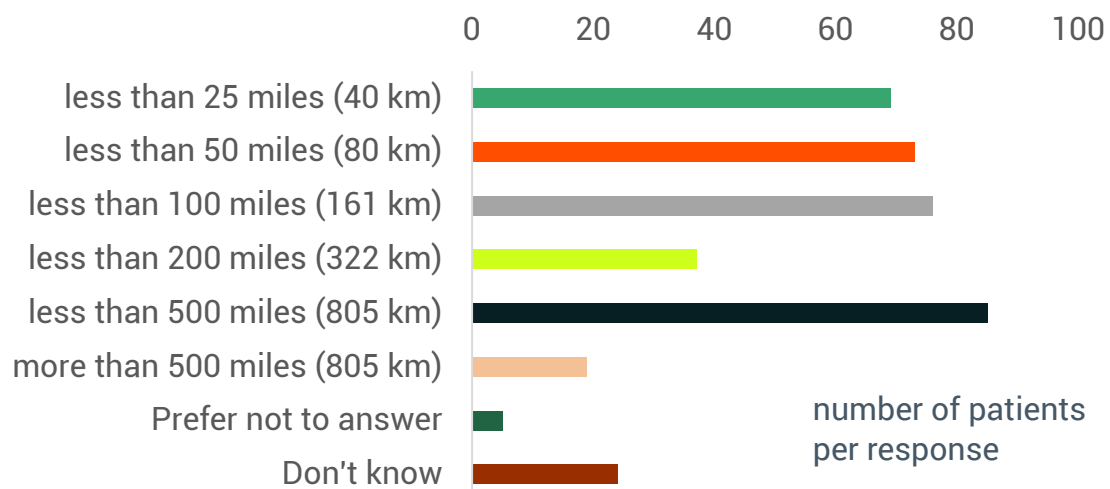
Has the patient previously participated in any clinical trials related to his/her kidney disease?



Would the patient be interested in participating in a research study about his/her kidney disease that would test an experimental therapy that may or may not help him or her?



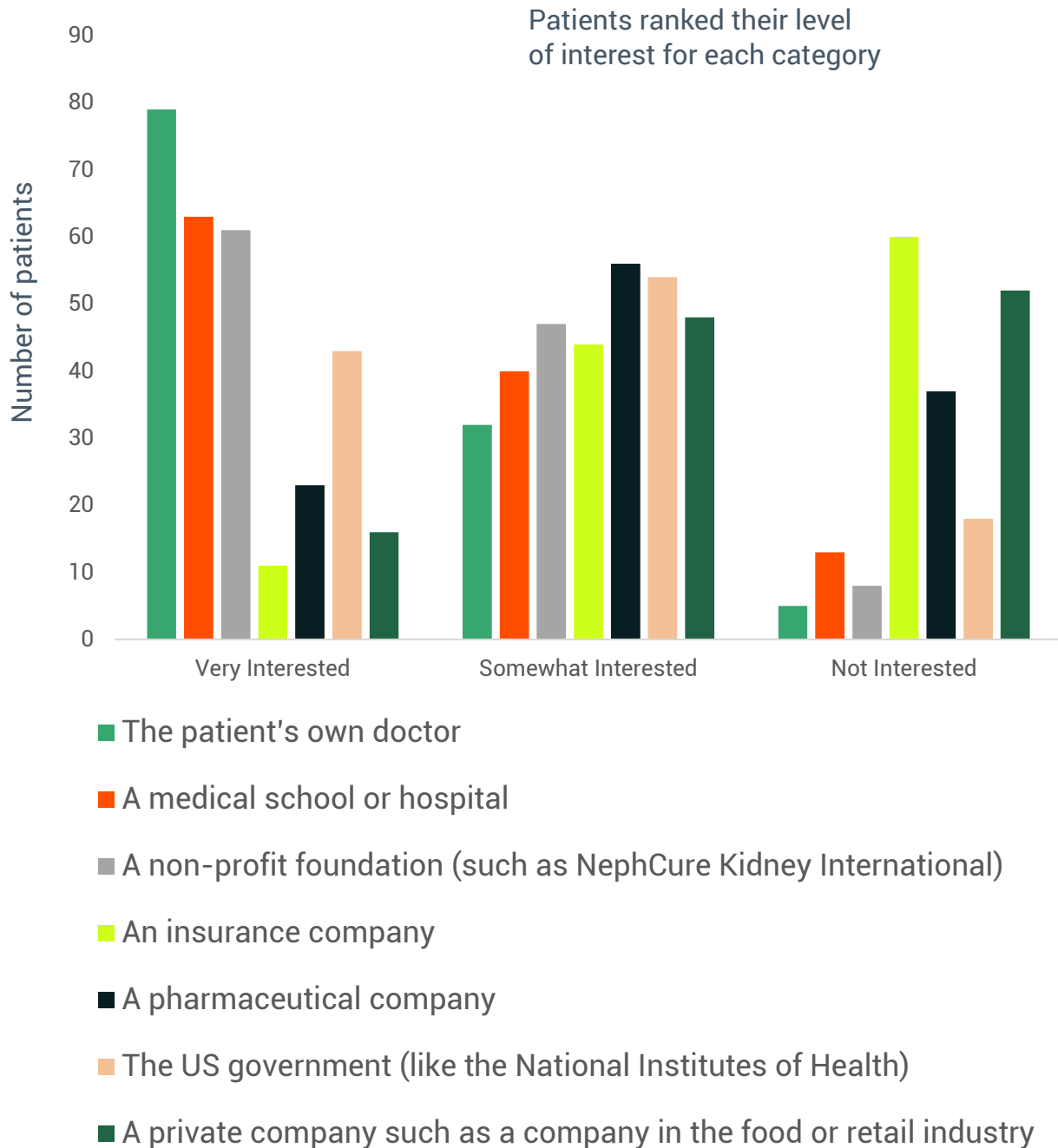
How far would the patient be willing to travel to take part in a research study?



**T**hese responses seem to indicate a disconnect between a patient's interest in participating in a clinical study and their actual rate of participation. The NKN hopes to help address this disconnect by ensuring that patient priorities influence what types of research studies are undertaken and how they are conducted.



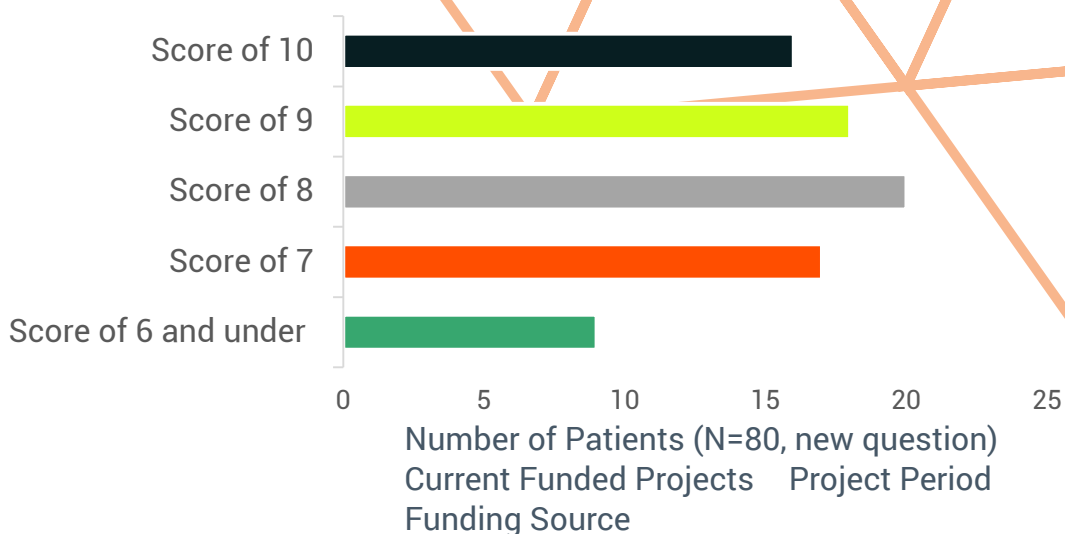
Are the patient's feelings about research affected by who is actually doing the project or study? (How does the patient's interest level change?)



N=115 (new question)

# Health Care

For the past 12 months... what number would you use to rate all your health care, where 0 is the worst health care possible and 10 is the best healthcare? (adults only)



Does the patient have health insurance or health care coverage?



# Your Data at Work

**N**o one knows the urgency for new therapies better than the patient community, and yet patients are rarely consulted when research is being planned. The NKN was designed to make sure the patient voice matters and patient data have power. In addition to connecting patients to research participation opportunities, the NKN promotes inclusion of patients in the prioritization and conduct of studies, so that researchers can improve their understanding of patient experience and focus on opportunities that make it a faster, safer journey.

The most exciting part of this research revolution is that the participants—the people affected by nephrotic syndrome—have the power to advance research right now. Even more exciting, we have already started! With increasing enrollment, opportunities to use NKN patient data in research studies are already emerging. Three studies are described in the table below, and many more are in development. We look forward to the work ahead of us!

Current Funded Projects	Project Period	Funding Source
<b>NKN Patient-Powered Research Network (Phase II)</b> <b>Major Activities &amp; Benefit to the NKN:</b> <ul style="list-style-type: none"> <li>– Recruit new members</li> <li>– Website improvements: NKN patient portal &amp; NKN researcher portal</li> <li>– Identify strategies to engage patients in the research process</li> <li>– Accelerate research!</li> </ul>	2015-2018	PCORI
<b>Establishing a National Pediatric Glomerular Disease Learning Health System</b> <b>Major Activities &amp; Benefit to the NKN:</b> <ul style="list-style-type: none"> <li>– Develop a set of codes to identify patients who may be eligible to participate in the NKN using electronic health record (EHR) data. These code sets will be shared openly so that all NS researchers can benefit.</li> <li>– Reach out to potentially eligible patients and invite them to join the NKN.</li> <li>– Expand the NKN database to include EHR data in addition to survey data.</li> </ul>	2015-	Mallinckrodt Pharmaceuticals
<b>A Model for Improving Patient Engagement and Data Integration with PCORnet Patient-Powered Research Networks and Payer Stakeholders</b> <b>Major Activities &amp; Benefit to the NKN:</b> <ul style="list-style-type: none"> <li>– Develop a set of codes to identify patients who may be eligible to participate in the NKN using insurance claims data. These code sets will be shared openly so that all NS researchers can benefit.</li> <li>– Compare NKN recruitment strategies to find out what works better (patient outreach by clinicians vs by insurance providers).</li> <li>– Expand the NKN database to include insurance claims data in addition to survey and EHR data.</li> </ul>	2016-2018	PCORI



# Meet our Team

## *The Steering Committee*

Julie Abramson (co-chair)  
Kathleen Broderick (co-chair)  
Peter Anderson  
Patrice Becker, MD  
Elizabeth Cope, PhD, MPH  
Chelsey Fix  
Debbie Gipson, MD, MS  
Jonathan Hogan, MD  
Lisa Holloman

Sergio Infante, MD  
Elaine Kamil, MD  
Radko Komers, MD  
Lauren Lee  
Laura Mariani, MD, MS  
Michael Mittelman  
Meg Modes  
Elaine Sotherden



### **NKN CO-PRINCIPAL INVESTIGATORS:**

Elizabeth Cope, PhD, MPH  
Lauren Lee

### **NKN CLINICAL INVESTIGATORS:**

Laura Mariani, MD, MS  
Debbie Gipson, MD, MS  
Michelle Denburg, MD, MSCE  
Michelle Hladunewich, MD, FRCP(C), M.Sc.

### **NEPHCURE KIDNEY INTERNATIONAL STAFF :**

Chelsey Fix,  
*Research Engagement Manager*

# Research Partners & Support

## PARTNERS

**NephCure Kidney International (NKI)**  
**Arbor Research Collaborative for Health**

### Research Partners:

**The University of Michigan**  
**The Children's Hospital of Philadelphia**  
**The University of Toronto**

## FUNDING

**NIH** The NKN pilot project (2013) was funded in part through a National Institutes of Health (NIH) award as part of the Global Rare Disease Patient Registry and Data Repository Program.

**PCORI** Expansion of the NKN (2014-2018) and its inclusion in PCORnet is funded in part through a Patient-Centered Outcomes Research Institute (PCORI) Award.

## ABOUT PCORI

**T**he Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers, and clinicians with the evidence-based information needed to make better-informed healthcare decisions. PCORI is committed to continually seeking input from a broad range of stakeholders to guide its work. More information is available at [www.pcori.org](http://www.pcori.org).

## ABOUT PCORNET

**P**CORnet, the National Patient-Centered Clinical Research Network, is an innovative initiative of the Patient-Centered Outcomes Research Institute (PCORI). The goal of PCORnet is to improve the nation's capacity to conduct comparative clinical effectiveness research efficiently by creating a large, highly representative network for conducting clinical outcomes research that directly involves patients in the development and execution of the research. More information is available at [www.pcornet.org](http://www.pcornet.org).



 [registry@nephcure.org](mailto:registry@nephcure.org)

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 [www.NephCureKidneyNetwork.org](http://www.NephCureKidneyNetwork.org)