



**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  
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  
\*Ad hoc

**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,**

Thank you for joining us for our second edition of the Gateway Initiative eNewsletter! We are eager to share updates with you as February was a very productive month. We publicly launched [KidneyHealthGateway.com](http://KidneyHealthGateway.com) and the Nephrotic Syndrome Specialists Program, in addition to making progress across all six of the Clinical Trial Recruitment Collaborative Workgroups.

We are pleased to formally recognize the support of the industry partners in this initiative: [Retrophin, Inc. \(Platinum Sponsor\)](#); [Pfizer Inc.](#); [Mallinckrodt Pharmaceuticals](#); [Reata Pharmaceuticals](#); [Variant Pharmaceuticals, Inc.](#); [Goldfinch Bio](#); [Achillion Pharmaceuticals](#); [ChemoCentryx](#); and [Vertex Pharmaceuticals](#). Their contributions will soon make new treatments for Nephrotic Syndrome patients possible. We thank them all and enthusiastically welcome them to the Clinical Trial Recruitment Collaborative Workgroups.

Please see included the monthly updates from the Gateway Initiative:

- [Six Clinical Trial Recruitment Collaborative Workgroups](#);
- [KidneyHealthGateway.com](http://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.

I would also like to thank Chelsey Fix for her abundant contributions to the Gateway Initiative. We wish her well in her new role and welcome Emma Palitz, NephCure Coordinator, Research & Engagement Marketing, to the Gateway team.

Thank you all for your collaborative efforts to improve the trial recruitment landscape and enable new therapies for Nephrotic Syndrome. Happy Rare Disease Day!





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

-  Hold a pilot webinar with GlomCon on IgAN trials
-  Expand workgroup membership to be geographically inclusive & representative of private practices
-  Develop a clinical trial curriculum to be presented on GlomCon, summer 2019
-  Work with NephCure to develop and market clinician-focused section of KidneyHealthGateway.com






## CONSORTIA WORKGROUP

-  Create a library that summarizes available resources of all consortia/networks
-  Explore possibility of a universal data dictionary
-  Work with relevant Patient Advisory Boards to collect feedback on KidneyHealthGateway.com
-  Select locations for regional trial-readiness meetings, to be hosted by NephCure






## PATHOLOGY WORKGROUP

-  Draft editorial that outlines the need for pathologists' involvement in clinical trials
-  Finalize and publish 1st editorial
-  Collaborate with the Renal Pathology Society Workgroup to create a website with information about all ongoing clinical trials
-  Create a proposal of workflow for a centralized renal pathology consultancy group

## STUDY OVERLAP WORKGROUP

-  Create a resource (mobile application/website) that highlights the differences of each ongoing clinical trial
-  Create a model of master protocol for FSGS
-  Work with NephCure to explore Study Overlap tool possibility on KidneyHealthGateway.com
-  Create a best practices list for engaging patients/families in trial design
-  Explore telehealth solutions through case studies

## PEDIATRIC INCLUSION WORKGROUP

-  Understand industry perspective of pediatric inclusion in trials through a survey
-  Document activities of workgroup through a white paper
-  Understand patient/family barriers to trial participation & create resources to address those barriers
-  Collaborate with relevant groups to unify efforts around creating a trial-ready culture among the professional pediatric nephrology community / institutions
-  Summarize the historical and current landscape of pediatric nephrology trials and make evidence-based suggestions on how to meaningfully include children in 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
- Explore current international meetings that can 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 Lickerman (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)

## KidneyHealthGateway.com

We successfully launched the first phase of the patient-friendly pre-screener/connector for all Nephrotic Syndrome clinical trials, KidneyHealthGateway.com, on February 19, 2019. In conjunction with the website launch, the full digital marketing campaign went live on February 22. We're excited to share more data around the marketing outreach and website users in the coming months—please stay tuned in upcoming newsletters for more on this new website.

We have heard great feedback from many of you and hope that you will continue to use the site and share it with patients, peers, and other colleagues. Thank you to everyone who has already helped spread the word about the launch of KidneyHealthGateway.com, including the Kidney Health Initiative and our many Facebook and Twitter supporters. As Kara Jones, patient mom and longtime NephCure supporter said, “Options beyond steroids... that is music to our ears! For 16 years we have watched steroids wreck havoc on Cheech’s body and crush his spirits. To know there are new therapies on the horizon and several clinical trials happening RIGHT NOW is literally a dream come true!” We couldn’t agree more, Kara!

We will be upgrading and enhancing the website’s offerings in future phases, to include: expanded global participation, a physician-access portal, daily health journal, and genetic screening opportunities.

Please be on the lookout for opportunities to provide more structured feedback in an online survey, coming soon.

## Nephrotic Syndrome Specialists Program

Together with the launch of KidneyHealthGateway.com, the Nephrotic Syndrome Specialists Program went live on February 19th, with an initial list of 86 academic and community-based experts in treating patients with glomerular disease. Each Nephrotic Syndrome Specialist has been carefully selected through a rigorous nomination and vetting process, and they now have a listing on KidneyHealthGateway.com with their contact information and more details about their practice. They will also be receiving a wall plaque for their office or clinic so that they may proudly display their designation as a Nephrotic Syndrome Specialist.

We plan to begin resourcing these specialists soon with patient-facing trial recruitment materials and other helpful one-sheets to facilitate and augment patient-provider conversations.

The next version of this program will include a “how-to guide” with best practices for working with other 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 Program is an ever-growing list. Our next call for nominations will begin on May 1, with a focus on adding specialists from Canada and the United Kingdom. Please note that physicians may self-nominate; please contact Lauren Lee for more information.

## Nephrotic Syndrome Community Expansion

We are thrilled to announce that Bonnie Schneider, Founder and Director of the IGA Nephropathy Foundation of America, is now a full-time member of the NephCure family as Vice President of IgAN Affairs. We are in the process of a formal merger with the IGAN Foundation to be finalized by the end of the year. We welcome Bonnie to our team and look forward to deepening our relationship with her organization. Together, NephCure and the IGAN Foundation are regularly meeting with interested pharmaceutical sponsors to ensure that the Gateway Initiative's resources and strategies fully encompass the expansion of our rare kidney disease community.

We have broadened the international representation on the six workgroups to include additional members from Canada and the United Kingdom. These new workgroup members will also be in attendance at the June 26 meeting in Washington, DC.

We received very high interest from the international patient community throughout the KidneyHealthGateway.com launch and eagerly look forward to expanding participation to the global community on this website.

We have continued to advance dialogues with various international registries and Nephrotic Syndrome organizations; we are also proactively seeking out additional partnerships with other international consortia, patient advocacy groups, etc. Please let us know if there is an international group you feel would enhance our activities. We welcome your introductions.

## June 26, 2019 - Next In-Person Meeting

The next in-person Clinical Trial Recruitment Collaborative Meeting will be held **June 26th, 2019, at the Georgetown Marriott in Washington, DC**. Registration for this will open in the next few weeks; please check your email for forthcoming information on securing your spot. Thank you in advance for making time for this pivotal meeting towards ensuring no Nephrotic Syndrome 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.
--	---	--	--	--	--	--	--	---	--

**Thank You!**