

It Really Does Take a Village

NEPHCURE
KIDNEY NETWORK
PATIENT REGISTRY™

**13 Month
Data Snapshot 2017-2018**



NEPHCURE®
Kidney International

Saving Kidneys • Saving Lives

ABOUT NKN

The NephCure Kidney Network (NKN) 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

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

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RESEARCH PARTNERS & SUPPORT

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

In 2014, when NephCure Kidney International launched the NephCure Kidney Network (NKN) there was little happening in Nephrotic Syndrome research for treatment options. At the same time, we learned about the patient journey through limited, anecdotal accounts shared on our website or in person. Fast forward to 2018, when 1000+ NKN participants have helped us understand their stories through a more focused and data-driven lens. With increasing clarity, we have gained important insight into the preferences and motivations shared by patients. In parallel, there has been new research in the field. Today, there are more than 10 clinical trials actively recruiting patients for innovative treatments. Wonderfully, this dramatic change came about due to the collective efforts of multiple stakeholders including dedicated clinicians, focused researchers, collaborative networks, and empowered patients.

In response to the dramatic increase of clinical trial activity, NephCure must shift its organizational priorities. We are no longer focusing on collecting health information through surveys, but instead hyper focused on raising awareness of clinical trials and helping to connect patients to clinical trial sites. This change in the research landscape means that we have stopped recruitment into the NKN Patient Registry, making this the final data report for the NKN.

Although the decision to halt recruitment to the NKN was a difficult one, we recognize the significance of what's to come in the form of promising new therapies for Nephrotic Syndrome patients. As we move resources towards engaging patients and physicians in trial opportunities, we are steadfast in knowing this shift falls directly in line with NephCure's mission. Rest assured knowing that the registry data and survey

responses will be safely and securely stored for 3 years, during which time researchers interested in accessing de-identified data for research purposes may request to do so. Any researcher requests for the data will be evaluated by a committee at NephCure, and we will alert participants of any projects that come about from the data.

None of the achievements from the past four years would be possible without our partnership with Arbor Research Collaborative for Health. Arbor Research provided expertise in participatory methods, biostatistical analysis, and database management, all of which contributed to the success of the registry. We also had wonderful research partners from the Children's Hospital of Philadelphia, University of Michigan, and University of Toronto that made meaningful contributions to the project throughout the year.

Additionally, a volunteer-led Steering Committee comprised of patients, clinicians, researchers and industry representatives, worked tirelessly and collaboratively to help ensure the success of the NKN. The insights, advice, and counsel given by Steering Committee members was invaluable to the long life of the project.

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. By participating in the NKN, your voice was heard and research is moving forward.

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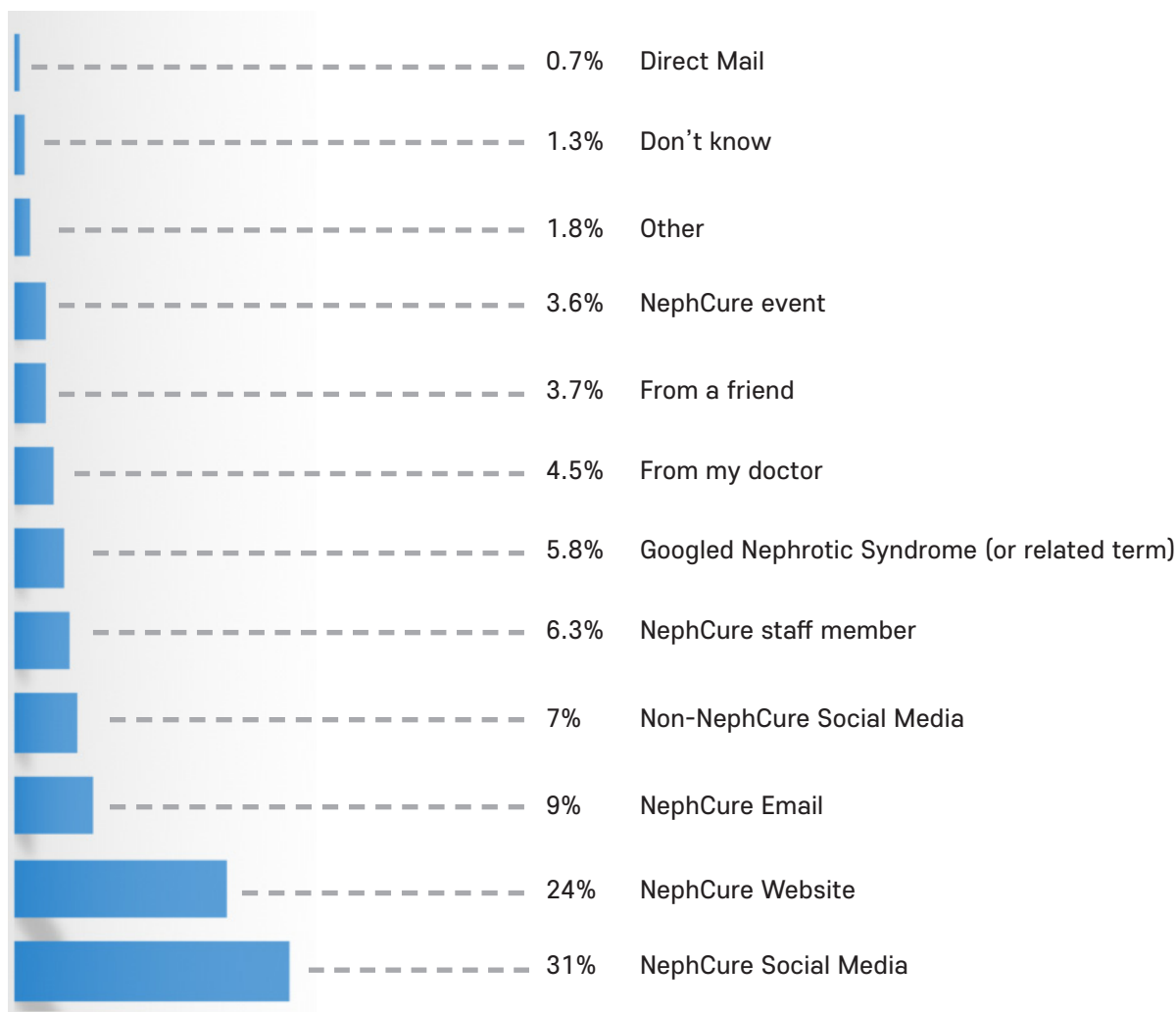
Lauren Lee

*NKN Co-Principal Investigator
Chief Research Officer
NephCure Kidney International*



RECRUITMENT

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

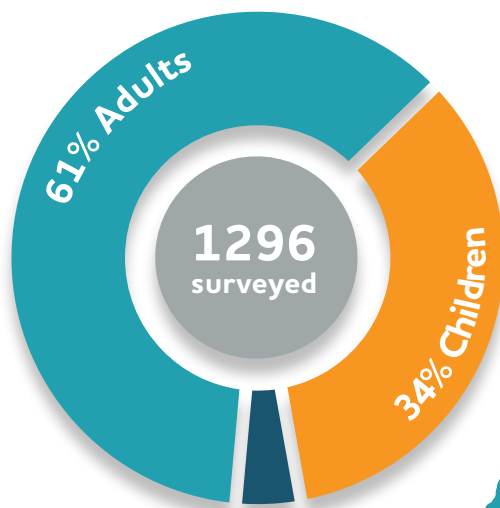


WALKER'S STORY: My son Walker was diagnosed in September 2016 at age 2. Over the first 9 months he was hospitalized 4 times for albumin and lasix treatments and once for a kidney biopsy. We were relieved with his MCD diagnosis. Walker quickly became steroid dependent. Not sure if y'all have ever dealt with a 2 year old on steroids, but it is no walk in the park! He relapsed every time we tried to wean him. After his 5th hospital stay in May 2017, we decided to try another treatment. It was a tough decision to make since Rituximab had not been given to very many kids Walker's age before.



AGE

Knowing the approximate age of Nephrotic Syndrome patients allows researchers to have a clear picture of the Nephrotic Syndrome community.



795 ADULTS

(18 and older)



446 CHILDREN

(younger than 18)

55 of the 1296

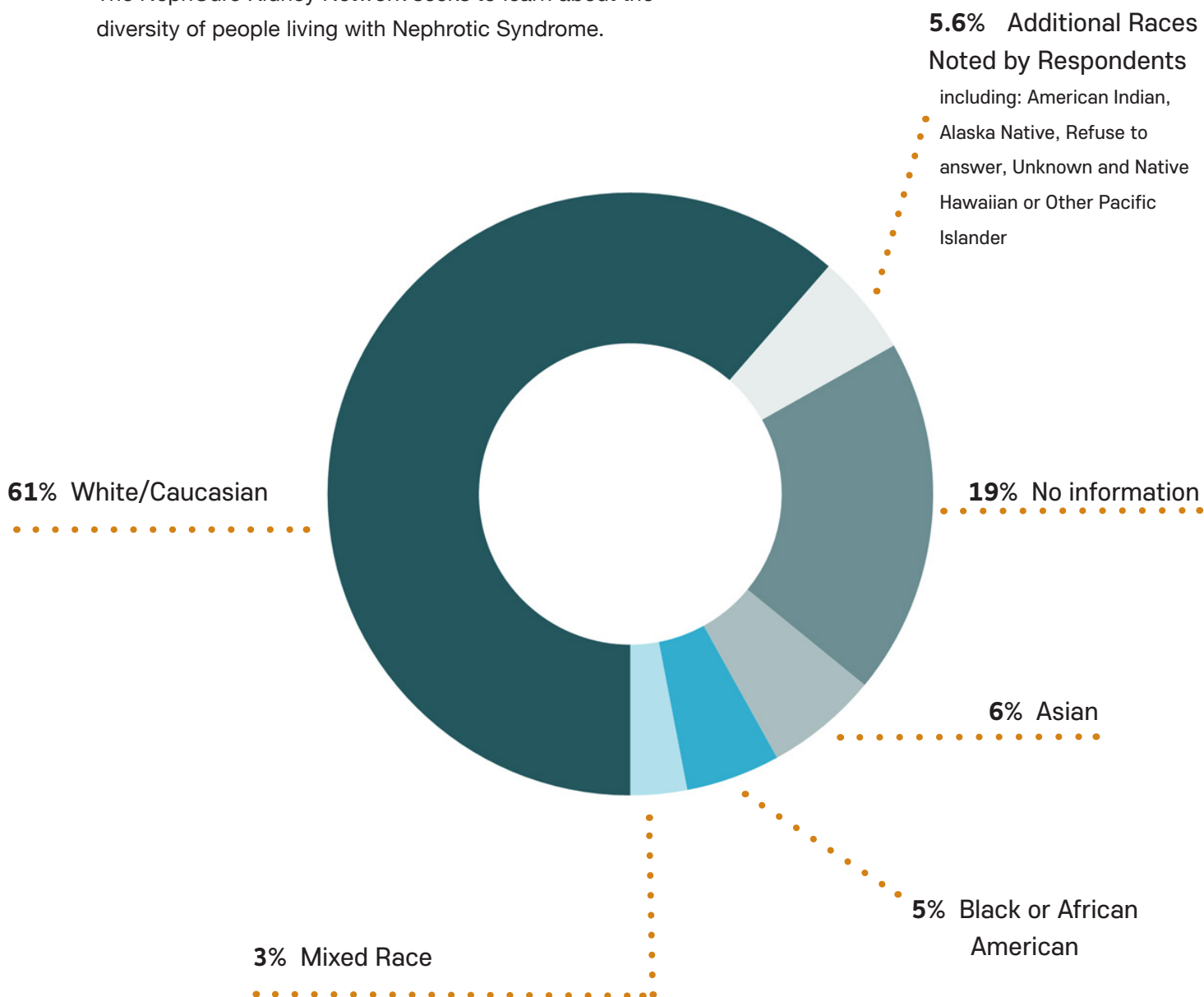
respondents did not indicate age group when completing survey

He had his first infusion in July 2017. He was hospitalized one more time because the treatment attacked his neutrophils and he couldn't fight infection. Luckily, his counts went up after a 5 day stay and he came home. We weaned him off the steroids and he DID NOT RELAPSE! It seemed too good to be true! And still does! Walker has been in full remission with no meds for 7 months! We pray every day that he stays healthy.

DIVERSITY

N = 1296

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



ZOE'S STORY: I was admitted to the hospital on Valentine's Day. I had 60-70lbs of water retention. I'm not sure why before all of this I thought I was dehydrated or just gaining weight rather rapidly. I do know I was scared. I didn't know what was going on I had never been admitted to the hospital before for an emergency. I spent the whole next day getting poked and prodded. After a biopsy and several blown veins, I had a diagnosis and a plan. I have Minimal Change Disease and now I am trying steroids and a handful of other prescriptions to combat the really negative side effects of the steroids. What I wasn't told was

FOOTPRINT

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

OTHER COUNTRIES REPRESENTED:

Philippines
Spain
Netherlands
Pakistan
Mexico
Ireland
Croatia
Brazil
Malaysia
Peru
New Zealand
Portugal
South Africa
Puerto Rico
Scotland
France
Afghanistan
Aland Islands
Argentina
Bulgaria

Bahamas
Bahrain
Trinidad & Tobago
Bangladesh
Bolivia
Dominican Republic
Ecuador
Finland
Chile
China
Colombia
Georgia
Germany
Greece
Hong Kong

Israel
Japan
Kenya
Korea, Rep. Of
Lithuania
Iraq
Turkey
Sweden
Switzerland
Viet Nam

Of the 1296 surveyed, 49 did not note country.

1074

UNITED STATES

28

CANADA

25

UNITED KINGDOM

15

AUSTRALIA

15

INDIA

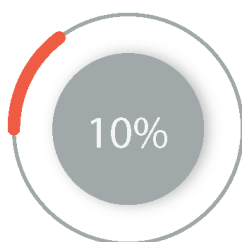
that adults have a 50/50 chance of a relapse. I haven't made it a month without a relapse. I am what they consider steroid dependent. I'm nervous about what the future holds for me. I try to be positive and take it day by day. Worrying only means I suffer twice.

Be your own advocate. Ask questions. Write them down if you think you're going to forget. Do not be afraid to ask those questions. Sometimes knowing why helps. Talk to someone about what's going on. Ask for help on the days that fatigue is going to kick your butt. Go easy on yourself. There's only one you.

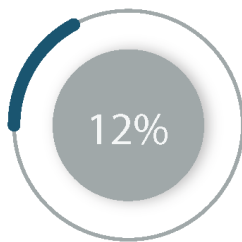
DIAGNOSIS

What is the patient's kidney disease diagnosis?

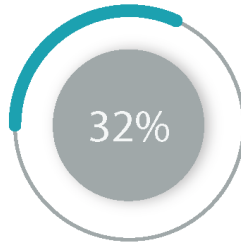
ADULT RESPONDERS (18 AND OLDER)



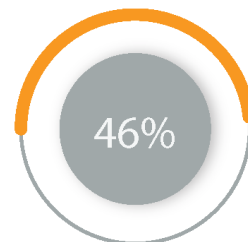
MCD
Minimal
Change Disease



NS
Nephrotic
Syndrome



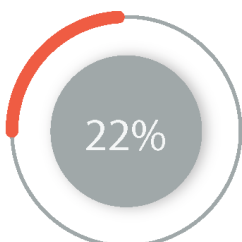
Other
Kidney
Diagnoses



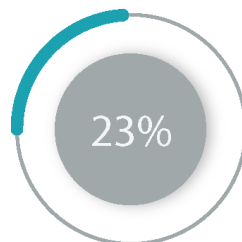
FSGS
Focal Segmental
Glomerulosclerosis

N = 850

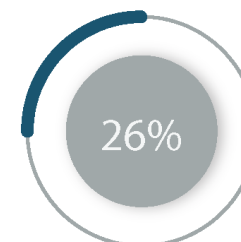
PEDIATRIC RESPONDERS (UNDER 18 YEARS OF AGE)



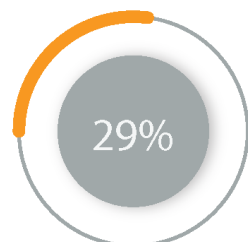
MCD
Minimal
Change Disease



Other
Kidney
Diagnoses



NS
Nephrotic
Syndrome



FSGS
Focal Segmental
Glomerulosclerosis

N = 583

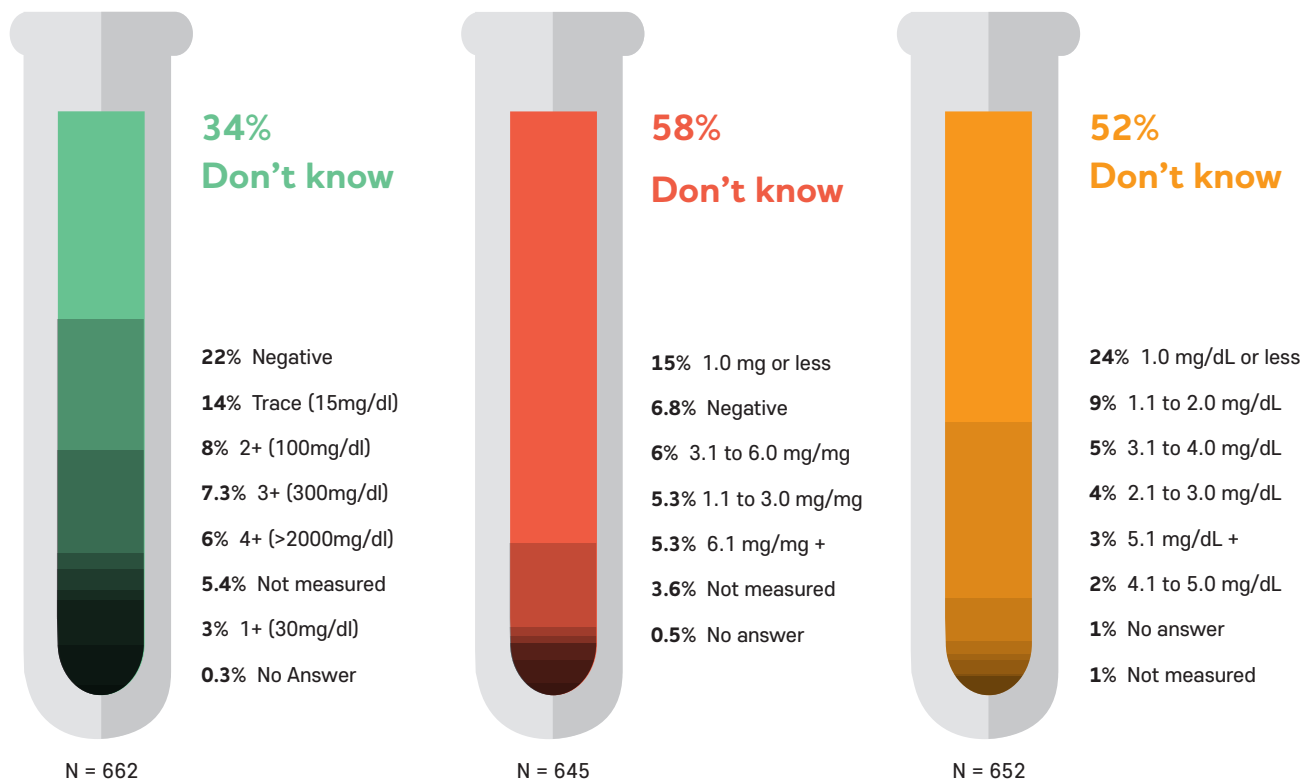


HEATHER'S STORY: After a slow progression of FSGS over the last 20 years, my kidneys finally were giving up. After many tests I was put on the transplant list a year and four months ago, but I didn't have to wait long... My husband Todd went in to get tested to see if he was a match. After many tests and a few months later he got the call... He was my person. Jan 9, 2017 Todd gave me the gift of life. The most beautiful gift a human being can give. We are both doing amazing over a year later and so far no recurrence of FSGS in my new kidney.

LAB VALUES

Many NKN participants responded “Don’t Know” to questions about lab values. It is the goal of NephCure Kidney International to help educate patients and families about the importance of tracking lab values.

- What is the patient’s most recent urine dipstick for urinary protein reading?
- What is the patient’s most recent urine protein to urine creatinine ratio (P/C ratio)?
- What is the patient’s most recent serum creatinine?



I can now see my son graduate from high school this spring, see him go off to college, see him get married someday... And I get to do all of these things with my kidney donor by my side. It doesn't get more beautiful than that! Never give up, fight! You will have bad days but you will also have good days.

Share your story, you may just inspire someone someday. Get involved with your disease... Join walks, join a kidney Facebook page! I have met some amazing people fighting the same fight as me, they have helped me more than they will ever know.

TREATMENT

Since diagnosis, has the patient taken any immunosuppressant medications?

N = 708



92% of 708 respondents have used an immunosuppressant since diagnosis.

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

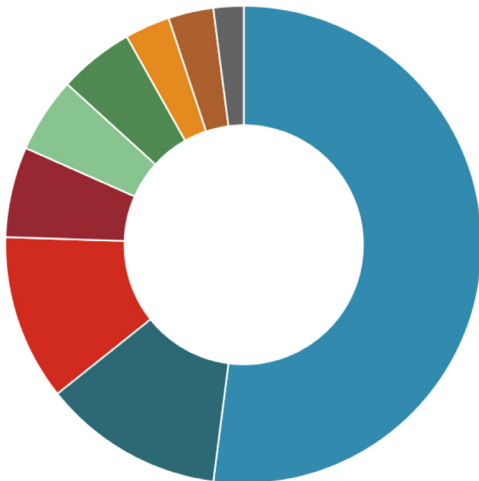
N = 701



80% of 701 respondents told us that they took some sort of blood pressure medication since their diagnosis.

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

N = 789

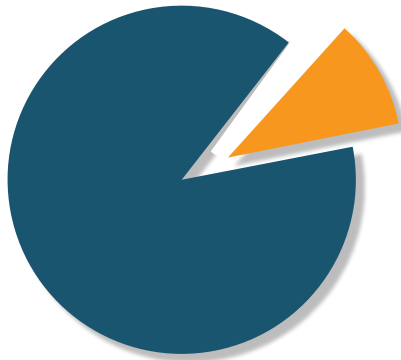


- **51% No**
- 12% Vitamin therapy
- 11% Therapeutic massage
- 6% Homeopathy
- 5% Acupuncture
- 5% Yoga
- 3% Chiropractic treatments
- 3% Osteopathic manipulative medicine (OMM)
- 2% Reiki

JAMIE'S STORY: In June of 2014, Jamie had what seemed like a typical childhood cold. A week later Jamie was not improving and we ended up in the emergency room with a diagnosis of pneumonia... The doctors began to become concerned with her kidneys, but didn't quite know what was happening. We were transferred to Montefiore Children's Hospital in the Bronx, where we were told they had pediatric nephrologists. I will always remember that middle of the night ambulance ride in the rain and being wheeled into an unfamiliar place lying on a stretcher holding my baby, unsure of our future. In the week that followed we learned more than we thought possible. Fortunately, Jamie responded well to IV lasix, albumin, and steroids. Jamie was discharged from the hospital and sent home just in time to celebrate her 4th birthday. She remained on high dose steroids for several months....Fast forward to 2018,

KIDNEY TRANSPLANT

Has the patient ever had a kidney transplant?



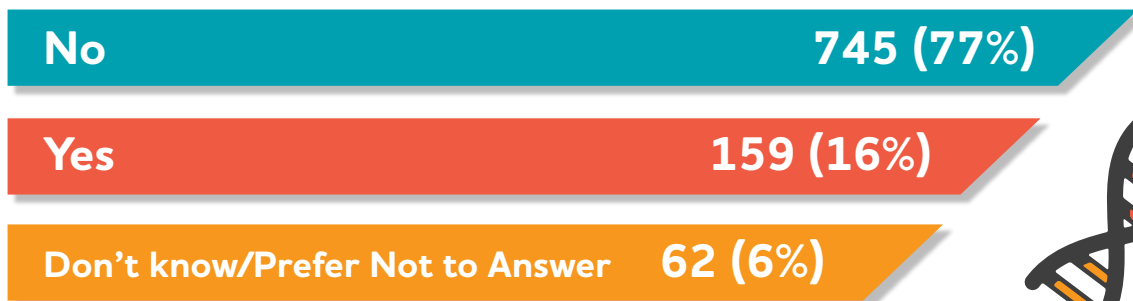
N = 972

Types of Kidney Transplant recieved:

Living unrelated	27
Living	26
Living related	22
Deceased Donor	19

N= 94

Did the patient have genetic testing?



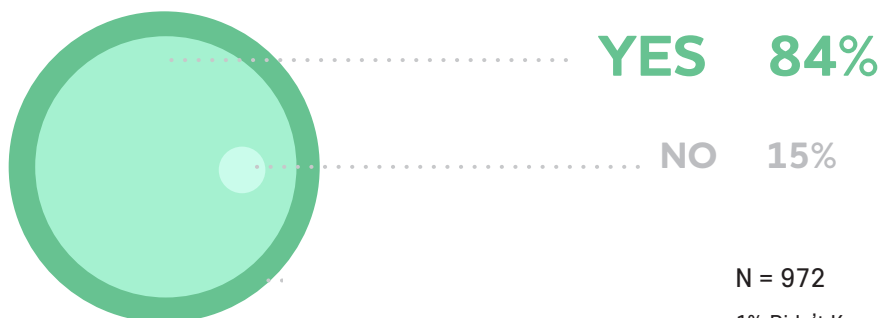
N = 966



Jamie is a happy and healthy second grader. She is an excellent student and an avid reader. She's an aggressive basketball player and recently won the second grade spelling bee. She has nephrotic syndrome and food allergies, but we do not let them define her. Over the past 4 years we have suffered with relapses. We have had plenty of ups and downs. There have been days when I didn't think I could handle another relapse... But guess what? We got through it and I know we will continue to get through any obstacle thrown our way, one day at a time because if this unpredictable disease has taught me anything, it's to cherish every moment we have. Knowledge is power. Take one day at a time. The sun will come out tomorrow!

DIALYSIS

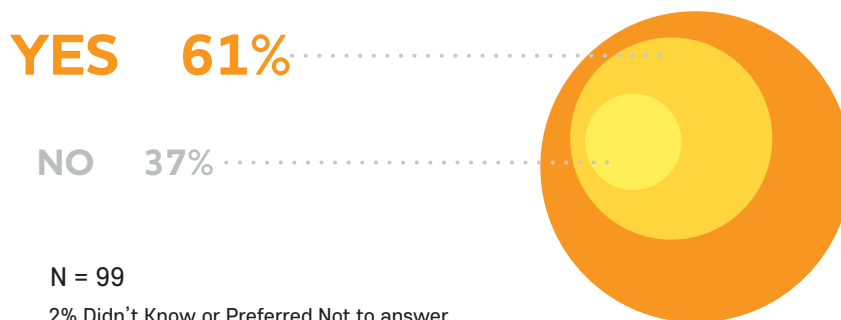
Has the patient ever had dialysis?



N = 972

1% Didn't Know or Preferred Not to answer

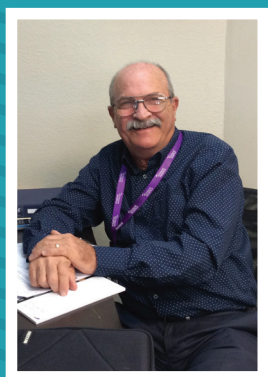
Is the patient currently on dialysis?



N = 99

2% Didn't Know or Preferred Not to answer

At what age did the patient start dialysis? (average age) **28 years old**

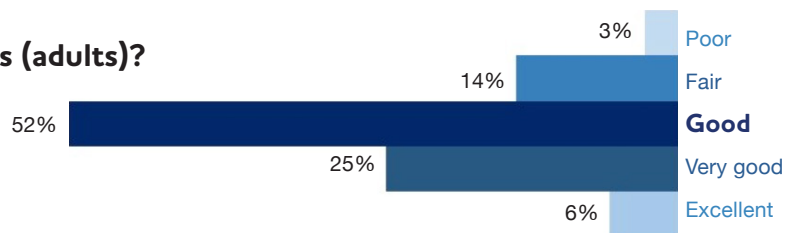


KENT's STORY: My brother gave me one of his kidneys almost 31 years ago. We have both done very well. I have spent a life time taking care of myself and in turn have been able to keep my kidney without rejection. I have been able to stay married to the same sweet person for 48 years. I have actively searched for the reason FSGS is so devastating and have been part of peer review projects reviewing research funding projects that target research in to finding a cure for FSGS.

QUALITY OF LIFE

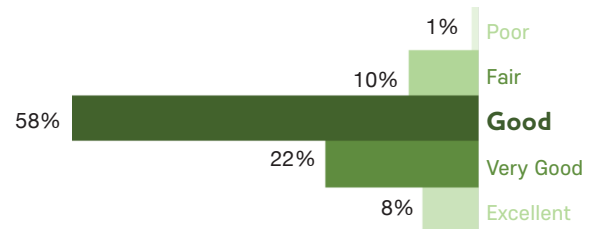
In general, how would you say your health is (adults)?

N = 751



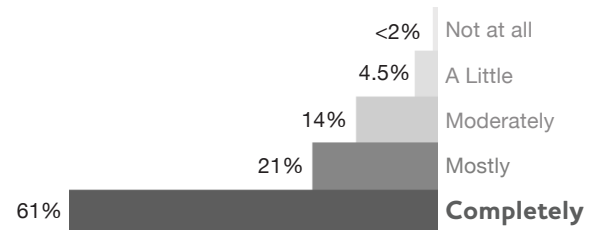
In general, how would you rate your mental health including your mood and your ability to think?

N = 460



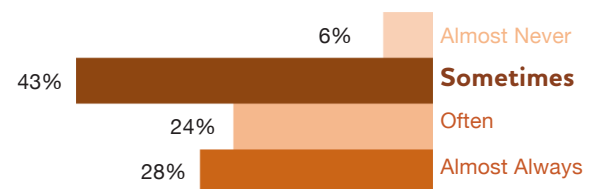
To what extent are you able to carry out your everyday physical activities*? (adults)

N = 464



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

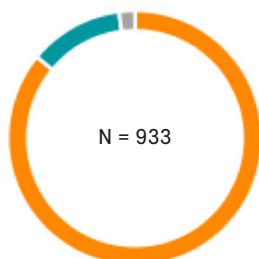
N = 108



* such as walking, climbing stairs, carrying groceries, or moving a chair

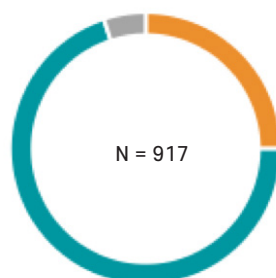
Best advice I could give is to invest in your own health. Know everything there is to know about your illness. Follow your doctor's advice, eat right exercise never miss a dose of medicine, ask questions and most of all pray for yourself and others. As with all things give thanks.

RESEARCH PARTICIPATION



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

86% Said NO



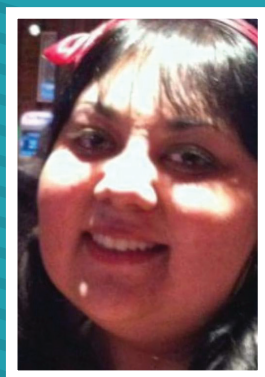
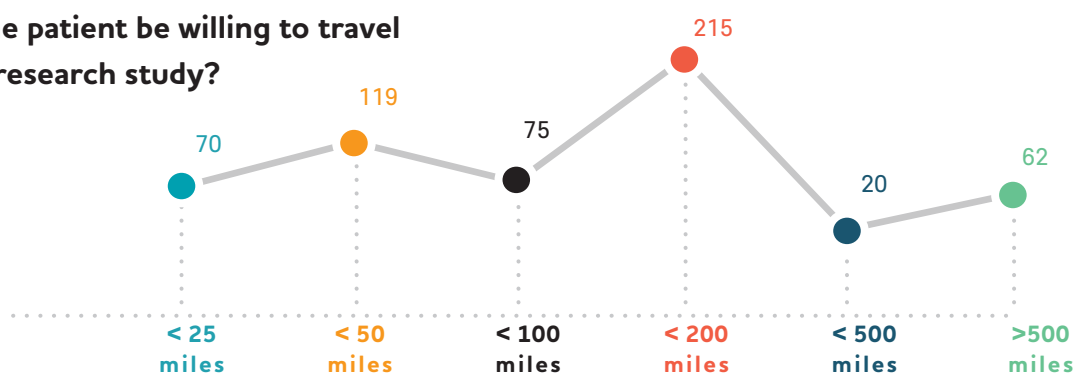
Would the patient be interested in participating in a research study about his/her kidney disease that would test an experimental therapy that may or may not help him/her?

70% Said YES

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

89 of the 650 responses were "Don't Know" or "Prefer Not to Answer"

N = 650



TRISTA'S STORY: Through NephCure I have met some amazing humans that know and understand what living with NS is like. And beyond that, they are advocates in finding a cure and better treatments for all of us living with NS. NS and the various rare CKDs that come along with it can be very isolating and devastatingly redundant. Being involved with NephCure and research has made things easier to cope with. As patients we can be involved and be advocates for ourselves and others. By spreading awareness, showing up and raising money for walks and other events, and being involved

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?)

■ Not Interested
 ■ Somewhat Interested
 ■ Very Interested

An Insurance Company



Medical School or Hospital



A Non-profit Foundation (like NephCure)



The Patient's Own Doctor



A Pharmaceutical Company



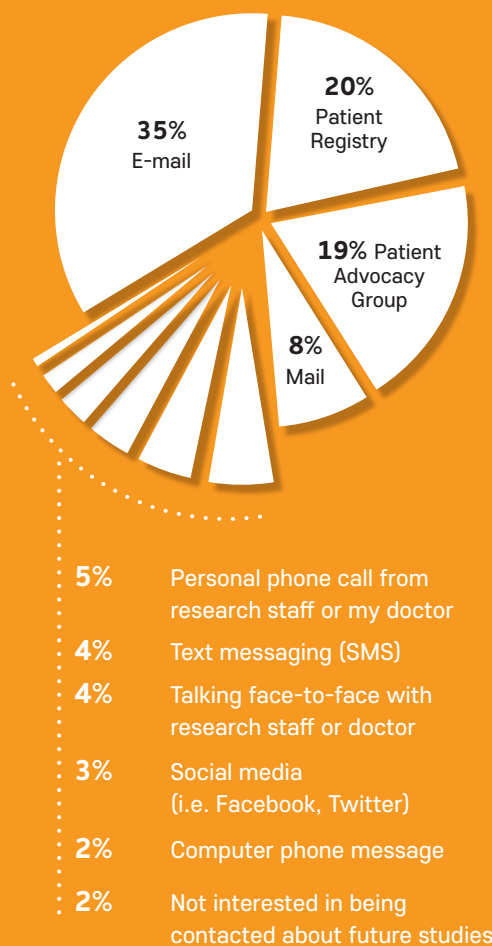
A Private Company (i.e retail, food, etc.)



The US Government



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

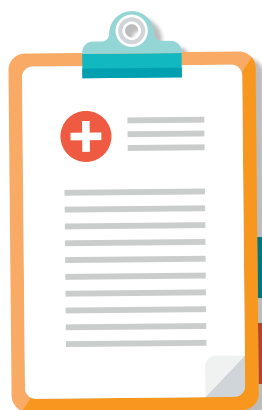


N = 1720 (multiple responses permitted)

in research (maybe that's being part of a trial or just answering some questions for a survey) we can make a difference and further the progress of more efficient treatments and a cure. This disease is awful but I've been introduced to some amazing people because of it and whether I'm having a good day or not it definitely keeps me going knowing that I am involved in the fight against it. Be your own advocate! Don't be afraid to ask questions to doctors and medical professionals! Help us in saving kidneys and saving lives! You are not alone, every individual story is unique and sometimes it just plain sucks but you are not alone in this journey, reach out.



HEALTHCARE



Does the patient have health insurance or health care coverage?

YES

819

NO

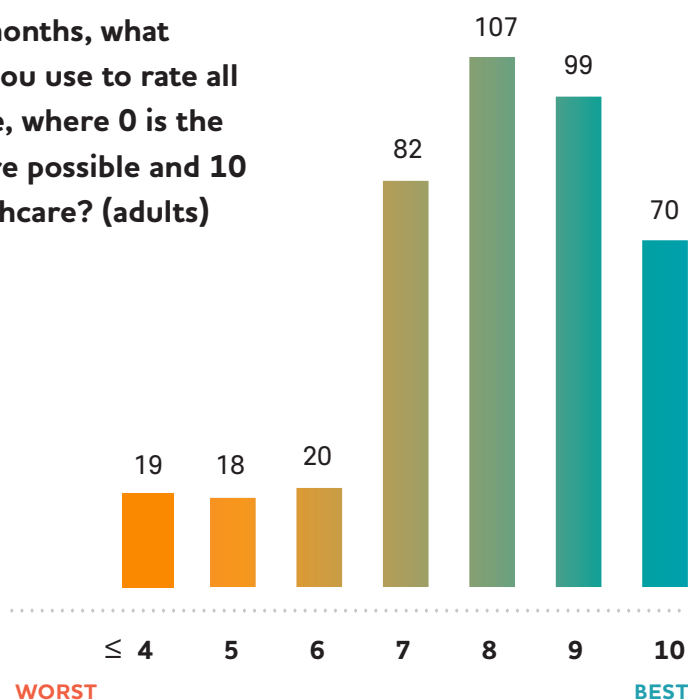
42

5 Don't Know/Prefer Not to Answer

N = 866



In 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)



N = 415

LOOKING TOWARDS THE FUTURE

Collectively, your stories and data in the NKN have been a driving force in moving the field to where it is today. With over 10 clinical trials actively recruiting for rare kidney diseases like FSGS, Minimal Change Disease, and IgA Nephropathy, we now turn our attention to spreading the word and connecting patients to these clinical trial opportunities. Below is a list of Frequently Asked Questions (FAQs) taken directly from patients and families interested in learning more about research and how to be involved in clinical trials that are evaluating potential new treatment options.



What are the benefits of participating in a clinical trial?

By participating in a clinical trial, you not only gain access to cutting-edge & expert care, but you are participating in a potential medical breakthrough that could help many more people in the future. During treatment, you will be monitored closely by top physicians and researchers. Should the medical treatment being tested in the clinical trial be effective, you will be one of the first people to reap the benefits before it is released to the public.



Who can I talk to if I want to learn more about clinical trials?

You can always talk to the doctor treating your kidney disease or the principal investigator of the study to learn more information about whether you qualify for a clinical trial, or what the requirements may be for a specific trial. You can find information about clinical trial locations and contacts by visiting NephCure.org/Research



What happens when the study is over?

After the study is completed, the data is then collected, analyzed, and shared with experts and several government agencies for the drug approval process. Participants can continue to take the medication, should it help, but they are not required to continue with any other phases of the trials. Each clinical trial has a different set of rules about what happens after the trial is over, so be sure to talk to your research team.



OUR TEAM

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FUNDING

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. An award from the Patient-Centered Outcomes Research Institute (PCORI) helped to fund the expansion of the NKN (2014-2018) and its inclusion in PCORnet.

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 pcori.org.

About PCORnet

PCORnet, the National Patient-Centered Clinical Research Network, is a groundbreaking initiative of 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 pcornet.org.



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