# Stories that Matter

## NephCure Kidney Network Patient Registry

Annual Report
March 2016-March 2017

ARBOR RESEARCH
COLLABORATIVE
FOR HEALTH

NEPHCURE®
Kidney International
Saving Kidneys • Saving Lives

# Contents

DATA OVERVIEW

# The NKN

The NephCure Kidney Network is a patient registry with information provided by individuals with Nephrotic Syndrome or family members of patients. 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:

- Give patients suffering with Nephrotic Syndrome diseases and their caregivers a platform to share their health stories to accelerate the development of new therapies
- Provide participants with more direct access to clinical research opportunities
- Support the development of new, safe, and reliable therapies for primary Nephrotic Syndrome

# Welcome

## The NephCure Kidney Network (NKN) is a unique platform where patients can share their health stories to better define their disease experiences.

Participation in the NKN has been described as both empowering and enlightening because it forces patients to wrap their arms around their disease in a whole new way. Our hope is that we can harness this sense of empowerment and use it for the greater good.

This past year, the NKN gained increasing visibility amongst patients across the globe. Each person tells a different story, yet there are themes common to everyone impacted by Nephrotic Syndrome. Knowing this, we introduced a new retention tool called Kidney Qs. These are simple one question surveys sent to NKN participants to deepen engagement and offer insight into community preferences and attitudes. Since October 2016, each Kidney Q has yielded more responses than the previous month. Not only do the answers provide a glimpse into the patient experience, they are 'conversation starters' for what we hope becomes a more meaningful dialogue.

Although there is more work to be done to recruit NKN participants, we are pleased with the progress we have made since the registry launched in March 2014. Because of the NKN, we know more today than we did then about how patients perceive clinical trials, what medications tend to have the worst side effects and whether or not patients adhere to dietary guidelines. Collectively, these stories give researchers a more holistic picture of Nephrotic Syndrome and, with continued growth, will ultimately help guide their research efforts.

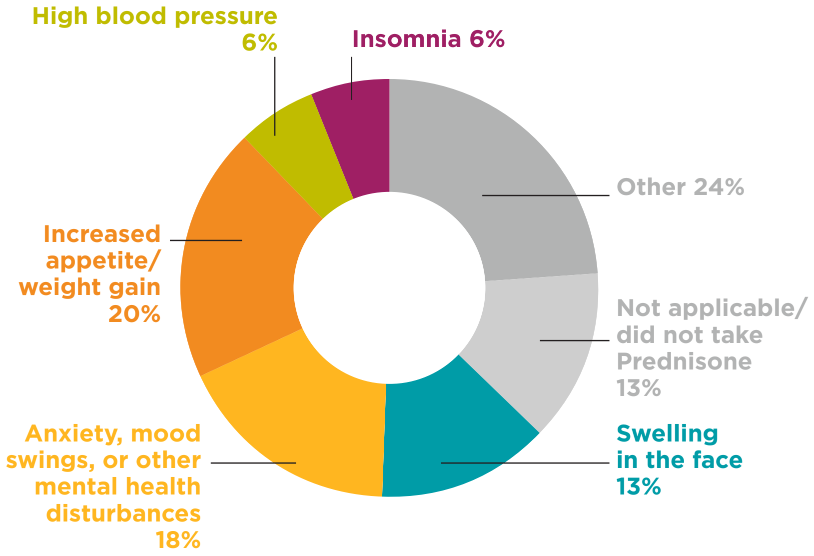
We are grateful for the myriad of partners we work with to make the NKN possible. Arbor Research Collaborative for Health provides exceptional leadership and expertise that is so vital to the success of the project. Partners at the University of Michigan, Children's Hospital of Philadelphia and the University of Toronto ensure that meaningful, patient-centric research is a priority. Very special thanks to the volunteers who comprise the NKN Steering Committee. We value your time, your commitment, and your voice that serves to represent the stakeholders invested in the Nephrotic Syndrome community. Finally, none of this would be possible without the patients and families who found the NKN and took the time to share their stories. We look forward to the day your contributions will pay off in the form of a better understanding of these diseases and medical breakthroughs leading to a cure for Nephrotic Syndrome.

> "...none of this would be possible without the patients and families who found the NKN and took the time to **share their stories.**"

**Lauren Lee**
NKN Co-Principal Investigator
Director of Research and Engagement
NephCure Kidney International

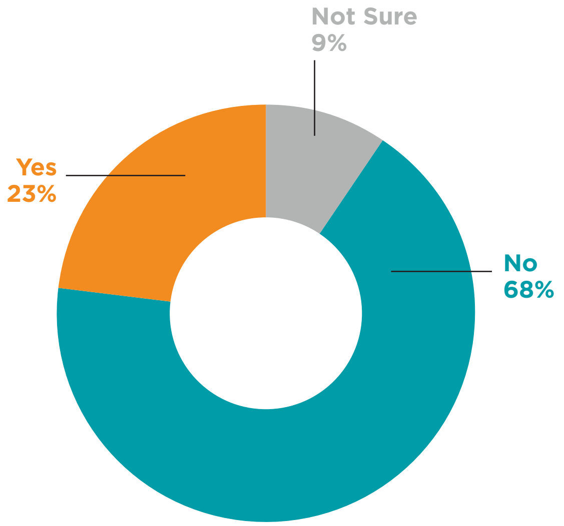
# Kidney Qs

Which side effect of Prednisone most affected (or still affects) your day-to-day life?



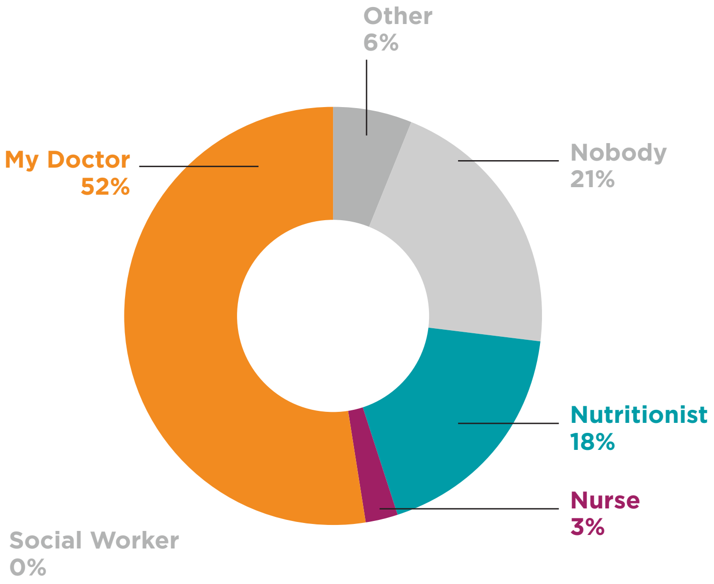
N=97

Do you recall hearing about the DUET clinical trial for FSGS patients (testing Sparsentan to reduce proteinuria)?



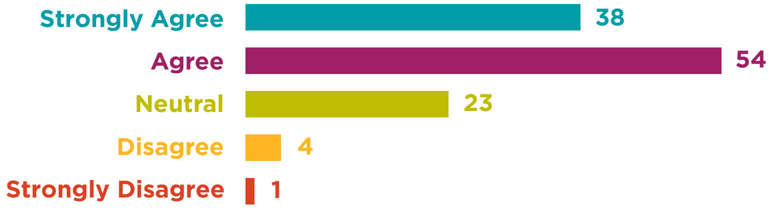
N=86

After receiving a diagnosis of Nephrotic Syndrome, who (as part of your health care team) talked to you about changing diet and nutrition habits?



N=116

To what extent do you agree with your current treatment plan?

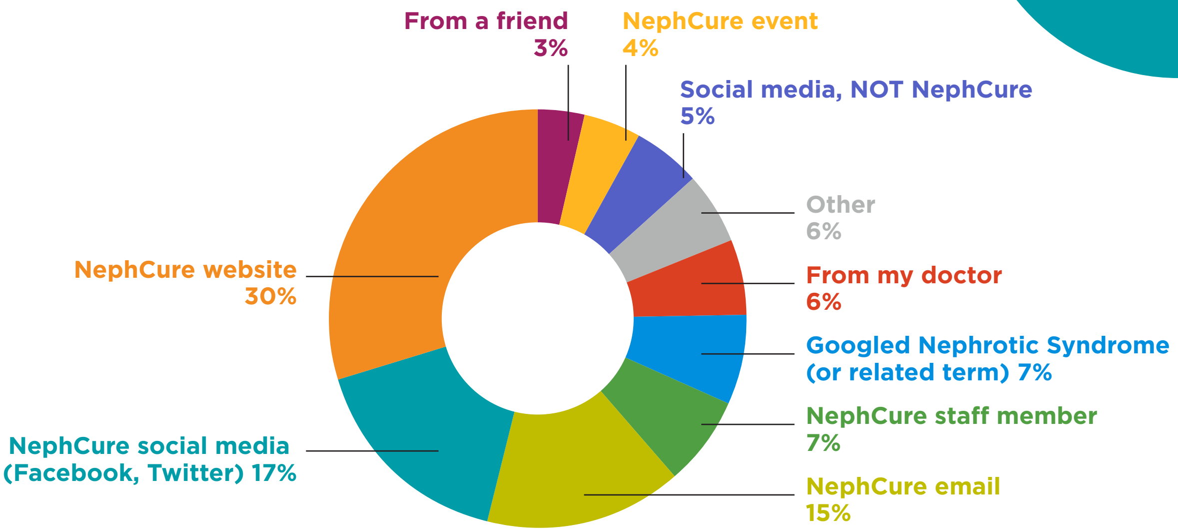


N=120

# Recruitment

**666 participants have enrolled in the NKN patient registry as of March 2017**

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



N=343

## Recruitment efforts for the NKN included:

- Emails and mailings to all patients in NKN'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

> "As part of the rare disease community, we wanted to do what we could to help find a cure and better treatment options for **Nephrotic Syndrome.** We knew the data we donated would be used for this purpose, so we were happy to do it!"

**Lisa**

# Geography

NephCure Kidney Network includes participants from around the world.

**Participants have enrolled in the NKN from 40 different countries**

## 583 Participants from top 5 countries:

| | |
|---|---|
| **511** | **United States** |
| **29** | **Canada** |
| **21** | **United Kingdom** |
| **11** | **Australia** |
| **11** | **India** |

N=666

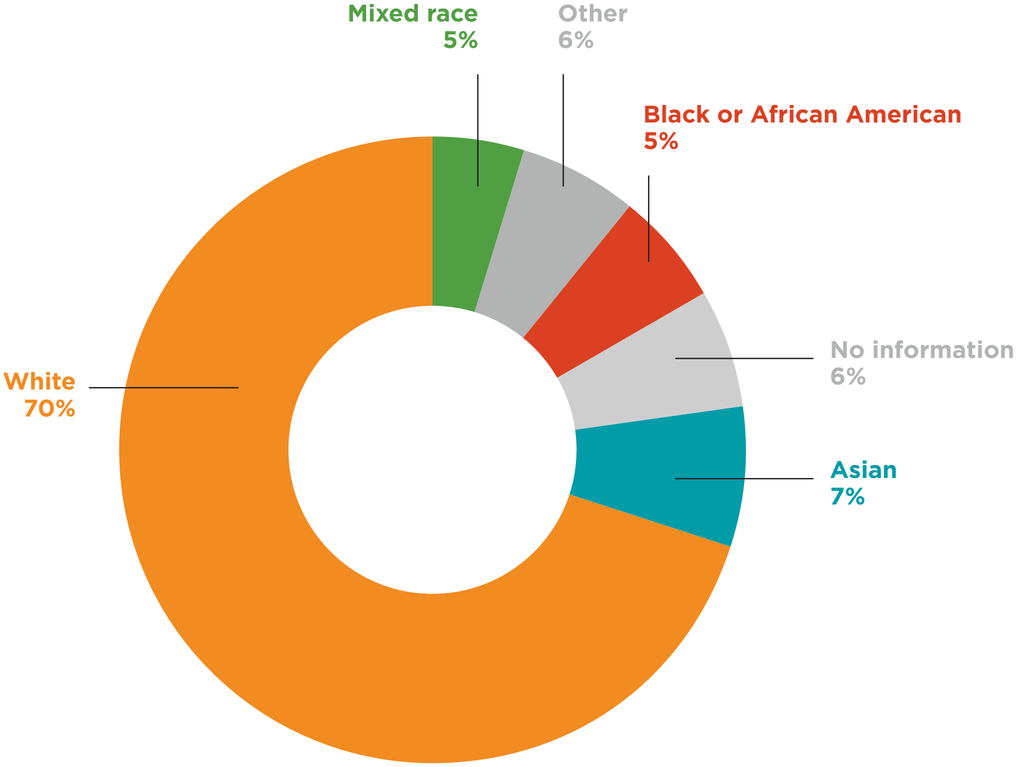
**Additional participants from the following countries:**

Bahamas
Bahrain
Bangladesh
Brazil
Bulgaria
Chile
Colombia
Croatia
Ecuador
Finland
France
Germany
Greece
Ireland
Israel
Japan
Kenya
Rep. of Korea
Lithuania
Malaysia
Mexico
Netherlands
New Zealand
Pakistan
Philippines
Portugal
Puerto Rico
Scotland
South Africa
Spain
Sweden
Switzerland
Trinidad and Tobago
Turkey
Viet Nam

# Demographics

The NephCure Kidney Network seeks to learn about the diversity of people living with Nephrotic Syndrome.

**10% of responding users also report Hispanic ethnicity**



N=666

"This has been a trying experience for my son (age 2) who has Nephrotic Syndrome, and our family. Anything that I (we) can do to make this easier on other families, **we're in!"**

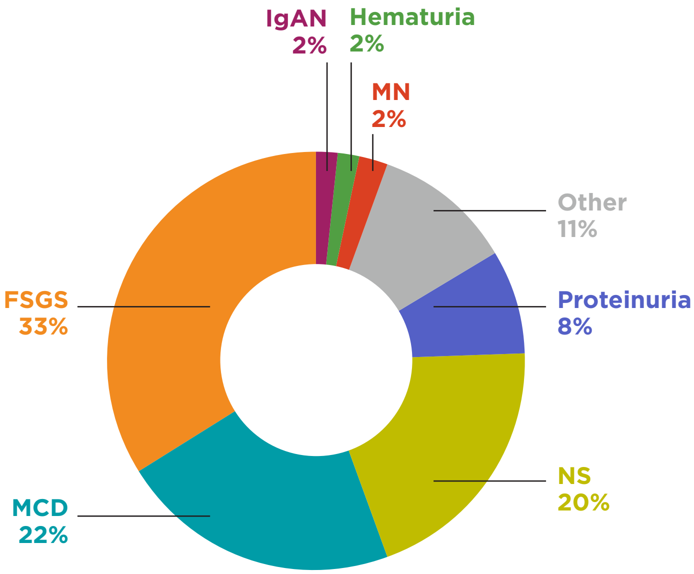
**Erin**

# Diagnosis

What is the patient’s kidney disease diagnosis?

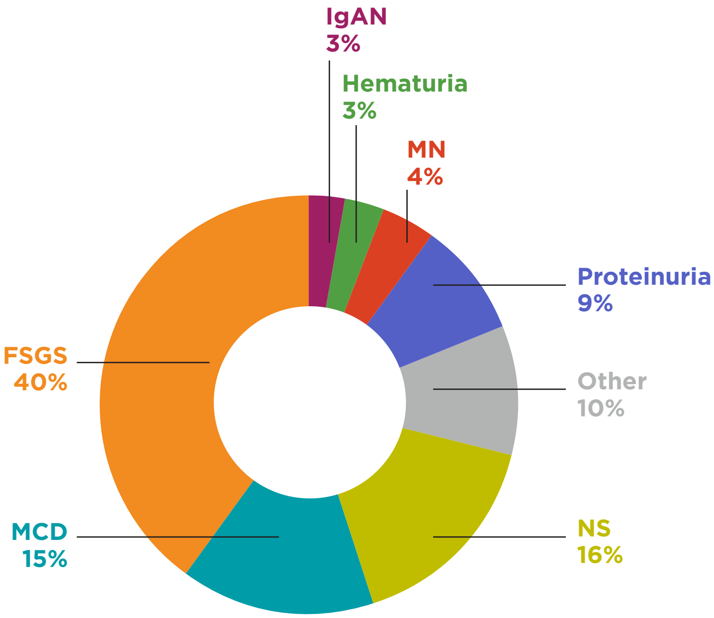
|  |  |  |
|---|---|---|
| ● | **FSGS** | Focal segmental glomerulosclerosis |
| ● | **IgAN** | IgA Nephropathy |
| ● | **MN** | Membranous nephropathy |
| ● | **MCD** | Minimal change disease |
| ● | **NS** | Nephrotic Syndrome |

### Adult and Pediatric Responders



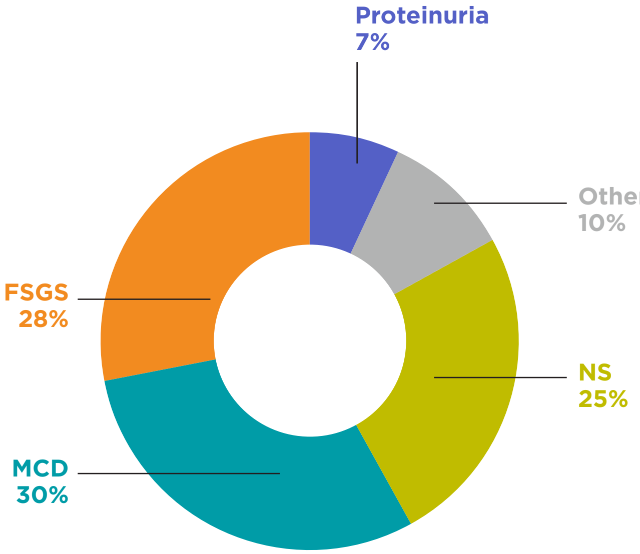
N=824
(multiple responses allowed)

### 342 Adult Responders
(18 years or older)



N=421
(multiple responses allowed)

### 295 Pediatric Responders
(under 18 years old)

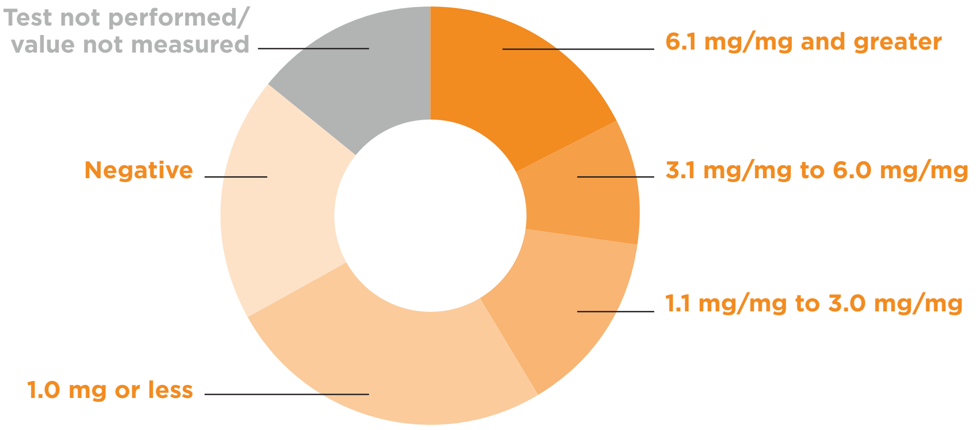


N=359
(multiple responses allowed)

# Lab Values

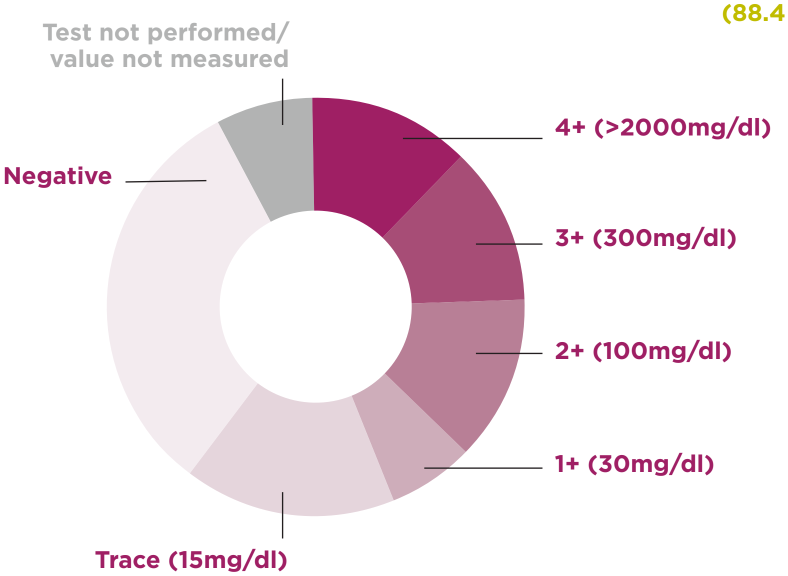
**Many NKN participants responded "Don't Know" to questions about lab values. It is the goal of the NKN to help educate patients and families about the importance of tracking lab values. Upcoming initiatives include tracking tools and a mobile app.**

What is the patient's **most recent** urine protein to urine creatinine ratio (P/C ratio)?



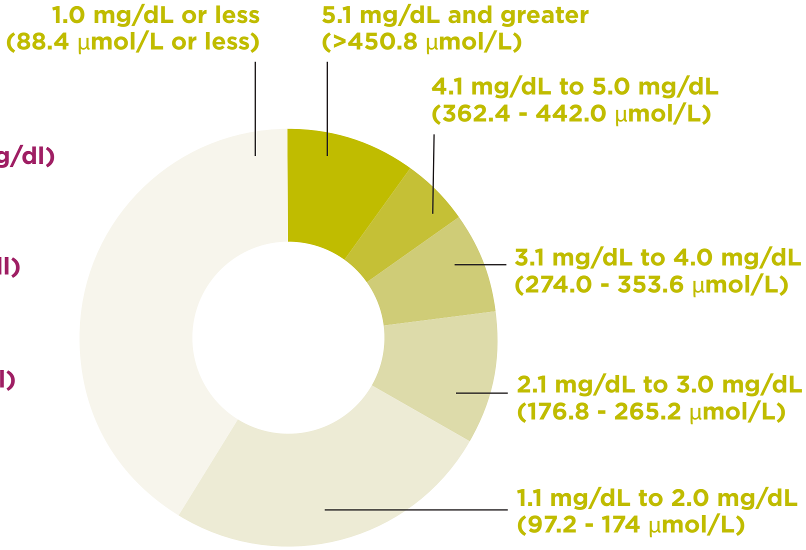
N=121

What is the patient's **most recent** urine dipstick for urinary protein reading?



N=385

What is the patient's **most recent** serum creatinine?



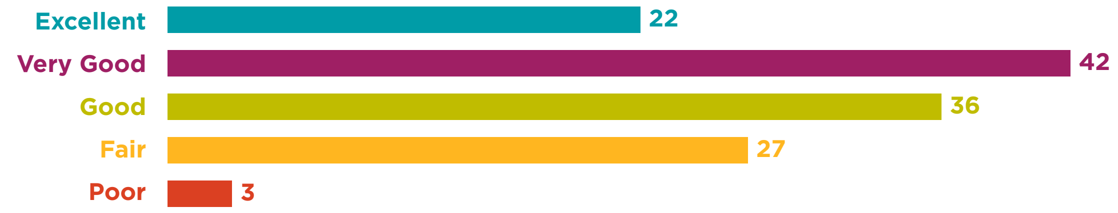
N=153

# Quality of Life

In general, how would you say your health is: (adults)

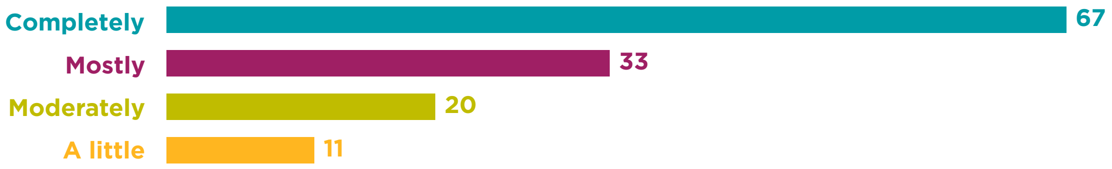
N=417

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



N=130

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)



N=131

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

N=43

**This question is answered by pediatric patients between the ages of 10 and 17.**

# Treatment

Since diagnosis, has the patient taken any immunosuppressant medications?

**86% of 276 responders have used an immunosuppressant since diagnosis.**

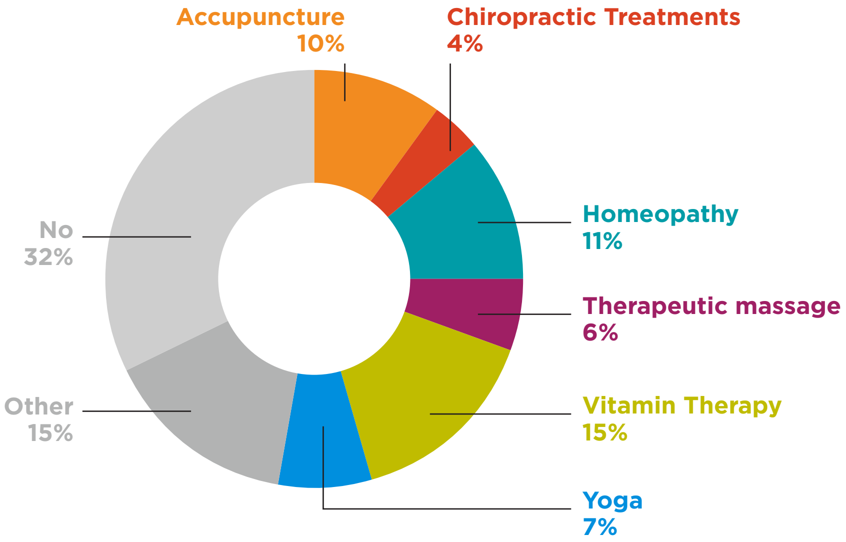
Since diagnosis, has the patient taken any blood pressure medications?

**72% of 272 respondents told us that they took some sort of blood pressure medication since their diagnosis.**

Is the patient currently taking any other medications for their kidney disease?

**69% of responding users take "other" medications for their kidney disease, including water pills, blood thinners, and cholesterol-lowering medications.**

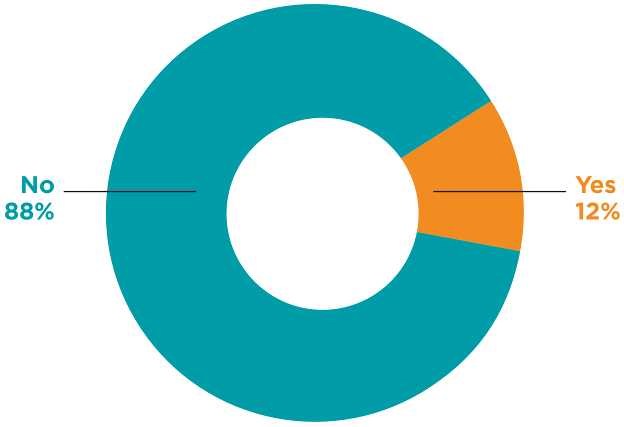
Has the patient used any methods of complementary/alternative therapies or supplements to treat his/her kidney disease? (Select all that apply.)



N=427

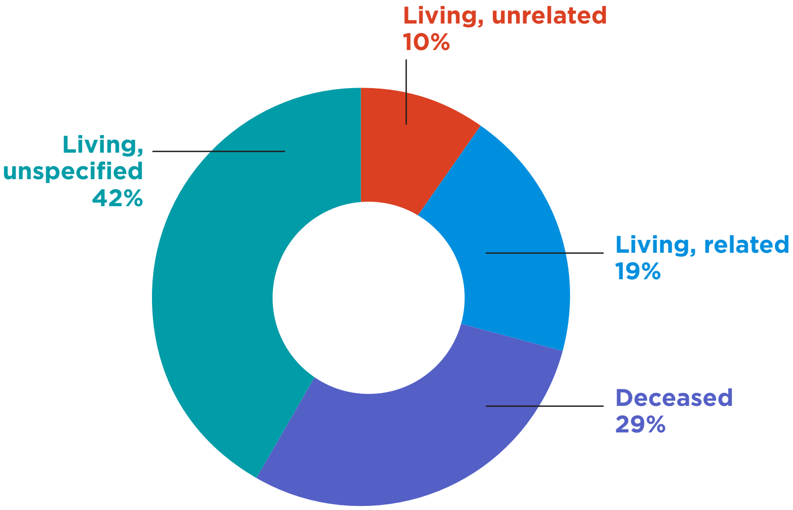
# Treatment (CONTINUED)

Has the patient ever had a kidney transplant?



N=531

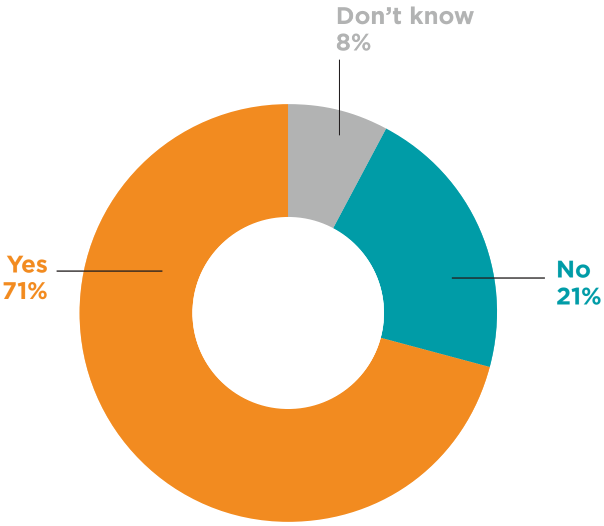
If yes, what type of a kidney transplant did the patient most recently receive?



N=62

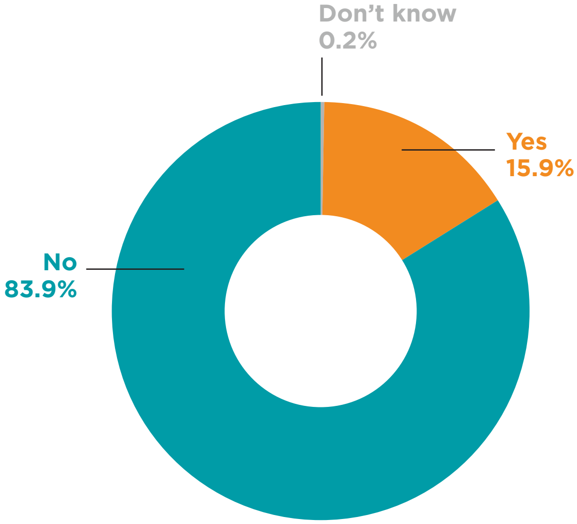
**This question is answered by those that responded "yes" to ever having a kidney transplant.**

Did the patient have genetic testing?



N=540

Has the patient ever had dialysis?

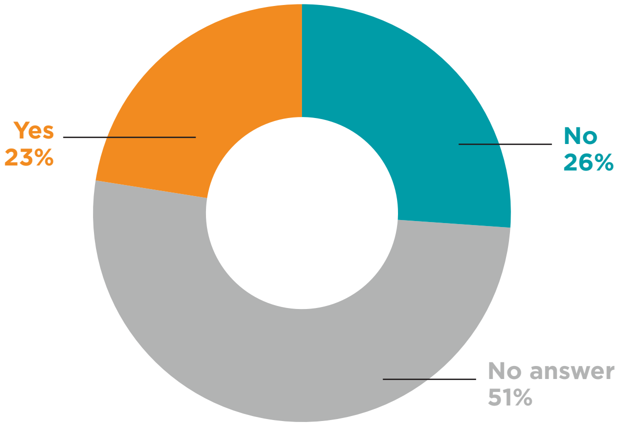


N=529

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

**24**

If yes, is the patient currently on dialysis?



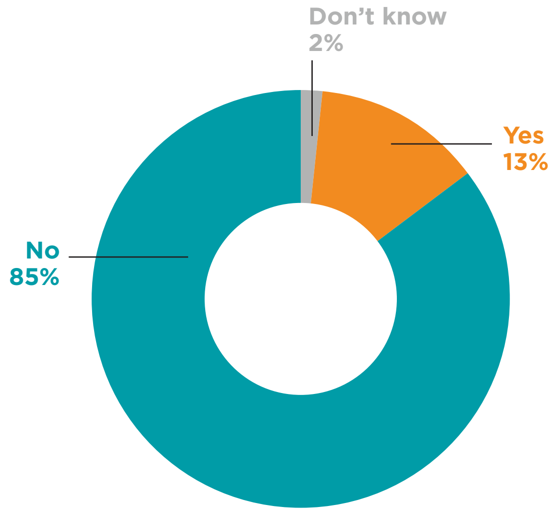
N=41 (out of 84)

> "Sharing data allows researchers to establish trends and uncover possible avenues to explore in hopes of **finding cures** for disease. Sharing is unselfish and lifesaving."

**Kent**

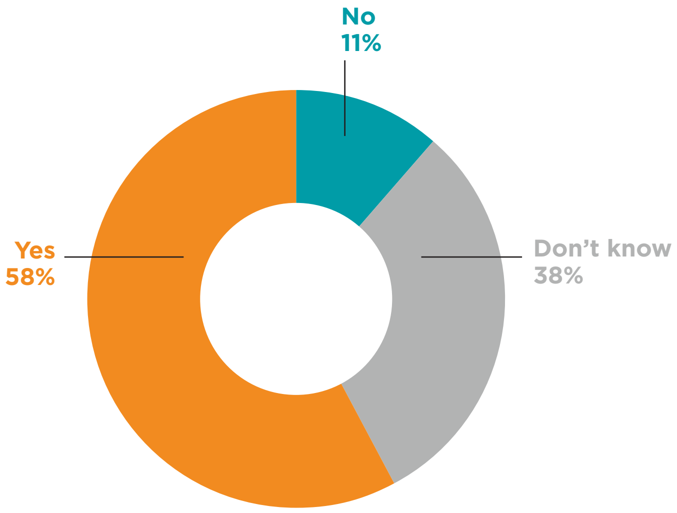
# Research Participation

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



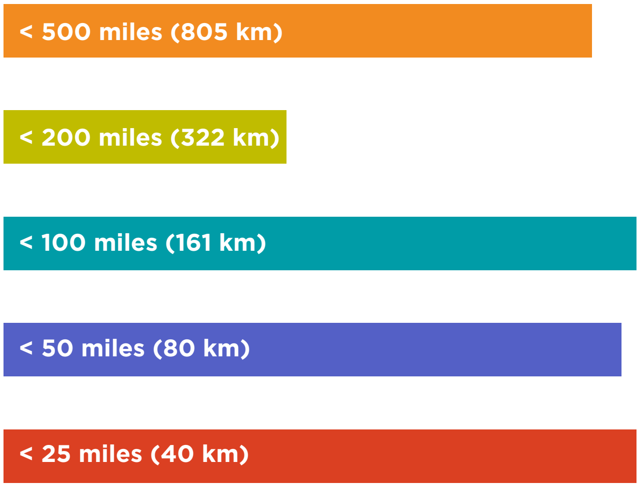
N=491

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/her?



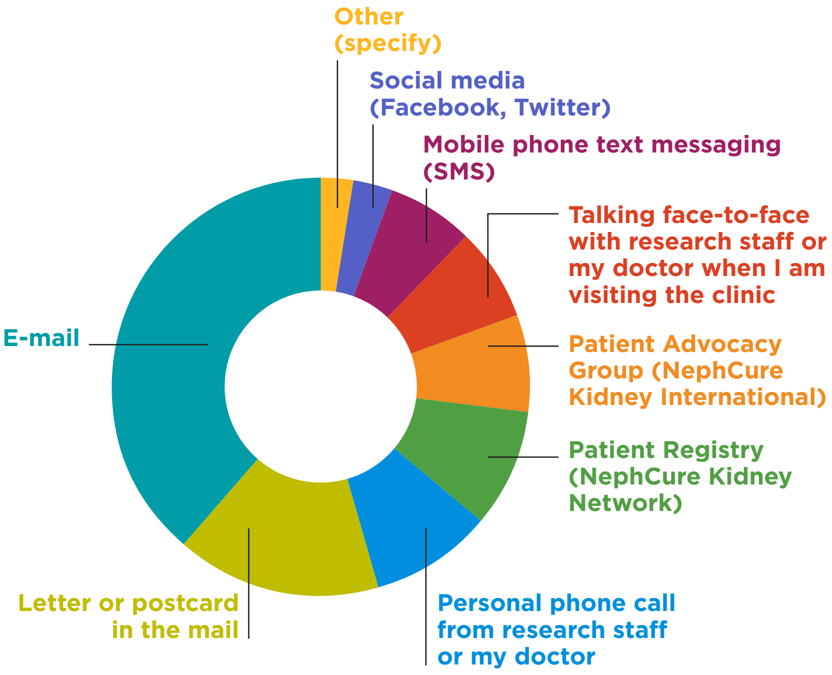
N=480

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



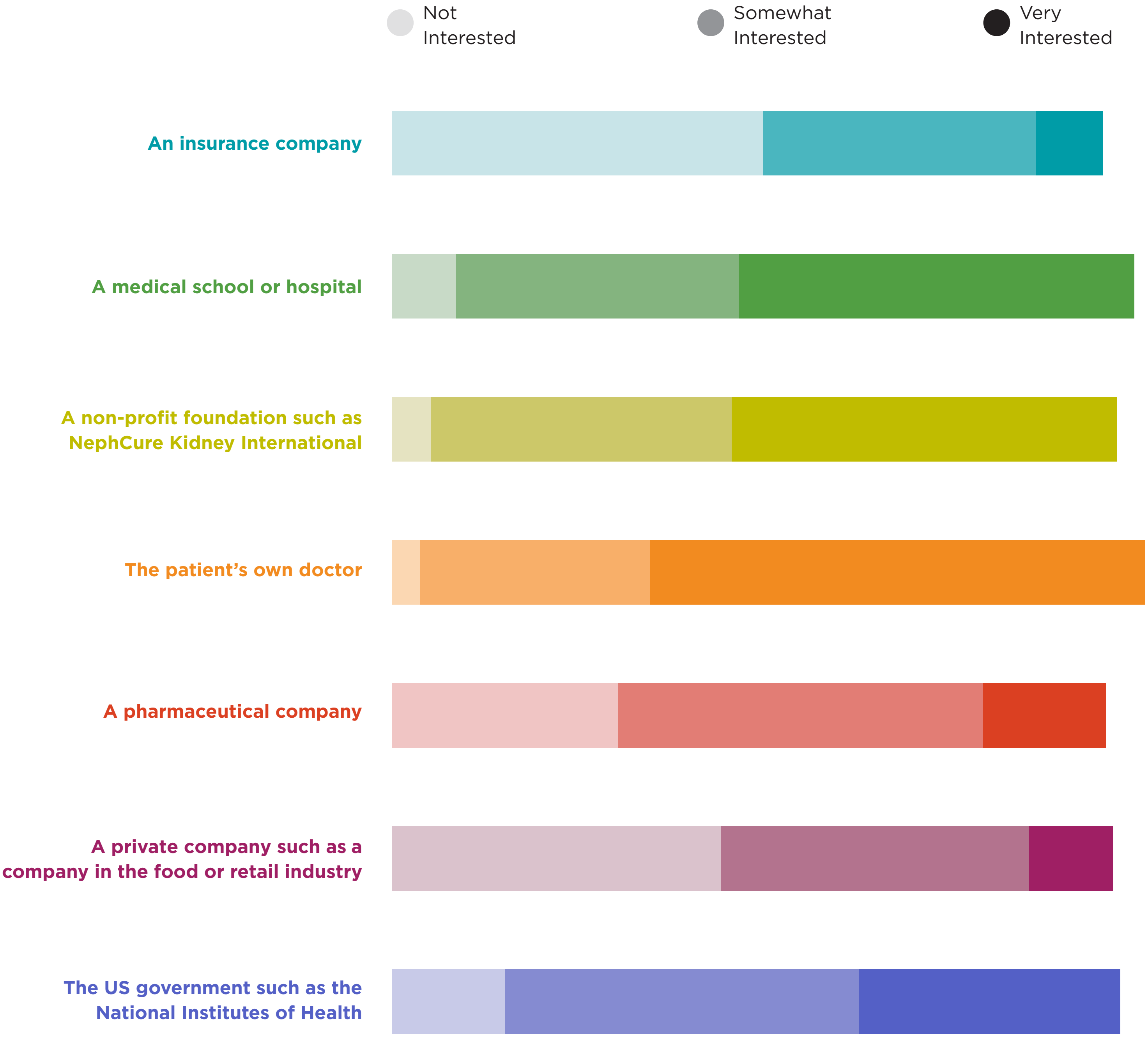
N=370

How would the patient prefer to be contacted to learn about potential research studies?



N=530

## 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?)



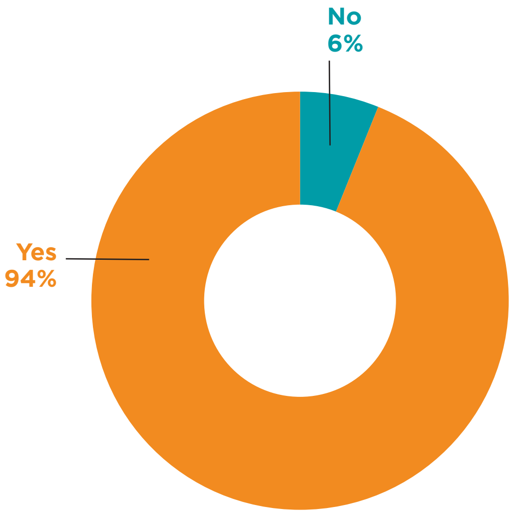
N=213

# Health and Healthcare

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

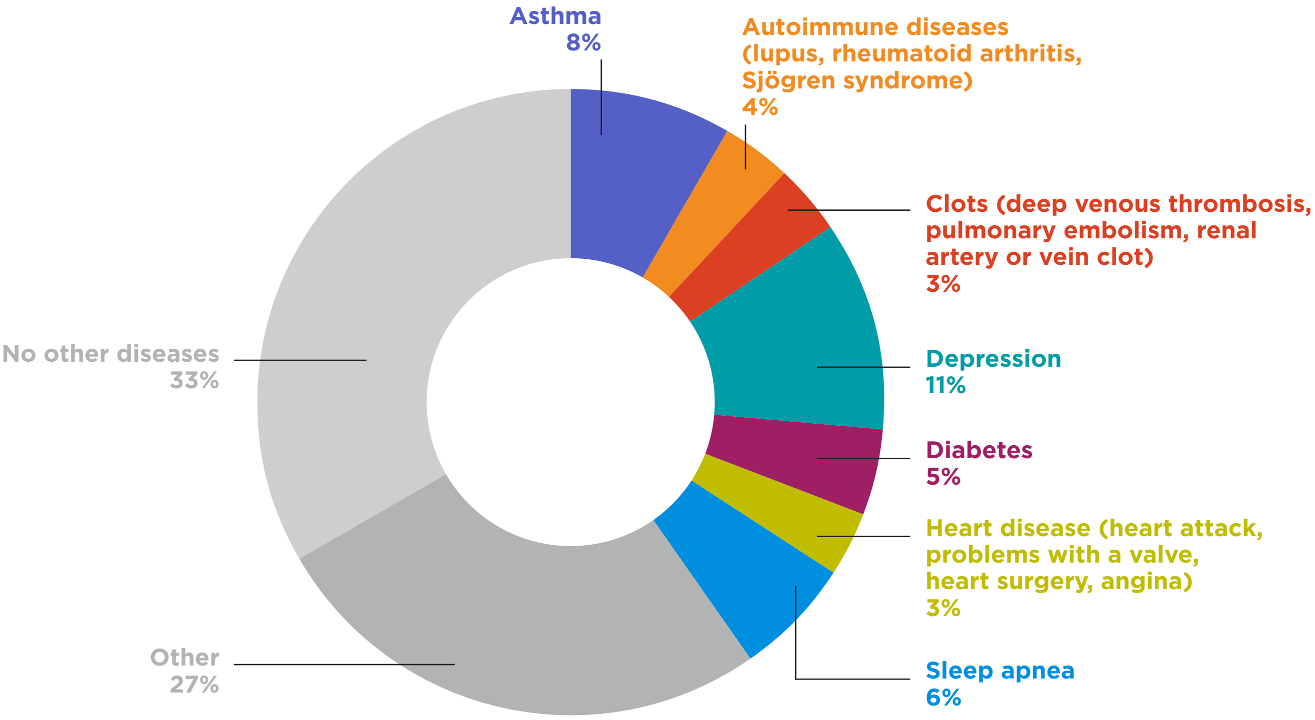
N=130

Does the patient have health insurance or healthcare coverage?



N=471

Does the patient have any diseases or other conditions in addition to his/her kidney disease?



N=294

**“Completing surveys** in the NKN is one simple thing that everyone can do to move research forward regardless of where you live or where you are in your journey.”

**Kelly**

# Your Data at Work

The NKN promotes inclusion of patients' perspectives in identifying and conducting studies so that researchers ask relevant questions and look for meaningful solutions to address the challenges faced by our community of patients. Some of these research efforts are highlighted below:

## Healthy Mind, Healthy You: A Dose Finding Study of Mindfulness

2017 saw the start-up of the NKN's participation in a project exploring how mindfulness practice can help reduce stress related to more than 100 different conditions, including Nephrotic Syndrome. Everyone who enrolls is randomly assigned to either three or eight sessions of an online mindfulness-based program. The results will help us learn which mindfulness program is best to manage stress and enhance wellness.

## Children's Hospital of Philadelphia Sleep Study

Children and their parents in the NKN registry were invited and have enrolled in this study that started in 2017. The study involves the development of a new pediatric sleep health survey. Participants are asked to complete a brief online survey. This project will help us better understand how sleep health relates to children diagnosed with Nephrotic Syndrome and kidney disease.

## Collaborating for Impact: The NephCure Kidney Network Adaptive Engagement Framework

The NKN team presented a poster at the 2017 Academy Health Annual Research Meeting describing the approach taken by the NKN to engage non-researchers (e.g., patients, family caregivers, patient advocates, doctors, and pharmaceutical representatives) in the development of a Nephrotic Syndrome research program. A manuscript is currently in development that will describe this engagement strategy in greater detail.

## Research Prioritization Work

In 2017, we began an initiative to understand what Nephrotic Syndrome research topics are most important to patients. You can help shape the future of NKN research by going to the NKN website and completing a brief survey to tell us about the kind of Nephrotic Syndrome research you would like to see in the future. Your responses will guide the research that we promote and conduct within the NKN.

# Meet our Team

## NKN Principal Investigators

Michelle Denburg, MD, MSCE
Lauren Lee
Lalita Subramanian, PhD, MPH

## NKN Clinical Investigators

Debbie S. Gipson, MD, MS
Michelle Hladunewich, MD, FRCP (C), M.Sc.
Alan Leichtman, MD
Laura Mariani, MD, MS

## NephCure Kidney International (NKI) Staff

Chelsey Fix, Registry Manager

## Arbor Research Collaborative for Health Staff

Rachel Tocco, Project Manager

## Steering Committee Members

Julie Abramson (co-chair)
Kathleen Broderick (co-chair)
Patrice Becker, MD
Kent Bressler
Michelle Denburg MD, MCSE
Chelsey Fix
Kimberly Flowers
Debbie Gipson, MD, MS
Lisa Holloman
Elaine Kamil, MD
Lauren Lee
Laura Mariani, MD, MS
Michael Mittelman
Elaine Sotherden
Suneel Udani, MD

# 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.

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## About PCORI

The **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.*

## About PCORnet

**PCORnet, 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.*

✉ registry@nephcure.org
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→ www.NephCureKidneyNetwork.org

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