



**NephCure Kidney International
The Gateway Initiative**

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**Clinical Trial Collaborative
Advisory Committee**

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Cindy Roy, PhD, National Institutes of Health and National Institute of Diabetes and Digestive and Kidney Diseases
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Joshua Tarnoff, NephCure Chief Executive Officer

Lauren Lee, NephCure Chief Research Officer

Bonnie Schneider, IGA Nephropathy Foundation of America Director and NephCure Vice President of IgAN Affairs

Lauren Eva, NephCure Director, Marketing & Communications

Emma Palitz, NephCure Coordinator, Research & Engagement Marketing

Dear Clinical Trial Recruitment Collaborators,

It's been a busy month, and we have a lot of great updates to share with you. Since the November 15th meeting in Washington, DC, all 6 of the workgroups have been meeting monthly, gaining significant further insights and refining their deliverables, in addition to identifying and/or completing 1-2 game-changing outputs. The workgroups are now scoping out program details, e.g. cost & human resources, timing, feasibility, as well as potential impact, for these as well as many new and very impactful deliverables still to come. Stay tuned for more to be announced in the next newsletter.

Below, I'm thrilled to share an editorial written by patient parents who attended the November 15th meeting. Please read and know how much your work matters.
- Lauren Lee, Chief Research Officer, NephCure Kidney International

**Brighter days ahead for Nephrotic Syndrome patients:
The Clinical Trial Revolution is here**

Three years ago, our adult son was diagnosed with Focal Segmental Glomerulosclerosis (FSGS). This was an extremely difficult time for him and our entire family. As healthcare professionals ourselves, we combed through the medical literature and turned to our colleagues for answers. To our surprise, all we found were a few generic treatments for the condition, nothing disease-specific, based on clinical research that had appallingly low numbers of participants. There was only one clinical trial in progress and no glimmer of better treatments or cures in our future.

Through our searching, we found NephCure Kidney International and quickly attended a local outreach meeting. We were heartened by what we discovered: enthusiastic staff from the organization, a local researcher focused on FSGS, and other patients and families who were facing the same challenges. While we had not found a solution, we had found a community, and that gave us a great deal of comfort and encouragement.

Fast forward to 2018 when an amazing shift occurred—the arrival of 16+ clinical trials testing potential new treatment options for FSGS, IgA Nephropathy and other rare kidney conditions. Finally, the dream of more effective medicines with

less harmful side effects became a closer reality for our son.

While this is incredibly encouraging news, it also presents significant challenges. Getting new treatments to the pharmacy shelf relies on willing patients to be part of clinical trials. History shows that filling trials for any disease is difficult, including those affecting millions of people such as cancer and heart disease. In the case of Nephrotic Syndrome, a rare condition, finding patient volunteers to participate in clinical trials is doubly hard. This is critical. **Only last week when our son applied to participate in a major trial he learned it had just been closed due to lack of participants.** Opportunity lost.

It was both the threat of unsuccessful trials and the promise of new and better medicines that prompted NephCure, together with members of the US Food and Drug Administration, the American Society of Nephrology, the Kidney Health Initiative, and the National Institutes of Health, to jointly develop the Clinical Trial Recruitment Collaborative meeting in Washington, DC on November 15, 2018. We were fortunate to have attended the 'all-hands' meeting where we witnessed firsthand the passionate and dedicated group of individuals laser-focused on filling clinical trials for Nephrotic Syndrome. It was encouraging and exciting to be in a room filled with 100 of the leading international researchers; clinicians; pathologists; staff members from the FDA, the NIH, and the ASN; representatives of pharmaceutical companies; patients; and family members coming together to find solutions to ensure these trials will not go unfilled. The sheer number of highly committed people in the room who truly cared about Nephrotic Syndrome treatments was inspiring.

With a clear understanding of the once in a lifetime opportunity unfolding before us, we left the meeting with a renewed sense of purpose and strong desire to spread the word to other patients and family members:

The time is now—with 16+ clinical trials in progress for Nephrotic Syndrome, 9 of which are specifically for FSGS, this day in time holds great promise for patients. It is imperative that all patients and families affected by these rare conditions heed the call to action by standing ready to be part of the clinical trial revolution.

Equally important, the Gateway Initiative has become a central hub for information, entry to clinical trials, and expert opinion resources. We encourage you to join this global movement by visiting KidneyHealthGateway.com, a user-friendly website created to help patients easily find the best clinical trial for their health condition. By taking this important step, you are not only finding trials right for you or your loved one—but also doing your part to move research forward and make better treatments a reality. In addition, NephCure has announced a strategic partnership with the IgA Nephropathy Foundation of America to combine resources and ensure that no potential new treatment fails due to lack of patient volunteers.

We hope to see better treatments and improved outcomes for our son and others like him, and one day, a cure. These once lofty goals may finally be on the horizon. We are grateful for the many dedicated individuals and organizations that are working to make this happen.

Sincerely,







David and Stephanie

Patient Parents






Clinical Trial Recruitment Collaborative: Workgroup Updates

KEY:  COMPLETE  IN PROGRESS  NOT YET STARTED  ON HOLD




CLINICIANS WORKGROUP

-  Develop a clinical trial curriculum, speakers, and featured trials to be presented on GlomCon, summer 2019
-  Partner with ASN to expand GlomCon series reach **NEW!**
-  Increase social media presence to engage clinicians with research, CME series, and current trials **NEW!**
-  Create clinicians' perspectives piece on importance of clinical trials in nephrology (GN especially) **NEW!**
-  Partner with international groups to reach global audience **NEW!**
-  Work with NephCure to develop and market clinician-focused section of KidneyHealthGateway.com






CONSORTIA WORKGROUP

-  Work with relevant Patient Advisory Boards to collect feedback on KidneyHealthGateway.com
-  Alert patients of KidneyHealthGateway.com trial finder tool to increase interventional trial enrollment **NEW!**
-  Select locations for regional trial-readiness meetings, to be hosted by NephCure
-  Create shared global library that summarizes available resources of all consortia/networks
-  Raise awareness of resource library through social media, emails, and newsletters for patients and clinicians **NEW!**





PATHOLOGY WORKGROUP

-  Publish editorial that outlines pathologists' role in identifying eligible patients for clinical trials
-  Collaborate with the Renal Pathology Society Workgroup to provide information about all ongoing clinical trials
-  Create a centralized renal pathology consultant group to assist in trial protocol development and refinement

STUDY OVERLAP WORKGROUP

-  Create a resource to highlight differences of each ongoing clinical trial for researchers
-  Develop centralized screen failure registry across NS trials to retain trial-ready patients **NEW!**
-  Survey stakeholders to gauge interest in master protocol for FSGS
-  Create a best practices list for engaging patients/families in trial design
-  Explore telehealth solutions through case studies

PEDIATRIC INCLUSION WORKGROUP

-  Survey industry perspective of pediatric inclusion in trials
-  Collaborate with government agencies to develop pediatric risk/benefit analysis for various forms of NS to include pediatric patients in Phase 2 + 3 studies **NEW!**
-  Develop strategies to address patient/family barriers to trial participation
-  Finalize and publish white paper addressing regulatory pathways for pediatric inclusion in rare disease clinical trials

EX-US INCLUSION WORKGROUP

- ✓ Meet with Can-SOLVE CKD Network
- Create a rolodex/directory of key international contacts/resources
- Dedicate an upcoming World Kidney Day to FSGS
- Attend current international meetings to showcase the trial landscape & Gateway Initiative
- Collaborate with NephCure to make KidneyHealthGateway.com international-friendly

Workgroup Members

Clinicians

Kirk Campbell (Mt. Sinai)
Suneel Udani (Nephrology Associates of Northern Illinois and Indiana)
Ahmed Awad (Kansas City Research Associates)
Martin Bunke (Retrophin)
Michelle Denburg (Children's Hospital of Philadelphia)
Kara Jones (Patient Parent)
Radko Komers (Retrophin)
Daniel Levy (Pfizer)
David & Stephanie (Patient Parents)
Ali Poyan Mehr (Kaiser Permanente)
Jose Perez (Baylor)
Michelle Rheault (Univ of Minnesota)
Nick Robertson (Apellis)
John Sedor (Cleveland Clinic)
Karen Townsend (IQVIA)
Liron Walsh (Goldfinch)

Consortia

Matthias Kretzler (Univ of Michigan)
Moin Saleem (Univ of Bristol)
Meaghan Allain (KHI)
Ryan Bucco (Retrophin)
Lawrence Holzman (Univ of Pennsylvania)
Mark & Becka Levondosky (Patient Parents)
Tina Mainieri (NEPTUNE)
Laura Mariani (Univ of Michigan)
Afshin Parsa (NIH)
Noah Rosenberg (Retrophin)
Cindy Roy (NIH)
William Smoyer (MWPNC)

Pathology

Laura Barisoni (Duke)
Patrick Walker (Arkana Labs)
Rana Dutta (Retrophin)
Brad Farris (Emory)
Joseph Gaut (Washington Univ St Louis)
Stephen Hewitt (National Cancer Institute)
Jeff Hodgkin (Univ of Michigan)
Jeffrey Kopp (NIH/NephCure SAB)
Martin Pollak (Beth Israel/Harvard/NephCure SAB)
Noah Rosenberg (Retrophin)
Brad Rovin (The Ohio State Univ)
Steve Shuey (Retrophin)
Robert Star (NIH)
John Tomaszewski (SUNY Buffalo)

Study Overlap

Barbara Gillespie (Covance)
Meg Jardine (The George Institute)
Sharon Adler (Harbor UCLA)
Geoffrey Block (Reata)
Kathleen Broderick (Patient Parent/NephCure BOD)
Claude Galphin (Nephrology Associates of Tennessee)
Vivek Jha (The George Institute)
Patrick Nachman (Univ of Minnesota)
Roberto Pecoits (The George Institute)
Ali Poyan Mehr (Kaiser Permanente)
Noah Rosenberg (Retrophin)
Angie Schinkel (Retrophin)
Kimberly Smith (FDA)
Chrissy St. Jean (Patient Parent)

Pediatric Inclusion

Debbie S. Gipson (Univ of Michigan)
Howard Trachtman (NYU)
Meaghan Allain (KHI)
Pam Baldyga (Patient Parent)
Melanie Bhatnagar (FDA)
Pam Duquette (Patient Parent)
David Feldman (NKF)
Kevin Fowler (Retrophin)
Keisha Gibson (UNC)
Cindy Jackson (Covance)
Elaine Kamil (Cedars Sinai/NephCure BOD)
Rick Kaskel (Einstein/Montefiore)
Mona Khurana (FDA)
Radko Komers (Retrophin)
Alyssa Laesser (Patient Parent)
Susan Massengill (Levine Children's)
Jun Oh (Univ of Hamburg)
Michelle Rheault (Univ of Minnesota)
Susan Samuel (Univ of Calgary)
William Schnaper (Lurie Children's)
Donna Snyder (FDA)
Chrissy St. Jean (Patient Parent)
Norman Stockbridge (FDA)
Perdita Taylor-Zapata (NIH – NICHD)
Nicholas Webb (Novartis)

Ex-US Inclusion

Tobias Huber (Univ of Hamburg)
Stuart Shankland (Univ of Washington)
Jonathan Barratt (Univ of Leicester)
Alessia Fornoni (Univ of Miami)
Tracey Gross (Patient)
Jula Inrig (IQVIA)
Alex Mercer (Retrophin)
Jun Oh (Univ of Hamburg)
Heather Reich (Univ of Toronto)
Jochen Reiser (Rush University)
Joshua J. Zaritsky (AI DuPont/Nemours)

Surveys and Resources: Please Review and/or Complete

- [Ex-US Directory of Experts](#)
- [Printable list of all glomerular disease trials](#)
- [GlomCon Conference Information](#)
- **For industry partners:**
- [Pediatric Inclusion in Trials](#)
- [Nominate a Nephrotic Syndrome Specialist](#)

KidneyHealthGateway.com

We are now in week 6 of the KidneyHealthGateway.com launch, a patient-friendly pre-screener for Nephrotic Syndrome clinical trials. We are pleased to share that more than 1,500 people have registered with a profile on the site, and 87% of those registrants are patients or parents of patients with a primary glomerular disease.

Next steps include a continuation of the professional marketing campaign which will continue to push new visitors and registrants to the site. As you browse the internet, you may see the drivers of this campaign, including display ads, search engine marketing results, YouTube ads, and social media ads on various platforms. In addition, NephCure is focused on re-engaging with the patient/patient parent registrants on both a broad and individual level to further educate, inform, and drive them towards contacting a trial site near them.

Please continue to spread the word about KidneyHealthGateway.com. Several workgroups have identified specific ways to enhance or promote the site, and we encourage you to share the site with your peers and patients in addition to providing feedback on the site to NephCure directly.

Nephrotic Syndrome Specialists Program

The Nephrotic Syndrome Specialists program is a new resource to help glomerular disease patients connect to experts as early as possible in their diagnoses. The goal is to improve patients' quality of life and reduce the potential for irreversible kidney damage by providing earlier access to clinicians who are specialists in and routinely treat Nephrotic Syndrome. The program went live on February 19th and has grown to include 91 academic and community-based experts. We hope to eventually expand this list to 300 total physicians worldwide. You can see a full list of the specialists and more information about the nomination and vetting process at kidneyhealthgateway.com/specialists.

In the coming weeks, these physicians will receive plaques which highlight their Nephrotic Syndrome Specialist designation, in addition to patient-facing trial recruitment materials and other one-sheets to facilitate and augment patient-provider conversations. We are also formulating a "best practices guide" for specialists to partner with local nephrologists who may not be experts in glomerular diseases. We aim to create regional trial-readiness through encouraging and deepening these physician-to-physician connections.

The Nephrotic Syndrome Specialists nomination form is now live at nephcure.org/NSS. Nominations may come from peers and other professional stakeholders, and physicians may also self-nominate. The international postal code search tool will be live by May 1, so there is now a focus on adding specialists internationally, starting with Canada and the United Kingdom.

Nephrotic Syndrome Community Expansion

We continue to receive high interest from the international patient community and are pleased to announce that global community access to KidneyHealthGateway.com will be available May 1.

In March, NephCure and IGANF met with a multi-disciplinary research team focused on IgAN at the University of Alabama, Birmingham. This lab includes clinical nephrologists, analytical biochemists, and molecular immunologists who aim to develop a blueprint for targeted treatments for this disease.

In mid-May, Gateway Initiative representatives will attend the ERKNet Annual meeting in Heidelberg, and on June 14th, NephCure will have the opportunity to facilitate a patient panel at the ERA-EDTA 2019 meeting in Budapest. In this vein, we would like to extend a formal invitation to partner with other stakeholders from the international community on patient programs related to the Gateway Initiative.

NephCure and IGANF look forward to attending the Externally-Led Patient-Focused Drug Development meeting on IgAN to be held Aug. 19th. The output of this meeting will be a “Voice of the Patient” report for the FDA, who will use it as a reference for future decisions on potential medicines for IgAN.

June 26, 2019 - Next In-Person Meeting

The next in-person Clinical Trial Recruitment Collaborative Meeting will be held June 26th in Washington, DC. [Please respond here by May 1 to ensure your participation.](#) There is no cost to attend. Thank you in advance for making time for this pivotal meeting towards ensuring no NS trial fails due to insufficient recruitment.

The Gateway Initiative: Clinical Trial Recruitment Collaborative

SEP. '18 Workgroup Landscape Analysis	OCT. '18 Workgroup Landscape Analysis	NOV. '18 Nov. 15, 2018 Clinical Trial Collaborative Meeting - Washington, D.C.	DEC. '18 Workgroup Solutions Engagement	JAN. '19 Workgroup Solutions Engagement	FEB. '19 Workgroup Solutions Engagement	MAR. '19 Workgroup Solutions Engagement	APR. '19 Workgroup Solutions Engagement	MAY '19 Workgroup Solutions Engagement	JUN. '19 June 26, 2019 Clinical Trial Collaborative Meeting - Washington, D.C.
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Thank You!