



**NephCure Kidney International  
The Gateway Initiative**

**Workgroup Co-Leads**

**Laura Barisoni, MD**  
**Kirk Campbell, MD**  
**Barbara Gillespie, MD, MMS, FASN**  
**Debbie Gipson, MD, MS**  
**Tobias Huber, MD**  
**Meg Jardine, MBBS, PhD**  
**Matthias Kretzler, MD**  
**Moin Saleem, MB, BS, PhD, FRCP**  
**Stuart Shankland, MD, MBA, FRCP**  
**Howard Trachtman, MD**  
**Suneel Udani, MD**  
**Patrick Walker, MD**

**Clinical Trial Collaborative  
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**Robert Star, MD**, National Institutes of Health and National Institute of Diabetes and Digestive and Kidney Diseases  
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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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**Aliza Thompson, MD, MS**, US Food and Drug Administration

**Kimberly Smith, MD, MS**, US Food and Drug Administration and Kidney Health Initiative

**Melissa West**, Project Director, Kidney Health Initiative

**Jonathan Barratt, PhD, FRCP**, National Health Service

**Elaine Kamil, MD**, NephCure Co-Medical Director

**William Smoyer, MD**, NephCure Co-Medical Director

**Laura Barisoni, MD**, President, Renal Pathology Society

**Moin Saleem, MB, BS, PhD, FRCP**, Head of Bristol Renal

**Irving Smokler, PhD**, NephCure Board President

**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,**

I am looking forward to seeing many of you at Wednesday's Clinical Trial Recruitment Collaborative, where we will review the current glomerular disease trial landscape, share updates about our progress thus far, and identify our collective paths to move forward. We'll hear from US Senator Debbie Stabenow (D-MI), US Congressman Ted Deutch (D-FL), FDA and ASN representatives, NephCure's founder and President, Dr. Irv Smokler, the six Workgroup Co-Leads, and input from a panel of patients and parents on what matters to them, as well as many other speakers. There will be interactive breakout sessions for each Workgroup and time to hear from industry directly on current recruitment struggles. We will be welcoming a robust group of attendees from around the US and worldwide.

As a reminder, if you are not able to make the in-person meeting, we will be live-streaming the morning sessions starting at 8:15 am EDT, Wednesday, June 26th. You can view the live-stream on YouTube at <https://youtu.be/nrb4aOgzn4A> or on NephCure's Facebook page at <https://www.facebook.com/NephCure>.

In this month's newsletter, we are starting a new "Featured Workgroup" section, where we will hear highlights from each Workgroup's Co-Leads directly on some of the initiatives that their group has identified or begun progress on.

In closing, I send my sincere thanks to all of the Gateway Initiative participants and sponsors. Many of you have found a way to fit Gateway Initiative activities into your already very busy schedules, and we are grateful for your time and attention to this critical issue. This week, we will be able to share the positive results of our collective labors, but of course much work remains to be done. On behalf on patients and families everywhere whose lives have been affected by these diseases, I thank you for your commitment to this cause.

Warmly,

Lauren Lee  
Chief Research Officer  
NephCure Kidney International

## Featured Clinical Trial Collaborative Workgroup: *Clinicians Workgroup*

### *Describe some of the initiatives your workgroup has identified/begun progress on thus far.*

One of our most impactful initiatives is a partnership with GlomCon, where we will highlight promising clinical trials while reviewing clinical trial methodology and pitfalls.

GlomCon, a web-based nephrology platform with a large international presence, reaches more than 2200 clinicians and pathologists from 108 countries. Leveraging this existing framework will help us spread the word about trials for a number of glomerular diseases. We have identified several experts in each disease state who will speak about the diseases, current standards of care, and the relevant trials that are currently running.

There are several goals for this series. For the general nephrologist who may not see many cases of GD, we hope to raise awareness about the trials by reviewing preliminary data and the mechanism of drug action, so that they are comfortable referring to a trial should a case arise.

It's also about changing the culture in nephrology, because this number of interventional trials is somewhat foreign to our field. We hope that nephrologists will consider how trials can fit into their practices, and when the right time to refer is. Many times, trials may be seen as an option of “last resort” when all other treatment options have failed. We want to change the thinking around that, because at that point, the patient may have progressed too far in their disease and will no longer qualify for any trials.

Once the nephrology community is aware of the trials, they can visit [KidneyHealthGateway.com](http://KidneyHealthGateway.com) to learn more about a specific trial/drug or find out where to make a referral should they see a patient who may be eligible.

The GlomCon Clinical Trial Conference Series will begin in July 2019 and run for 12 months. Though the time demands for both the speakers and the target audiences can be challenging, we are confident that it will engage a broad audience of practicing nephrologists, trainees and patients to make the case that trial participation and awareness are an absolute necessity in the current glomerular disease treatment landscape.

We are also developing a Memorandum of Understanding to optimize interactions between referring nephrologists and trialists. This would be initiated by the trialist when a patient goes for trial screening. If the patient would like to continue seeing their general nephrologist, the MOU will allow the trialist and general nephrologist to have a clear understanding and open line of communication in terms of how care would work. Many nephrologists may fear “losing” their patients; nephrology is a unique field where patients tend to be chronic, and they develop relationships with their providers—they may even see them for the rest of their life. Nephrologists may feel they've made a significant investment in these patients and fear losing them when referring for a trial. This MOU can help assuage those fears.







Finally, we are seeking out AI software, like IBM Watson, which could analyze patients' EHRs, looking at ICD10 codes, pathology results, physician notes, etc. to narrow down the list of patients who may be eligible for a given trial. This would be specific to each practice—not shared information. But this could help practices identify patients who may be eligible for a given trial and lower the burden for the initial screening.

***– Drs. Kirk Campbell and Suneel Udani, Clinicians Workgroup Co-Leads***






# 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

The number of KidneyHealthGateway.com registrants continues to grow at a regular pace, with now close to 4,000 people registered with a profile on the website. At least 77% of these registrants are patients or parents of patients with a primary glomerular disease. The majority, 37%, are patients with Nephrotic Syndrome, while 19% have FSGS and 17% have MCD. IgAN and MPGN patients each represent 9% of the patient/parent registrants, while 6% have identified as having MN and 3% with C3G. Strikingly, 94% of these registrants are “new” to NephCure—not previously found in our database.

International postal code search is 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 130 emails to trial sites directly to learn more about and get enrolled in a clinical trial. Once patients reach out to a study site, it is incumbent on the study coordinator, PI, or study sponsor to follow up with the patient’s outreach.

With the crucial support of the NephCure Regional Community Volunteers, we launched a grassroots, peer-to-peer phone campaign in mid-June to welcome KidneyHealthGateway.com registrants to their NephCure Regional Community and answer any questions regarding clinical research participation, seeking a Nephrotic Syndrome Specialist, etc. We are still gathering the results of these efforts, but anecdotal feedback indicates that many of the patients have poor disease education and are in need of care from a glomerular disease specialist. As one volunteer shared, “I was struck by their stories in dealing with doctors. Most are just controlling blood pressure and have no thought of a biopsy or diagnosis beyond Nephrotic Syndrome. I feel like these physicians are just waiting to send them to dialysis. One patient told me his doctor said he had end stage renal disease but just to control his blood pressure... really just a shocking standard of care.”

## Nephrotic Syndrome Specialists Program

We are now actively seeking ex-US Nephrotic Syndrome Specialist nominees to expand the program globally. 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.

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](http://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.

Since the beginning of this year, Gateway Initiative representatives have presented at or attended at least 12 US and international meetings, including the ERKNet Annual meeting in Heidelberg, the ERA-EDTA 2019 meeting in Budapest, and the Coalition of Patient Advocacy Groups in Washington, DC.

This summer, a taskforce made up of patient representatives from the IGA Nephropathy Foundation of America and NephCure, as well as clinical experts in IgAN, will come together to guide the organizational integration of IGANF and NephCure and create and vet new patient resources tailored to the IgAN community. NephCure and IGANF also look forward to attending the Externally-Led Patient-Focused Drug Development meeting on IgAN, to be held Aug. 19th.

## 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!