



**NephCure Kidney International
The Gateway Initiative**

Workgroup Co-Leads

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Howard Trachtman, MD
Suneel Udani, MD
Patrick Walker, MD

**Clinical Trial Collaborative
Advisory Committee**

Robert Star, MD, National Institutes of Health and National Institute of Diabetes and Digestive and Kidney Diseases
*Ad hoc

Cindy Roy, PhD, National Institutes of Health and National Institute of Diabetes and Digestive and Kidney Diseases
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Aliza Thompson, MD, MS, US Food and Drug Administration

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Joshua Tarnoff, NephCure Chief Executive Officer

Lauren Lee, NephCure Chief Research Officer

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Lauren Eva, NephCure Director, Marketing & Communications

Emma Palitz, NephCure Coordinator, Research & Engagement Marketing

Dear Clinical Trial Recruitment Collaborators,

I am pleased to share that there has been robust activity from all workgroups and other pillars of the Gateway Initiative as we continue our focused efforts on glomerular disease clinical trial recruitment. The Gateway Initiative enjoys strong international support across KOLs, FDA, ASN, NIH, consortia, patients, and other important stakeholders in the glomerular disease community.

As a reminder, the Gateway Initiative encompasses multiple strategic interventions working in parallel:

- **Six Clinical Trial Recruitment Collaborative Workgroups**; made up of 100 of the world's leading glomerular disease researchers, clinicians, trialists, and other professionals; in addition to government and patient advocacy group representatives, patients and parents, and study sponsors;
- **KidneyHealthGateway.com**, connecting patients to trials near them;
- **Nephrotic Syndrome Specialists Program**, a public recognition and referral to NS experts who regularly treat NS patients and recruit for clinical trials;
- **Nephrotic Syndrome Community Expansion**, the need for a shared sense of urgency across the globe and inclusion of all patient groups.

Of particular note are two new potentially significant programs from the Workgroups: to develop a pediatric risk/benefit analysis which may lower the barriers towards pediatric inclusion in Phase 2/3 studies, and to ensure retention of trial-ready patients by creating a centralized "Screen Failure Registry" across Nephrotic Syndrome trials.







I would like to close by thanking all of the Gateway Initiative participants and encouraging everyone to continue to stay focused on their near-term deliverables. We are already beginning to see positive results, but there is much work to be done. Don't forget to register for the [June 26th meeting in Washington, DC](#), where we will share more information about successes thus far, and identify our collective paths moving forward.

Warmly,
Lauren Lee
Chief Research Officer
NephCure Kidney International






Clinical Trial Recruitment Collaborative: Workgroup Updates

KEY:  COMPLETE  IN PROGRESS  NOT YET STARTED  ON HOLD




CLINICIANS WORKGROUP

-  Partner with ASN to expand GlomCon series reach for summer, 2019
-  Participate in several US-based Nephrotic Syndrome meetings (eg ASN, MWPNC, Kidney NExT5)
-  Finalize clinical trial curriculum, speakers, and featured trials to be presented on GlomCon, summer 2019
-  Increase social media presence to engage clinicians with research, CME series, and current trials
-  Create clinicians' perspectives piece on importance of clinical trials in nephrology (GN especially)
-  Work with NephCure to develop and market clinician-focused section of KidneyHealthGateway.com






CONSORTIA WORKGROUP

-  Select locations for regional trial-readiness meetings, to be hosted by NephCure
-  Alert patients of KidneyHealthGateway.com trial-finder tool to increase interventional trial enrollment
-  Work with relevant Patient Advisory Boards to collect feedback on KidneyHealthGateway.com
-  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

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

-  Develop trial segmentation tool to highlight differences of each ongoing clinical trial for researchers and clinicians
-  Develop centralized screen failure registry across NS trials to retain trial-ready patients
-  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
-  Evaluate patient/family barriers to clinical trial participation
-  Collaborate with government agencies to develop pediatric risk/benefit analysis for various forms of NS to include pediatric patients in Phase 2 + 3 studies
-  Finalize and publish white paper addressing regulatory pathways for pediatric inclusion in rare disease clinical trials

EX-US INCLUSION WORKGROUP

- Create a rolodex/directory of key international contacts/resources
- Dedicate an upcoming World Kidney Day to FSGS
- Attend current international meetings (eg ERKNet, ERA/EDTA) 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)
Nicolas Garnier (Pfizer)
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)
Nicolas Garnier (Pfizer)
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

- [Consortia Resource Library](#)
 - [Where Do You Get Your News? Survey](#)
 - [Ex-US Directory of Experts](#)
 - [GlomCon Conference Information](#)
 - [Nominate a Nephrotic Syndrome Specialist](#)
 - [Printable list of all glomerular disease trials](#)
- For industry partners:**
- [Pediatric Inclusion in Trials](#)

KidneyHealthGateway.com

This is the tenth week of the KidneyHealthGateway.com launch, and the number of registrants continues to grow at a steady pace, with now more than 2,500 people registered with a profile on the website. More than 84% of these registrants are patients or parents of patients with a primary glomerular disease.

Wonderfully, the international postal code search is now live on the website, and trials with active international sites that are listed on clinicaltrials.gov should also appear on KidneyHealthGateway.com.

As visitors and registrants engage with the website, they are guided towards reaching out to nearby study sites for trials for which they qualify. To date, patients or parents have sent more than 100 emails to trial sites directly to learn more about and get enrolled in a clinical trial.

NephCure follows up with each patient individually after a two-week waiting period, but **if you are a PI, study coordinator, or study sponsor, please be on the lookout for and respond promptly to these patient emails.** The subject line will read “Patient trial inquiry from NephCure.” These patients have been pre-screened for the trials they are inquiring about and are ready to get enrolled.

Additionally, more than 100 patients or parents have reached out to NephCure directly through the website for additional questions; 20% of these inquiries pertained to clinical research opportunities or getting connected to a specialist. NephCure follows up individually with each patient inquiry within a 48-hour period.

To further drive patient/parent registrants to return to the website and contact a study site near them, we are initially focused on two methods:

- **Email re-engagement campaign:** Registrants receive 5 emails distributed over 3 weeks to welcome them to NephCure and encourage them to return to KidneyHealthGateway.com and “Contact a Site” to learn more about trial eligibility.
- **Grassroots phone campaign via Regional Volunteers:** Registrants receive personal phone calls to welcome them to their NephCure Regional Community and answer any questions regarding clinical research participation, seeking a Nephrotic Syndrome Specialist, etc.

Details and results from these re-engagement campaigns will be shared in future Gateway eNewsletters.

Nephrotic Syndrome Specialists Program

The Nephrotic Syndrome Specialists nomination form is live at nephcure.org/NSS. Please take a moment to nominate a physician you believe should be considered for inclusion. Nominations may come from peers and other professional stakeholders, and physicians may also self-nominate. In the next few weeks, we will be soliciting ex-US nominees to expand the program globally.

The goal of the Nephrotic Syndrome Specialists Program 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. You can see a full list of the specialists and more information about the nomination and vetting process at kidneyhealthgateway.com/specialists.

Nephrotic Syndrome Community Expansion

Global postal code access to KidneyHealthGateway.com is now available. We anticipate that the website will be translation-ready into several different languages, focusing on Spanish, French, and Portuguese initially, by mid-late summer.

We are pleased to share that the inaugural board meeting for NephCure Canada will be taking place May 8th.

Next week, Gateway representatives will attend the ERKNet Annual meeting in Heidelberg, and on June 14th, NephCure will facilitate a patient panel at the ERA-EDTA 2019 meeting in Budapest. NephCure and IGANF also look forward to attending the Aug. 19th Externally-Led Patient-Focused Drug Development meeting on IgAN.

June 26, 2019 - Next In-Person Meeting

The next Clinical Trial Recruitment Collaborative Meeting will be held June 26th in Washington, DC. [Please respond here 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 (Completed)	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!