

THE
NEPHCURE
KIDNEY
NETWORK
PATIENT
REGISTRY

THE
FIRST
YEAR



March 2014 - March 2015

An Annual Data Report
from NephCure Kidney International
& Arbor Research Collaborative for Health



NEPHCURE
Kidney International



ARBOR RESEARCH
COLLABORATIVE
FOR HEALTH

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WELCOME!

NephCure Kidney International (NKI) is a non-profit patient advocacy group dedicated to accelerating the development of new treatments and finding cures for children and adults with primary Nephrotic Syndrome (NS) diseases. In the past 3-5 years, we have seen an increase in the number of medical research studies focused on this group of rare kidney diseases that includes Focal Segmental Glomerulosclerosis, Minimal Change Disease and Membranous Nephropathy.



That's the good news. The bad news is that it is challenging to find patients who might be eligible to participate in trials for a rare disease, and some of these studies have struggled to enroll enough participants - delaying the testing of potential therapies.

Recognizing that educated and motivated patients can speed up the development of better treatments, NKI launched the **NephCure Kidney Network Patient Registry (NKN)** in March 2014. A patient registry collects information about patients provided by patients (or their caregivers) to:

- Help connect patients interested in participating in medical research with clinical studies for which they may be eligible
- Provide information about patient priorities for research to improve health and life outcomes most important to patients
- Collect and report the information so that it is meaningful to researchers


We are pleased to present the **2014 NephCure Kidney Network Patient Registry Annual Data Report**. This report uses graphics to summarize the data provided by 350+ NKN participants. We hope this report encourages people with FSGS and other primary Nephrotic Syndrome diseases to take an active role in accelerating research by participating in the NKN Registry. We also encourage the scientific and medical community to examine this data for trends that may be of interest and contact us to learn more about NKN and the value of a Patient Powered Research Network.

Thank you to all of the NKN participants for donating their data to this program. We also thank our partners at Arbor Research Collaborative for Health in Ann Arbor, Michigan and recognize the Patient Centered Outcomes Research Institute for awarding a National Patient-Centered Clinical Research Network (PCORnet) grant to help grow the NKN.

Sincerely,

Marilyn Hailperin, M.S.
National Director, Research
NephCure Kidney International

WHAT IS THE NEPHCURE KIDNEY NETWORK?



The NKN is a registry for patients with Focal Segmental Glomerulosclerosis (FSGS), Minimal Change Disease (MCD), Membranous Nephropathy (MN) and other rare diseases that cause primary Nephrotic Syndrome. It allows **your voice to be heard** and **helps advance research** to find better therapies and cures. We **protect your privacy** by following IRB protocols and use de-identified data from the registry to inspire researchers to **study causes, treatments, and cures.**

Registering and participating in the NKN benefits...

Patients:

- Help **increase research** on NS patient care and new medicines
- Help **advance research** to find better therapies & cures
- Help researchers better understand **topics that are most important to you** that they should study
- Be notified of **clinical trials** for which you may be eligible
- Become a part of a **community** where you can **advocate** and **contribute** for the benefit of yourself and others

Researchers:

- Access to a diverse **community of engaged patients** ready to participate in clinical research
- Repository of **patient-reported outcomes** to inform the design of observational and interventional research studies
- Patient **preferences for research participation** in order to increase the likelihood of participant retention throughout the life of a study

RESEARCH PARTNERS AND SUPPORT

Partners

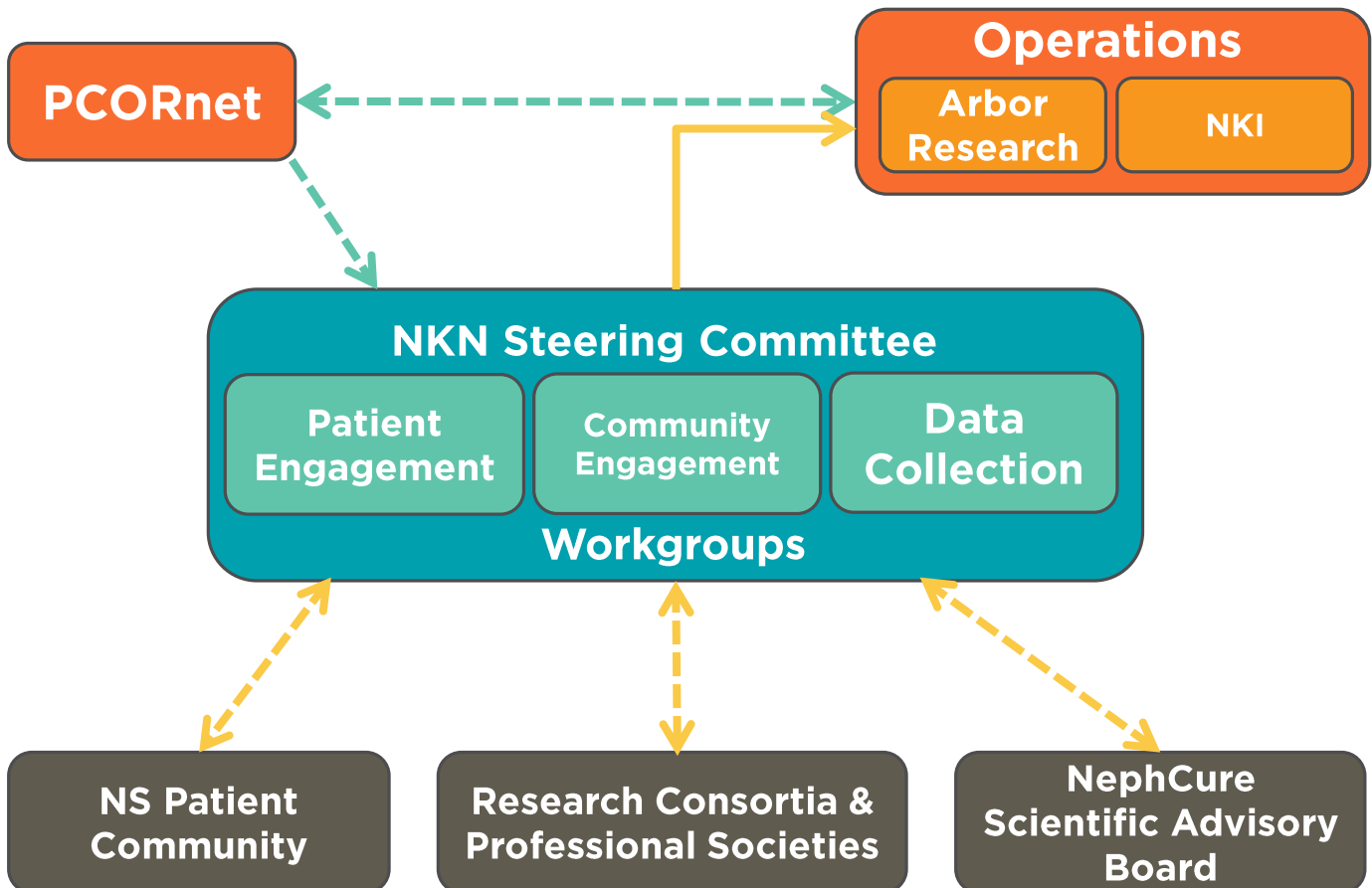
[NephCure Kidney International \(NKI\)](#) is a not-for-profit organization made up of patients, families, researchers, and clinicians seeking better treatments and a cure for Nephrotic Syndrome.

[Arbor Research Collaborative for Health](#) is a not-for-profit organization that conducts major studies in health outcomes research.

NKN Funding

[NIH/NCATS](#) grant award to develop NKN as a pilot project in the Global Rare Diseases Patient Registry and Data Repository Program

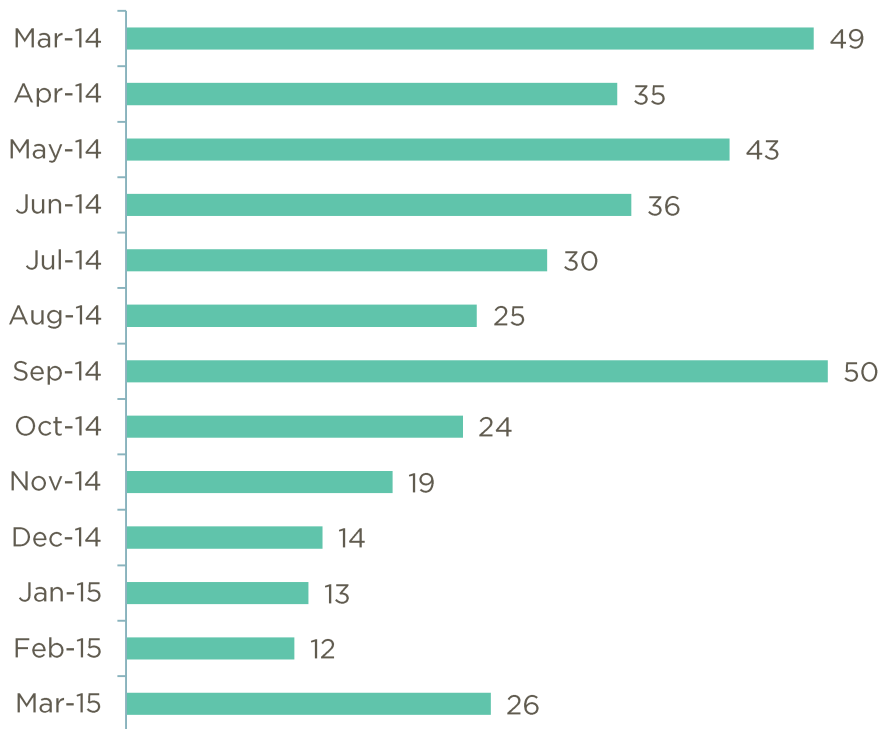
[PCORI](#) grant recipient to develop NKN Patient Registry into a Patient-Powered Research Network



DATA OVERVIEW

RECRUITMENT

New Participants by Month



As of March 2015, 376 participants have enrolled in the registry. That's about 29 participants per month.

Recruitment efforts for the NKN included:

- Emails and mailings to all patients in NKI's contact database
- Announcements on 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
- Flyers and brochures
- A Fitbit incentive program (one fitness tracker raffled off for every 100 people)

All recruitment materials are approved by an Independent Review Board (IRB).

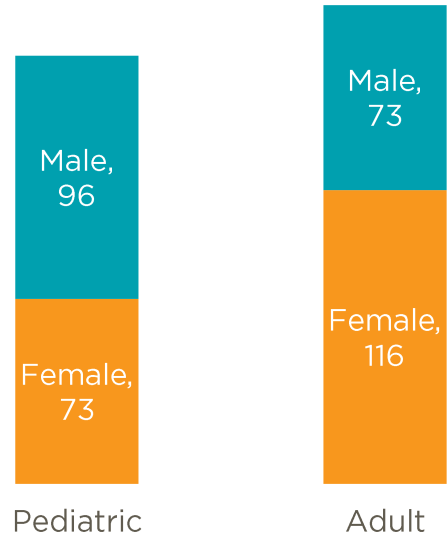
DEMOGRAPHICS

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

American Indian/Alaska Native	1%
Asian	7%
Black/African American	6%
Hispanic or Latino	10%
Native Hawaiian/Pacific Islander	0%
White	73%
Multiple, Other, Unknown	13%

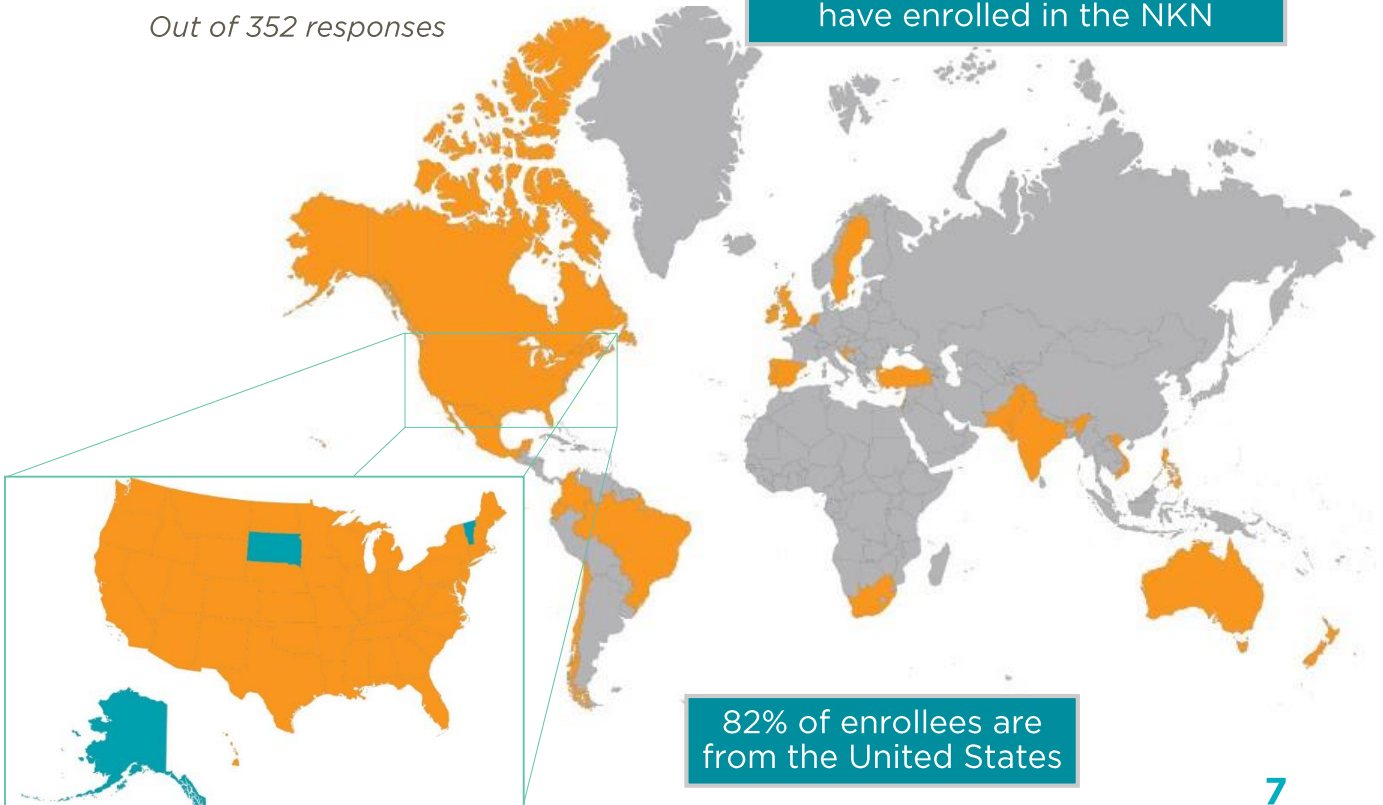
Out of 352 responses

Number of Pediatric and Adult Participants by Gender



Out of 358 responses

Participants from 26 countries have enrolled in the NKN

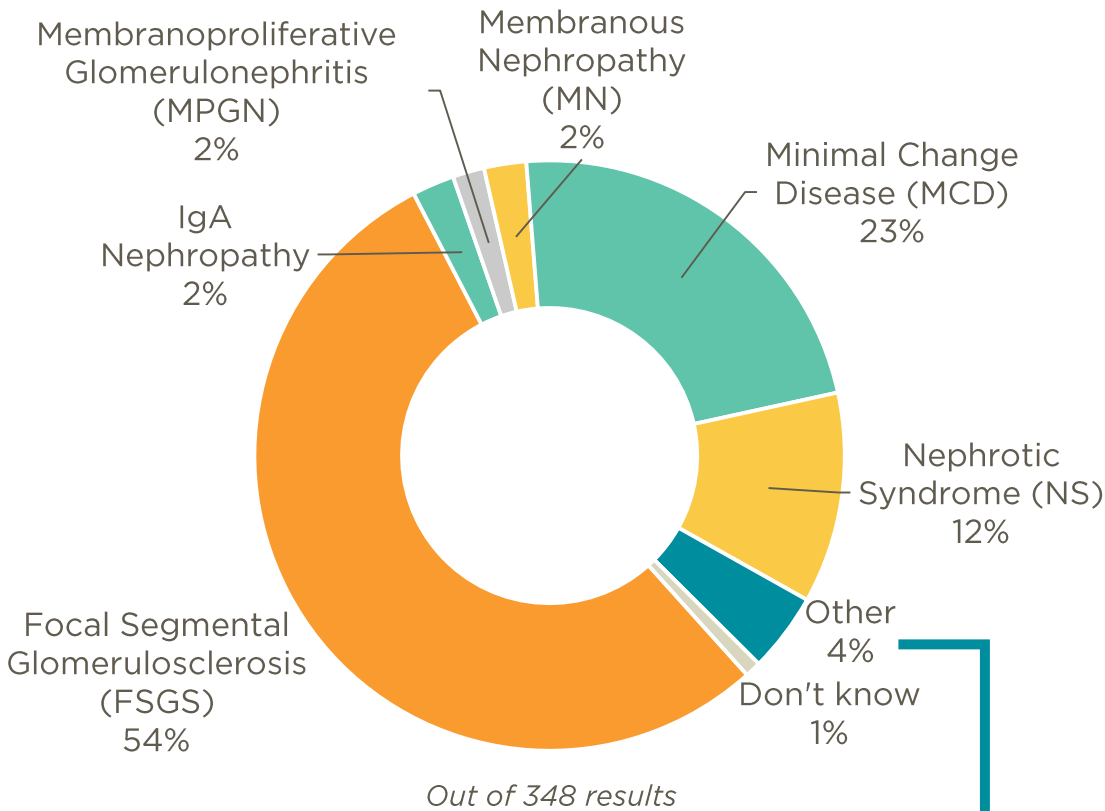


82% of enrollees are from the United States

DIAGNOSIS

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

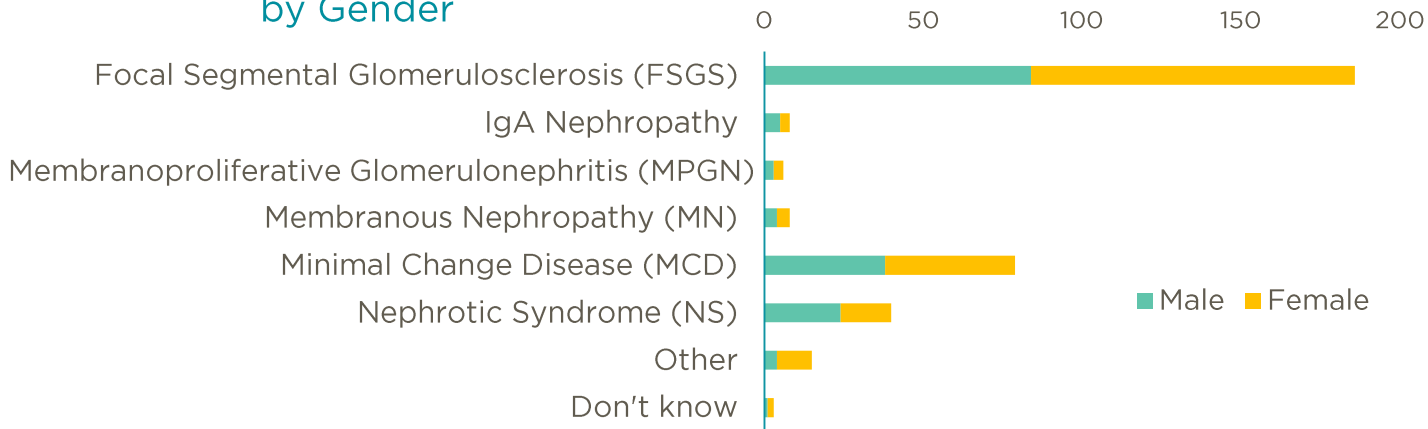
“The NKN registry has allowed me to feel like I am part of a community that is working to find solutions to this terrible disease. I feel that by including my information in the registry, I am helping to build a database of information about a disease for which little is known. My nephrotic syndrome is caused by C1q nephropathy which is extremely rare. There is no cure nor a specified treatment. It is my hope that this registry will help to develop a standard treatment for my disease and others that cause nephrotic syndrome.” – Gretchen H.



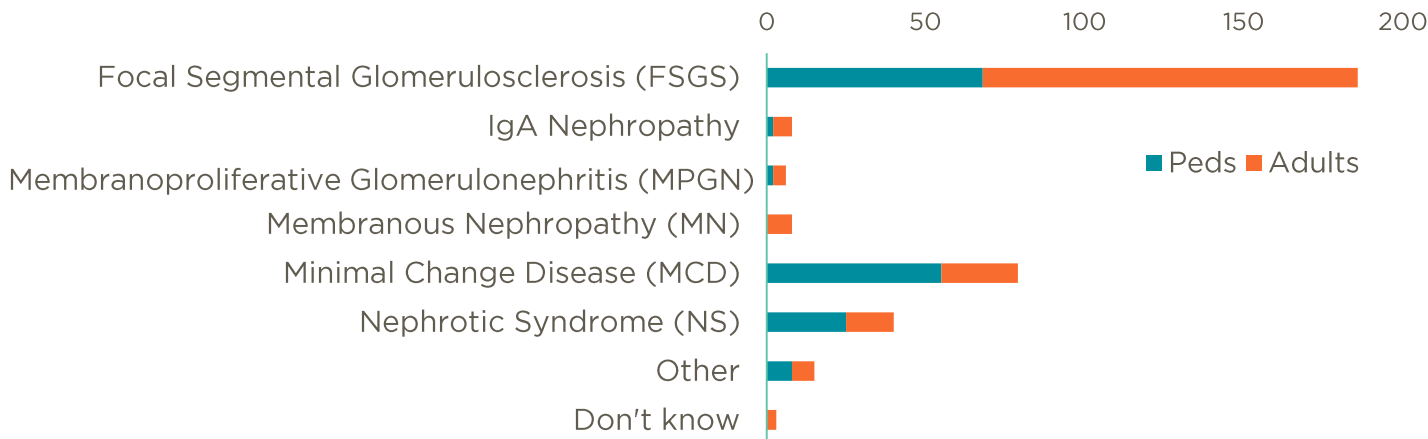
Other diagnoses include:
C1Q Nephropathy
C3 Nephropathy
Diffuse Mesangial Sclerosis
Fibrillary Glomerulonephritis
Fibronectin Glomerulopathy
IgM Nephropathy

DIAGNOSIS - A CLOSER LOOK

Diagnosis Breakdown by Gender



Diagnosis Breakdown by Age



Out of 346 results

"It is a great way to take a stand, be heard, and be an advocate in your own health or the health of your loved one...The only way to get the word out is by sharing your story and putting a face to the disease that could affect anyone."

- Tessa

LABORATORY VALUES

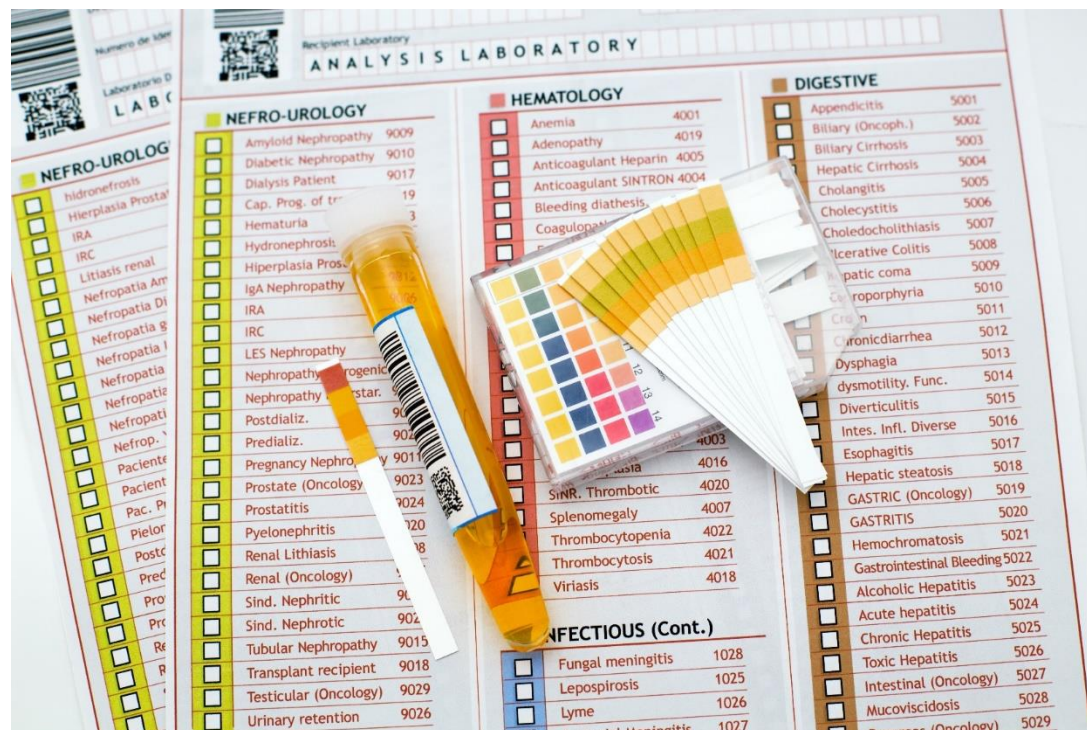
Lab results are an important element of the data captured in the NKN. These values are important for understanding the progression of your disease. For those who are interested in clinical research, they are also the most critical in determining eligibility for studies.

Serum creatinine

Urine protein

Urine protein to urine creatinine ratio (P/C ratio)

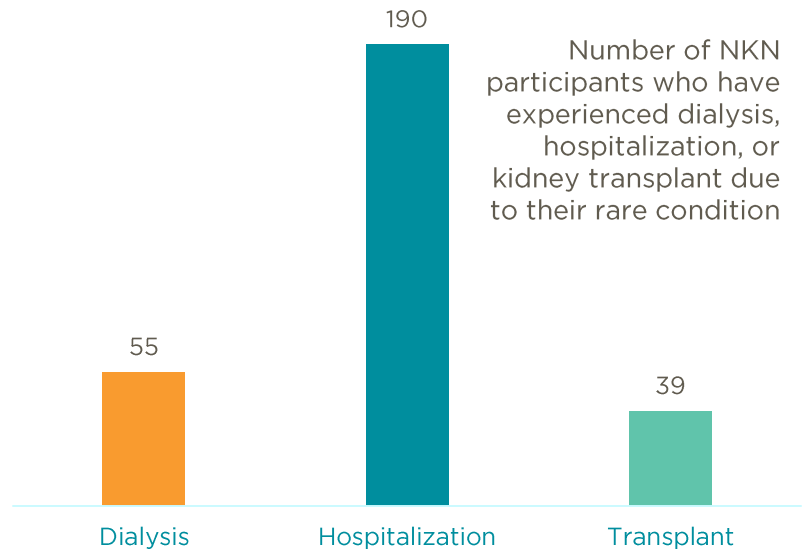
Urine dipstick reading



“This is a great way to “invest” in my FSGS. Patient registry keeps me informed on new studies for my disease. It’s one step closer to a cure.” - Heather L.

TREATMENTS

Because the progression of Nephrotic Syndrome diseases varies, so do the treatments that patients receive. Currently, there is only one drug in the U.S. labeled for use in Nephrotic Syndrome.



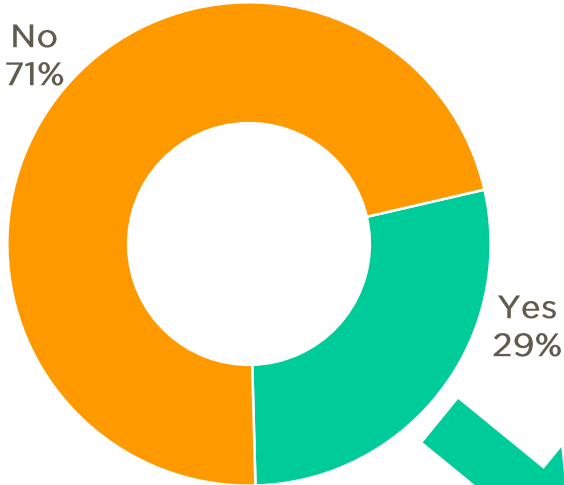
“Registering for the NephCure Kidney Network is one way I can contribute to the body of information about FSGS and related diseases. As a former teacher I understand that without patient input it is very hard to make accurate assumptions. Everyone’s experience of these diseases is so different. It will help identify common symptoms, successful treatments and could contribute to a cure in the long term. FSGS makes you feel so powerless. This is a way each of us can make a meaningful contribution.”
- Jane S.

Medications participants report taking (in order of most often prescribed)

- Prednisone, Prednisolone, Solumedrol (steroids)
- ACE inhibitors (Angiotensin-converting-enzyme inhibitor such as Lisinopril, Enalapril, Ramipril etc.)
- Blood pressure lowering medicine of any type
- Cholesterol lowering medication
- Prograf (Tacrolimus)
- Others
- Water pill (diuretic) of any type
- Cellcept or Myfortic (Mycophenolate Mofetil, Mycophenolic Acid)
- ARB (Angiotensin II Receptor Blocker such as Losartan, Candesartan etc.)
- Neoral, Sandimmune, Gengraf (Cyclosporine)
- Rituxan (Rituximab)
- Acthar (Adrenocorticotrophic hormone, ACTH)
- Cytoxan (Cyclophosphamide)
- Imuran (Azathioprine)
- Plasmapheresis (plasma exchange)

TREATMENTS (ALTERNATIVE THERAPIES)

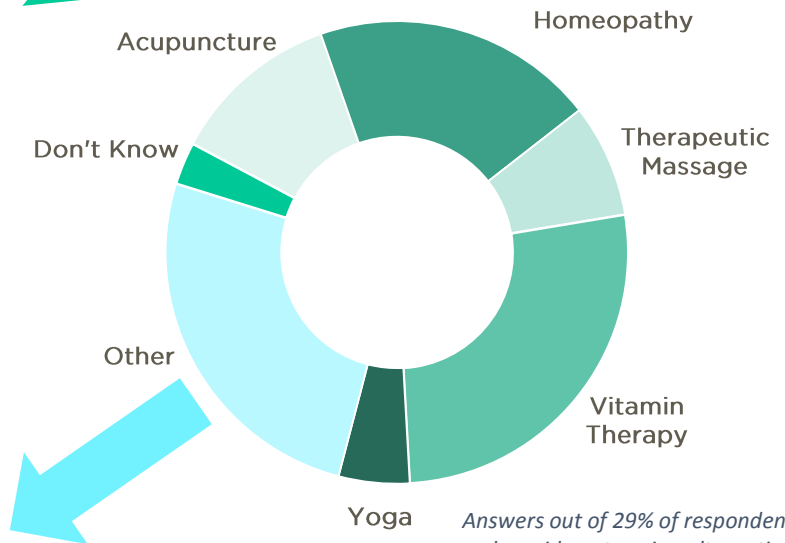
Do you use Alternative Treatment Methods?



Out of 260 responses



Alternative Treatments used by Patients



Answers out of 29% of respondents who said yes to using alternative medicine; may have reported multiple therapies

“Other” answers were:

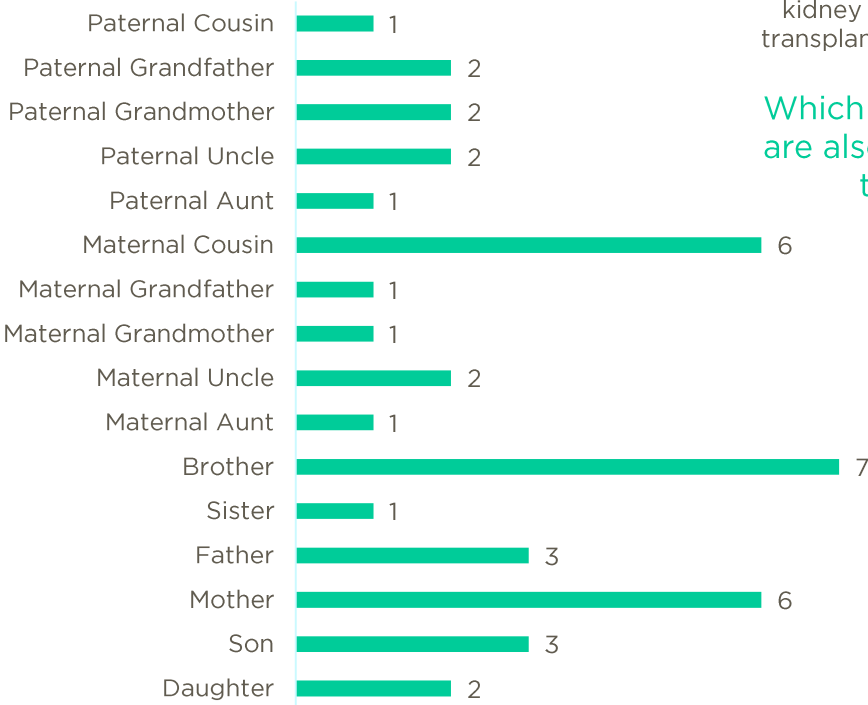
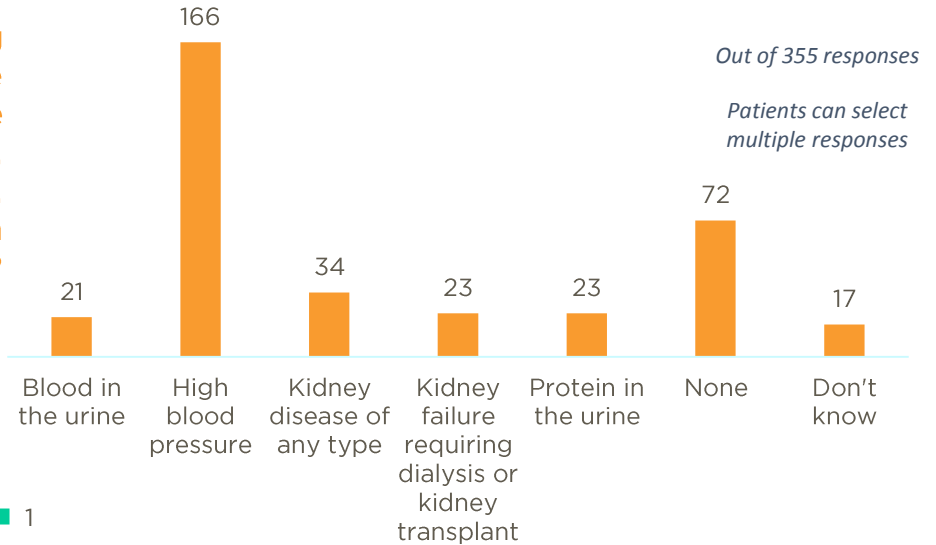
- Acupressure
- Alkaline water
- Anti-inflammatory diet
- Chinese herbal medicine
- Chiropractor
- Dairy free
- Essential oils
- Exercise
- Fish oil
- Fitotherapy
- Galactose
- Gluten-free
- High dose omegas
- IVIG infusions
- L-glutamine
- Meditation
- Osteopathic manipulation
- Probiotic
- Reflexology
- Reiki
- Weight loss

Because treatment options are limited, many patients seek alternative or complementary therapies. However, little research is currently being conducted in this area. Your participation in the NKN could help drive new research priorities.

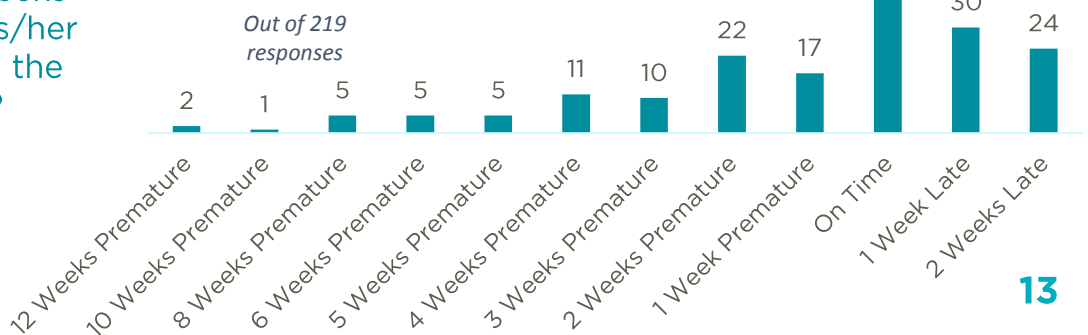
FAMILY HISTORY

Because many diseases have a genetic component, family history is an important topic to cover in our registry.

Is there a history of any of the following disorders in the patient's immediate family (grandparents, mother, father, brother, sister, children or grandchildren)?

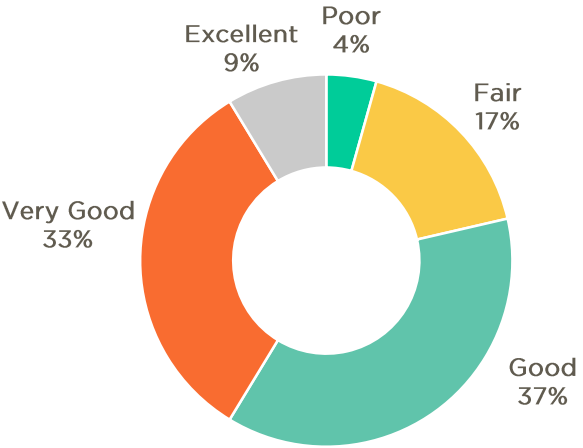


How many weeks away from his/her due date was the patient born?



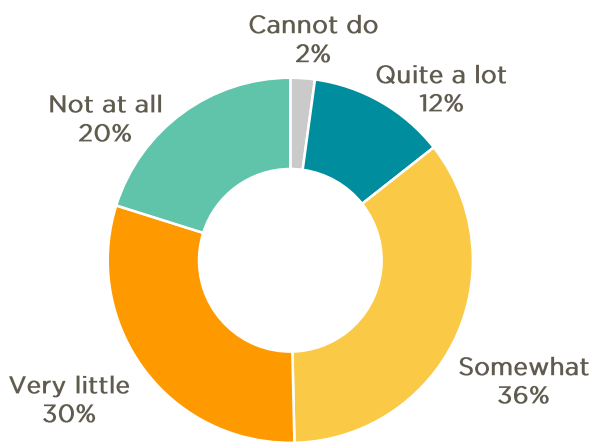
PATIENT REPORTED OUTCOMES (PROs)

In general, how is the patient's health?



Out of 276 responses

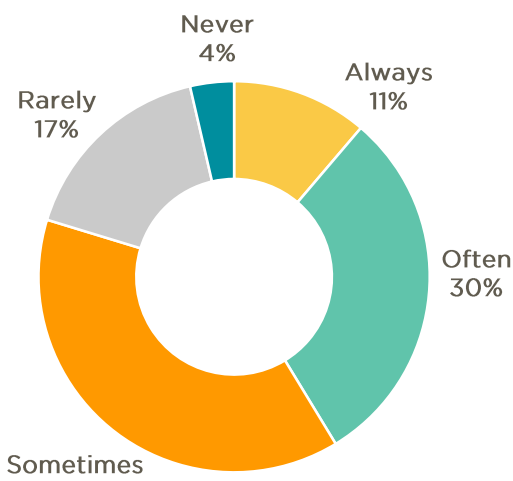
Does the patient's health now limit him/her in doing vigorous activities?



Out of 279 responses

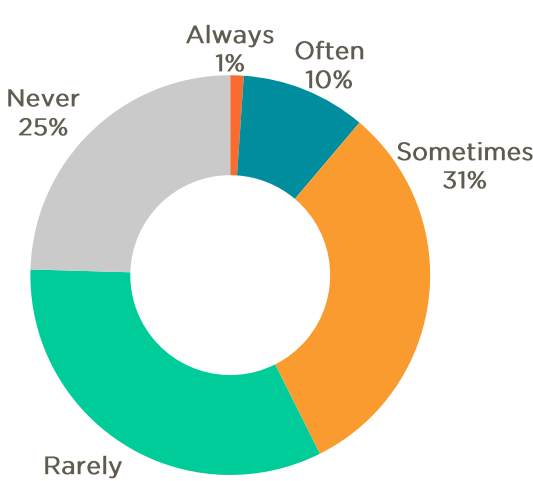
Physicians can collect many lab values, but only patients can tell us how their disease affects them. Patient reported outcomes help researchers understand the true impact these diseases have on a patient's life.

How often does the patient feel tired?



Out of 277 responses

How often does the patient feel depressed?

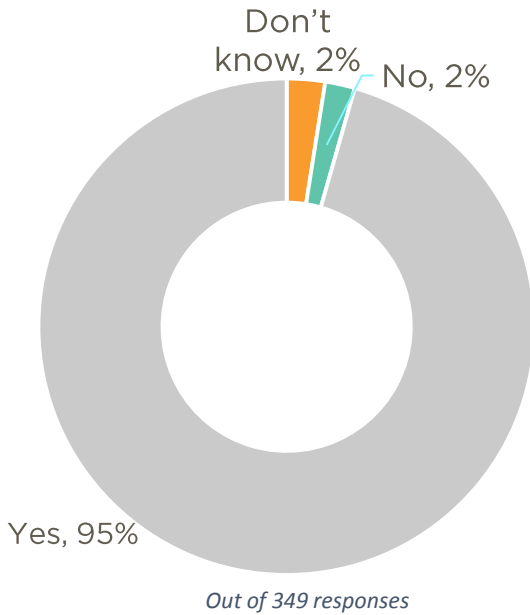


Out of 277 responses

RESEARCH

“Because this is a rare disease, we really need to make some noise to keep research going, to find better treatment options and possible cures.” - Jeff B.

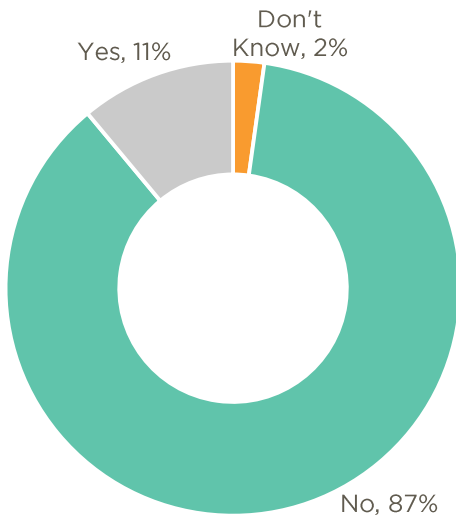
Is the patient willing to be contacted in the future about research related to their rare disease?



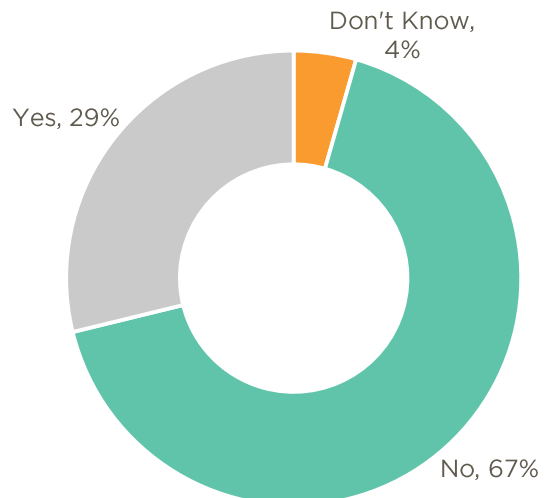
Without patient participation in research, new therapies will not make it to market.

Because Nephrotic Syndrome diseases are rare, your participation is vital. Joining the NKN is a great first step!

Has the patient PREVIOUSLY participated in any clinical trials related to their rare disease?



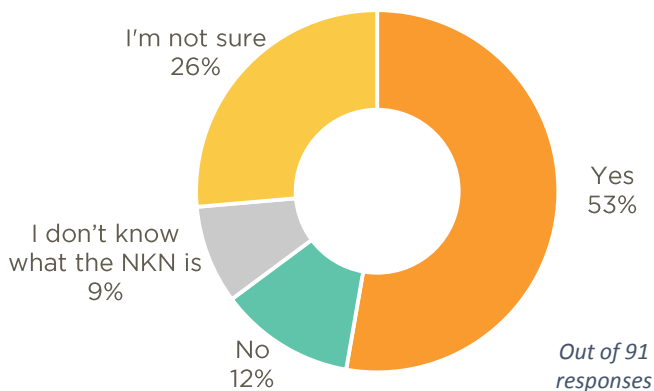
Has the patient donated a sample of blood, tissue, or other biospecimen for research in the past?



FEEDBACK SURVEY

To assist us with NKN enrollment efforts, NKI recently surveyed our broader patient community about whether or not they had enrolled in the NKN and if so, why they chose to participate. For those not enrolled, we wanted to understand what prevented them from doing so.

“Have you enrolled in the NephCure Kidney Network (NKN) empowered patient registry?”



If no, please share with us why you've chosen not to enroll in the NKN (select all that apply)	
I don't want to share medical information	2
I don't know enough about the NKN	8
I don't understand how the information in the NKN will be used	4
I am unsure how to enroll	7
I believe it will take too much time to enroll	1
I don't want to be in a clinical trial	2
I don't believe it will benefit me	2
I didn't know I was eligible	7
I am already enrolled in another registry	0
I haven't enrolled yet but plan to soon	3
Other	7

If yes, please tell us why you chose to enroll (select all that apply)	
I want to donate my data to help find better treatments	45
I want to learn how NS affects other patients	21
I want to be connected to new therapies via clinical trial notification	28
I believe a patient registry could encourage researchers to study our community	48
My doctor suggested it to me	3
Another patient suggested it to me	2
Other	4

Why are you unsure if you've enrolled in the NKN?	
I answered questions for NephCure previously but I am unsure if it was to join the NKN	20
I think I created an account and can't find my username/pasword	8
I believe someone else enrolled me, as I receive information from NephCure already	0

“What I would like others to know about the registry is upon diagnosis everyone wishes there was just one “thing” that could be done to fix the problem of our family member or ourselves. Filling out this questionnaire is perhaps that one “thing” that leads a drug company or researchers down the path of discovering something helpful. There aren't many of us out there to provide this data so every data point is critical. We all need to do our part and make an impact toward a solution.”

– Shannon C.

THANK YOU!

Thank you for your continued participation in the NephCure Kidney Network (NKN) patient registry. The sharing of each patient's journey, including yours, through data is vital in our search for better treatments. As the registry grows, so does our knowledge about these diseases. Our hope is that the registry will help researchers generate new ideas on how to diagnose, treat and potentially cure Nephrotic Syndrome (NS) and its related diseases.

We hope that your participation in the NKN patient registry has empowered you. By donating your information you have taken an active role in research that will benefit the entire NS community. We are honored that so many patients and families have shared their personal stories with us and remain committed to the fight against these diseases.

With your help, we have learned a lot about the needs of our patient community in the year since launching the NKN. Because of your feedback, we are in the process of updating the survey to make it more user-friendly. We will also be introducing a better way to integrate your Electronic Health Records data with your registry profile. And we are excited to announce that we are developing a new mobile app that will make updating your registry profile even easier.

To learn more contact the patient registry manager at registry@nephcure.org or call 610-540-0186 x19.

Thank you for your support.

Joanna Dauber



MEET OUR TEAM

The Steering Committee (2014):

Kathleen Broderick (co-chair)	Elaine Kamil, MD
Randall Snyder, MD (co-chair)	Radko Komers, MD, PhD
Julie Abramson	Laura Mariani, MD, MS
Patrice Becker, MD	Michael Mittelman
Elizabeth Cope, PhD, MPH	Meg Modes
Joanna Dauber	Martin Pollak, MD
Debbie Gibson, MD, MS	Junelle Speller
Marilyn Hailperin, MS	Angela Sample
Sergio Infante, MD	

NKN Co-Primary Investigators:

Elizabeth Cope, PhD, MPH
Marilyn Hailperin, MS

NKN Investigators:

Laura Mariani, MD, MS
Debbie Gipson, MD, MS

NephCure Kidney International Staff:

Joanna Dauber, Patient Registry Manager
Abbey Swan, Research Operations Manager

Arbor Research Collaborative for Health Staff:

Ashley Schork, MNM, Senior Project Associate
Lalita Subramanian, PhD, MPH, Project Manager

Work Groups:

*Community
Engagement
Group*

*Patient
Engagement
Group*

*Data
Collection
Group*



STAND UP & BE COUNTED!

THE NEPHCURE KIDNEY NETWORK