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
# TABLE OF CONTENTS

<table>
<thead>
<tr>
<th>Section</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>Letter from NKI Research Director</td>
<td>3</td>
</tr>
<tr>
<td>What is the NephCure Kidney Network?</td>
<td>4</td>
</tr>
<tr>
<td>Research Partners &amp; Support</td>
<td>5</td>
</tr>
<tr>
<td>Data Overview</td>
<td></td>
</tr>
<tr>
<td>Recruitment</td>
<td>6</td>
</tr>
<tr>
<td>Demographics</td>
<td>7</td>
</tr>
<tr>
<td>Diagnosis</td>
<td>8</td>
</tr>
<tr>
<td>Laboratory Values</td>
<td>10</td>
</tr>
<tr>
<td>Treatments</td>
<td>11</td>
</tr>
<tr>
<td>Family History</td>
<td>13</td>
</tr>
<tr>
<td>Patient Reported Outcomes</td>
<td>14</td>
</tr>
<tr>
<td>Research</td>
<td>15</td>
</tr>
<tr>
<td>Feedback Survey</td>
<td>16</td>
</tr>
<tr>
<td>Thank You!</td>
<td>17</td>
</tr>
<tr>
<td>Meet Our Team</td>
<td>18</td>
</tr>
</tbody>
</table>
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

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Operations

NKN Steering Committee

Patient Engagement

Community Engagement

Data Collection

Workgroups

PCORnet

Arbor Research

NKI

NS Patient Community

Research Consortia & Professional Societies

NephCure Scientific Advisory Board
# Recruitment

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

### New Participants by Month

<table>
<thead>
<tr>
<th>Month</th>
<th>Participants</th>
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<tbody>
<tr>
<td>Mar-14</td>
<td>49</td>
</tr>
<tr>
<td>Apr-14</td>
<td>35</td>
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<td>May-14</td>
<td>43</td>
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<tr>
<td>Jun-14</td>
<td>36</td>
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<td>Jul-14</td>
<td>30</td>
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<td>Aug-14</td>
<td>25</td>
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<td>Sep-14</td>
<td>50</td>
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<td>Oct-14</td>
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<td>Nov-14</td>
<td>19</td>
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<td>Dec-14</td>
<td>14</td>
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<td>Jan-15</td>
<td>13</td>
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<tr>
<td>Feb-15</td>
<td>12</td>
</tr>
<tr>
<td>Mar-15</td>
<td>26</td>
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Recruitment efforts for the NKN included:

- Emails and mailings to all patients in NKI’s contact database
- 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.

<table>
<thead>
<tr>
<th>Demographic</th>
<th>Percentage</th>
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<tbody>
<tr>
<td>American Indian/Alaska Native</td>
<td>1%</td>
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<tr>
<td>Asian</td>
<td>7%</td>
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<tr>
<td>Black/African American</td>
<td>6%</td>
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<tr>
<td>Hispanic or Latino</td>
<td>10%</td>
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<tr>
<td>Native Hawaiian/Pacific Islander</td>
<td>0%</td>
</tr>
<tr>
<td>White</td>
<td>73%</td>
</tr>
<tr>
<td>Multiple, Other, Unknown</td>
<td>13%</td>
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</tbody>
</table>

Out of 352 responses

Number of Pediatric and Adult Participants by Gender

- Male, 96
- Female, 73
- Male, 73
- Female, 116

Out of 358 responses

Participants from 26 countries have enrolled in the NKN

82% of enrollees are from the United States
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.

**Out of 348 results**

- Membranoproliferative Glomerulonephritis (MPGN) 2%
- Membranous Nephropathy (MN) 2%
- IgA Nephropathy 2%
- Minimal Change Disease (MCD) 23%
- Nephrotic Syndrome (NS) 12%
- Other 4%
- Don't know 1%

**Other diagnoses include:**
- C1q Nephropathy
- C3 Nephropathy
- Diffuse Mesangial Sclerosis
- Fibrillary Glomerulonephritis
- Fibronectin Glomerulopathy
- IgM Nephropathy
“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.
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.

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 (Adrenocorticotropic hormone, ACTH)
- Cytoxan (Cyclophosphamide)
- Imuran (Azathioprine)
- Plasmapheresis (plasma exchange)

“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.
Out of 260 responses

TREATMENTS (ALTERNATIVE THERAPIES)

Do you use Alternative Treatment Methods?

No 71%

Yes 29%

Alternative Treatments used by Patients

“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

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

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)?

- Blood in the urine: 21
- High blood pressure: 166
- Kidney disease of any type: 34
- Kidney failure requiring dialysis or kidney transplant: 23
- Protein in the urine: 23
- None: 72
- Don’t know: 17

Out of 355 responses

Patients can select multiple responses

Which family members are also known to have the patient’s rare disease?

- Paternal Cousin: 1
- Paternal Grandfather: 2
- Paternal Grandmother: 2
- Paternal Uncle: 2
- Paternal Aunt: 1
- Maternal Cousin: 6
- Maternal Grandfather: 1
- Maternal Grandmother: 1
- Maternal Uncle: 2
- Maternal Aunt: 1
- Brother: 7
- Sister: 1
- Father: 3
- Mother: 6
- Son: 3
- Daughter: 2

Out of 355 responses

Patients can select multiple responses

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

- 2 Weeks Preterm: 2
- 10 Weeks Preterm: 1
- 12 Weeks Preterm: 5
- 6 Weeks Preterm: 5
- 5 Weeks Preterm: 5
- 4 Weeks Preterm: 11
- 3 Weeks Preterm: 10
- 2 Weeks Preterm: 22
- 1 Week Preterm: 17
- On Time: 87
- 1 Week Late: 30
- 2 Weeks Late: 24

Out of 219 responses
PATIENT REPORTED OUTCOMES (PROs)

In general, how is the patient’s health?
- Excellent 9%
- Very Good 33%
- Good 37%
- Fair 17%
- Poor 4%

Out of 276 responses

Does the patient’s health now limit him/her in doing vigorous activities?
- Cannot do 2%
- Quite a lot 12%
- Somewhat 36%
- Very little 30%
- Not at all 20%

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?
- Never 4%
- Rarely 17%
- Sometimes 38%
- Often 30%
- Always 11%

Out of 277 responses

How often does the patient feel depressed?
- Never 25%
- Rarely 33%
- Sometimes 31%
- Often 10%
- Always 1%

Out of 277 responses
Is the patient willing to be contacted in the future about research related to their rare disease?

Out of 349 responses

Don’t know, 2%
No, 2%
Yes, 95%

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?

Out of 362 responses

Yes, 11%
Don’t Know, 2%
No, 87%

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

Don’t Know, 4%
Yes, 29%
No, 67%

“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.
“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

Work Groups:

Community Engagement Group

Patient Engagement Group

Data Collection Group

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
STAND UP & BE COUNTED!
THE NEPHCURE KIDNEY NETWORK