



KIDNEY HEALTH INITIATIVE

2018 ANNUAL REVIEW

“...As medicine evolves into the provision of personalized care, so too must care for patients or people living with kidney diseases. KHI through its multidisciplinary partners is poised to meet these challenges and ensure the safe introduction of new technologies and therapeutics to enhance the care of patients with kidney diseases.”

- Mark Okusa, MD, FASN

President, American Society of Nephrology (ASN)

The Kidney Health Initiative's (KHI) mission is to advance scientific understanding of kidney health and patient safety implications of new and existing medical products and to foster development of therapies for diseases that affect the kidney by creating a collaborative environment in which U.S. Food and Drug Administration (FDA) and the greater nephrology community can interact to optimize evaluation of drugs, devices, biologics, and food products.



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Interview with

Prabir Roy-Chaudhury, MD, PhD, FASN ASN Co-Chair of KHI

HOW HAS KHI IMPACTED THE NEPHROLOGY COMMUNITY OVER THE PAST FIVE YEARS?



I think that the greatest impact of KHI over the last five years has been that it has provided a platform, a meeting place if you will, for all the different stakeholders within the kidney community. What I have learned more than anything else is that if you get talented and committed people from different backgrounds together then wonderful and impactful things just seem to happen. And yes; at times I think that some of the intangibles that have come about because of KHI, such as hope to our patients; excitement for our trainees and hopefully more confidence within our industry partners about the kidney disease area, are worth even more than the more tangible impact of our projects.

WHAT HAS BEEN THE MOST REWARDING PART OF YOUR ROLE AS THE ASN CO-CHAIR FOR KHI?

The most rewarding aspect of my role has been that I have had an incredible opportunity to meet with so many talented, committed and innovative people from extremely diverse backgrounds with very different views and experiences, about not just kidney disease but life in general. And I truly believe that I have come out of these five years as a more holistic person with a better and more thoughtful mind; and for that I will forever remain indebted to KHI, ASN, the U.S. Food & Drug Administration (FDA), to the incredibly brave patients that have been an integral part of KHI, and all of the wonderful people who I have interacted with over the last few years.

WHAT DO YOU ENVISION FOR THE FUTURE OF KHI?

I know that KHI will go from strength to strength. I know that because of the quality and commitment of the people who make up KHI: the staff, our board, ASN, FDA, and our more than 90 member organizations. The real impact and the real work of KHI is just beginning! Every organization has a natural life cycle from infant and toddler to teenager, and I think now for KHI, into young adulthood. I think that this is the time for KHI to be bold in a very collaborative sense; to be the facilitator/convenor/manager in the kidney disease innovation area, to address head on some of the big issues that we face in nephrology (from infrastructure to telemedicine to CKD progression), all while keeping the patient perspective front and center.

Interview with

Janet Woodcock, MD

Director, Center for Drug Evaluation and Research (CDER)
U.S. FDA

WHY ARE PUBLIC-PRIVATE PARTNERSHIPS IMPORTANT TO FDA? HOW DO THEY HELP SUPPORT FDA'S PRIORITIES?

FDA requires many scientific tools in order to accomplish its mission. For example, we need good testing and monitoring methods for drug manufacturing, we need non-clinical methods that can predict safety problems before people are tested, we need ways to assess candidate drugs in the clinic, both for safety and whether they work. These clinical methods—everything from trial design and conduct to various biomarkers—should enable us to make a thorough assessment of a drug's performance, both its effectiveness and its safety—before it gets on the market. Unfortunately, many current tools are not very good, or we may lack them altogether. The FDA is not charged (or funded) to develop such tools, and unfortunately, other groups aren't either. So we need to work collectively through public-private partnerships to get such tools developed. A lot of science has to be done to get a tool to the level that it can be used to make decisions about people's health.

HOW CAN A DIVERSE PUBLIC-PRIVATE PARTNERSHIP, LIKE KHI, HELP ACCELERATE DISCOVERY OF NEW THERAPIES?

KHI and other consortia can help in many ways, depending on the need and the feasibility. Innovators won't invest in a therapeutic area if they feel the path to market is too risky and unclear. Partnerships can help by developing and modeling new trial designs, by working on new endpoints and the trials they can be used in, by evaluating novel methods for monitoring safety, by reaching out to patients who are interested in enrolling in trials, by studying the natural history of poorly-understood diseases, and in many other areas.

WHAT ARE WAYS CONSORTIA CAN HELP TO INCLUDE THE PATIENT PERSPECTIVE IN THE DRUG DEVELOPMENT PROCESS?

Patient-focused drug development meetings can serve as a kickoff to understand what matters to patients about the disease and its treatment. From there, more formal approaches to understanding the burdens of the disease and of treatment can be conducted. FDA has started to put out guidance to groups on how to do this. Once these are better understood, then instruments like patient reported outcome measures can be developed for the disease. Following the patient point of view in natural history studies is also very useful to see how these patient-identified burdens evolve over time.



KHI's Impact

As the largest consortia in the kidney community, KHI serves as a catalyst. By bringing together a diverse set of stakeholders in a pre-competitive environment, KHI workgroups create deliverables that assist innovators with the development of therapies for people living with kidney diseases.



KHI Strategic Priorities & Projects

Developing new therapies and treatments in any patient population is complicated and challenging. KHI's interconnected priorities help address these challenges and focus the kidney community on working together to meet the needs of people living with kidney diseases. Visit the KHI website to learn more about each current project listed below.

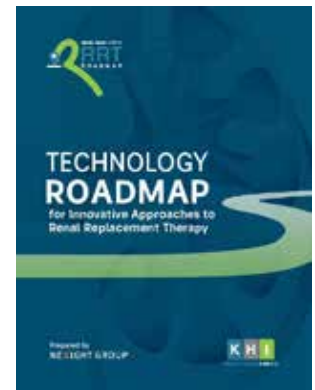


Linde et al: Overcoming Barriers in Kidney Health—Forging a Platform for Innovation. JAmSoc Nephrol 27: 1902–1910, 2016

2018 Signature Initiative

TECHNOLOGY ROADMAP FOR INNOVATIVE RENAL REPLACEMENT THERAPY

The roadmap is a technical document that catalogues the challenges, identifies solutions, and outlines the research activities in short, mid and long-term timeframes. Benchmarks were developed by a team representing every stakeholder within the kidney community; patients, payers, regulators, industry, physicians, researchers, and entrepreneurs.



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kidneyhealthinitiative.org

Patient-Centered Approach



CURRENT STATE



**FUTURE
STATE**



**Multi-
Disciplinary
Collaboration**

**Disruptive
Innovation**

**Increased
Investment**

“ ...While we should not underestimate the complexities of both the technological challenges and regulatory requirements involved in bringing innovative ESRD products to market, a roadmap will help to bring innovators from various fields into the “kidney space” with new ideas, “out of the box” approaches, and substantive financial resources from corporate, philanthropic and venture capital communities. Our current and future patients rely on our drive and persistence to provide solutions which improve their quality of life.”

- Joseph V. Bonventre, MD, PhD, FASN

2012–2018 Milestones & Deliverables

2012

KHI Established

2014



WORKSHOP

- First Workshop: Standardized Pharmacokinetics Assessment in Patients Receiving CRRT

2013



MEETING

- First Annual Stakeholders Meeting



PUBLICATION

- First Publication: “Fostering Innovation, Advancing Patient Safety: The Kidney Health Initiative”



PROJECTS AND MEMBERS

- First Three Pilot Projects Endorsed
- 61 Pilot Members Join KHI

2015



PUBLICATION

- Second Publication: “Pharmacokinetic Assessment in Patients Receiving Continuous RRT: Perspectives from the Kidney Health Initiative”



WORKSHOP

- Second Workshop: Understanding Patient Preferences for Medical Device Development



PROJECTS AND MEMBERS

- Patient & Family Partnership Council Founded

2016



PUBLICATIONS

- “Overcoming Barriers in Kidney Health – Forging a Platform for Innovation”
- “Pragmatic Trials in Maintenance Dialysis: Perspectives from the Kidney Health Initiative”

2017



PUBLICATIONS

- “Stimulating Patient Engagement in Medical Device Development in Kidney Disease: A Report of a Kidney Health Initiative Workshop”
- “Perspectives from the Kidney Health Initiative on Advancing Technologies to Facilitate Remote Monitoring of Patient Self-Care in RRT”
- Publications on Clinical Trial Endpoints For Vascular Access
 - “Clinical Trial End Points for Hemodialysis Vascular Access: Background, Rationale, and Definition”
 - “Recommended Clinical Trial End Points for Dialysis Catheters”
 - “Definitions and End Points for Interventional Studies for Arteriovenous Dialysis Access”
 - “FDA Regulatory Perspectives for Studies on Hemodialysis Vascular Access”

2018



WORKSHOPS

- Prioritizing Symptoms of ESRD Patients for Therapeutic Interventions
- Identification of Appropriate Endpoints for Clinical Trials in Hyperoxaluria
- Understanding and Overcoming the Challenges to Involving Patients with Kidney Disease in Cardiovascular Trials



PUBLICATIONS

- Publications on Hemodiafiltration Systems:
 - “Hemodiafiltration to Address Unmet Medical Needs ESKD Patients”
 - “Regulatory Considerations for Hemodiafiltration in the United States”
- “Symptom Prioritization Among Adults Receiving In-Center Hemodialysis”
- “Technology Roadmap for Innovative Approaches to Renal Replacement Therapy”
- Upcoming: “Establishing Endpoints for Lupus Nephritis Clinical Trials”
- Upcoming: “Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy”



MEETING

- Held Sixth Annual Stakeholder Meeting



PROJECTS AND MEMBERS

- 93 companies and organizations participate as members of KHI

Project Spotlights



ADVANCEMENT OF PATIENT AND FAMILY PARTNERSHIP

Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions

This workgroup was formed to prioritize the symptoms experienced by in-center ESRD patients. People living with kidney diseases were involved in the planning and execution of all phases of this project. The first phase included three focus groups and an online survey to prioritize physical and mood symptoms.

A workshop was then hosted in January 2018 to identify strategies to address the three physical symptoms. Patient participants in the workshop had a critical role in ensuring the solutions are meaningful and patient-centered.

“

...The ESRD Symptoms project has laid a foundation for patient voices and priorities to be heard. I hope that this project will not only serve as insight and a guide for researchers, but also spawn additional research projects that hopefully bring new treatments to these symptoms. ”

- Caroline Wilkie

Prioritized Symptoms:

Physical

- Insomnia
- Muscle Cramping
- Fatigue

Mood

- Depression
- Anxiety
- Frustration



CLINICAL TRIAL ENDPOINT AND DESIGN

Outcome Measures in Lupus Nephritis

In collaboration with leaders in nephrology and rheumatology, this KHI workgroup reviewed data from a large number of lupus nephritis (LN) patients. These data sets came from LN cohorts established by nephrologists and rheumatologist from around the world and were followed over time for outcomes after treatment of active disease. The goal was to identify one-year surrogate endpoint(s) that could be used in clinical trials of new LN therapeutics that could predict future adverse kidney outcomes. The workgroup is proposing predictive models for ESRD, chronic kidney disease, and severe acute kidney injury. The models were tested and validated in an independent cohort.

KHI would like to thank the Lupus Nephritis Trial Network for their support with the project.

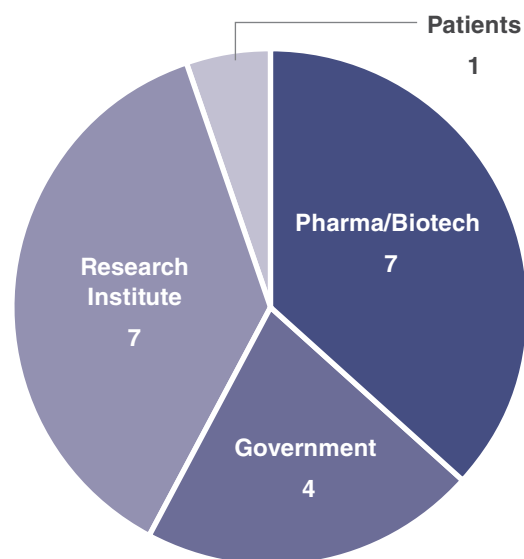
COLLABORATION WITH FDA AND OTHER GOVERNMENT AGENCIES

Drug Development in Children with Chronic Kidney Disease

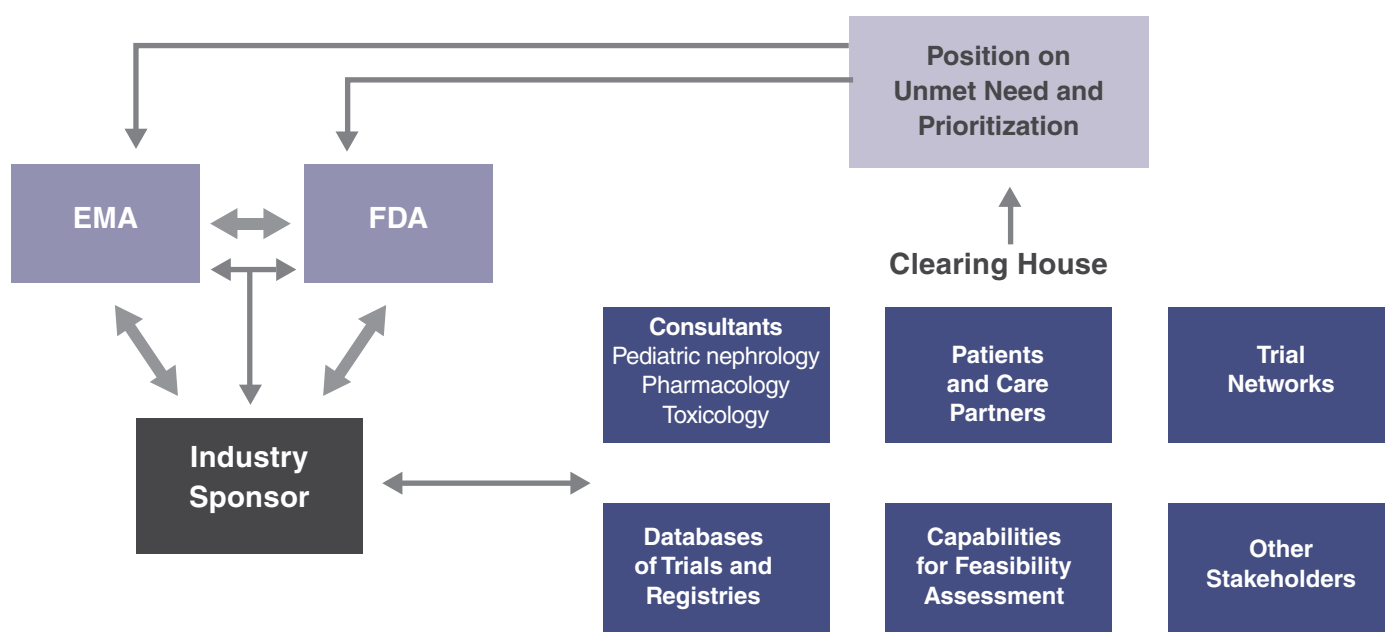
This project aims to foster drug development in children with chronic kidney disease (CKD) by commissioning a workgroup to take inventory of current challenges, share insights and lessons learned, and develop consensus-based recommendations for overcoming the barriers.

The workgroup is hoping to establish a standing entity, or "clearing house". This "clearing house" will serve as a central location for interested stakeholders to connect with trialists and sources of data and subjects. This workgroup is hoping to fill the unmet need of coordination of drug studies in children with CKD to optimize success, clinical resources, and completion of regulatory requirements. The overarching goal of this project is to facilitate harmonization, prioritize drugs, and to optimize trial design and execution.

Workgroup Demographics



The Future: Drug Development in Children with CKD



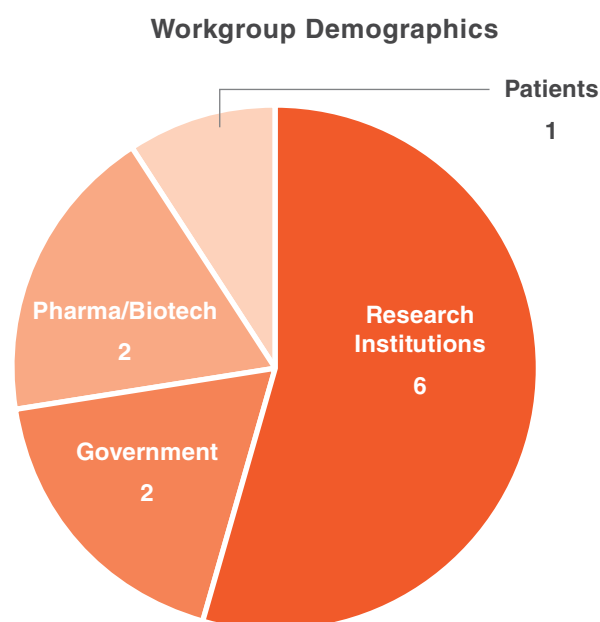
Project Spotlights



CLINICAL TRIAL ENDPOINT AND DESIGN

Understanding and Overcoming the Challenges Involving Patients with Kidney Disease in Cardiovascular Trials

This project aims to understand the barriers to the inclusion of people living with kidney diseases in cardiovascular trials, with an emphasis on those with advanced CKD (stage 4) and ESRD, and to identify strategies for enhancing inclusion of these patients in cardiovascular trials. The workgroup gained community feedback on the challenges through a patient and care partner survey as well as a trial sponsors and stakeholders survey. The results of these surveys identified four areas that are considered barriers amongst the patient and stakeholder community which included: clinical trial endpoints and design, financial and feasibility challenges, patient engagement and the clinical trial culture in nephrology.



“

...Managing cardiovascular disease in people with kidney diseases is clinically important, but the evidence to support optimal treatment recommendations is limited by their exclusion from cardiovascular trials. Our project aimed to understand the challenges with involving people with kidney diseases in cardiovascular trials and identify actionable strategies to address them. Ultimately, our goal is to enhance the evidence base so that we can make more informed decisions when treating our patients. ”

- Julie Ishida, MD
Workgroup Co-Chair

Sixth Annual Stakeholders Meeting

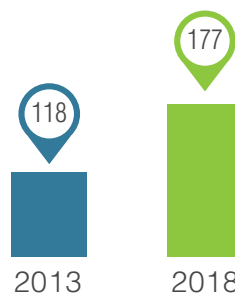


"Patients at the Center"
May 14–15, 2018
Silver Spring, MD

L-R: Prabir Roy-Chaudhury, MD, PhD, FASN, Janet Woodcock, MD, Bruce Greenstein, Pamela Duquette, and Paul Conway

Janet Woodcock, MD, Director of the Center for Drug Evaluation and Research (CDER) at the FDA was the keynote speaker and focused on the role of KHI in the regulatory process and the importance of including the patient perspective. Bruce Greenstein, former Chief Technology Officer at the U.S. Department of Health and Human Services discussed the newly launched KidneyX program, which should accelerate innovations and technologies into the hands of doctors and patients. Pamela Duquette, a member of the KHI Patient & Family Partnership Council highlighted the urgency for new therapies and treatments by sharing her experience as a parent of a child with kidney disease.

Attendance



Meeting Highlights

- Presentation from a teenage boy with a genetic kidney disease on hoping for the development of an artificial kidney
- Session on the potential impact of patient entrepreneurs
- Presentations on innovations in diabetic kidney disease
- Updates on KHI Projects
- Breakout sessions for future projects

THANK YOU TO THE 2018 PLANNING COMMITTEE

Katherine R. Tuttle, MD, FASN, Co-Chair
Paul T. Conway, Co-Chair
Richard D. Fissel
Kevin J. Fowler
Kristen Miller, PharmD; FDA Point of Contact
Larry Kessler, Sc.D.

Reshma Kewalramani, MD, FASN
Krishna Polu, MD
Marlon Pragnell, PhD
Prabir Roy-Chaudhury, MD, PhD, FASN
Linda Upchurch
Roberta L. Wager, MSN, RN

KHI Patient & Family Partnership Council



L-R: Richard D. Fissel, Amanda Grandinetti, Pamela M. Duquette, Roberta L. Wager, MSN, RN, David M. White (*Chair*), Terry F. Litchfield, Denise Eilers, BSN, RN, Nichole M. Jefferson, Kevin J. Fowler (*Vice Chair*), Caroline Wilkie

In 2015, the KHI Patient and Family Partnership Council (PFPC) was created to further patient involvement in KHI activities. The group achieves this by:

1. Helping KHI engage and network with other patients and patient organizations;
2. Identifying patients who are willing to participate in KHI workgroups and projects;
3. Advising industry and research partners of patient needs and preferences as new products are developed

The 10-member KHI PFPC is diverse, representing people who have been on dialysis and/or received a transplant or have cared for a family member with kidney disease.

KHI WOULD LIKE TO RECOGNIZE TEN PATIENT ORGANIZATIONS AND FOUNDATIONS FOR THEIR SUPPORT OF THE PFPC



2018 Highlights

- Participated in three workgroups
- Reviewed seven project proposals
- Attended twelve conferences
- Advised member organizations on patient involvement methods
- Met with Department of Health and Human Services Secretary Alex Azar



E-NEWSLETTER

The KHI PFPC E-Newsletter was created to assist in communicating and disseminating information on KHI PFPC activities. Edited by Richard Fissel, the first issue was released in May and will be distributed quarterly to KHI members and a broad group of patient networks.



KHI PFPC members meet with Department of Health and Human Services Secretary Alex Azar



2018 Board of Directors

Thank you to the KHI Board of Directors for its leadership and support.



2017–2018 Board of Directors,
Taken December 2017

Anupam Agarwal, MD, FASN

University of Alabama at Birmingham,
ASN Council Liaison

David Baron, PhD

PKD Foundation

Paul T. Conway

American Association of Kidney
Patients

Hampton Corley

Clinipace Worldwide

Mark Costanzo

Fresenius Medical Care North America

Ronald J. Falk, MD, FASN

University of North Carolina Kidney
Center

Martin Gerber

Medtronic, Inc

Barbara S. Gillespie, MD, FASN

Covance Global CRO

Markus Ketteler, MD

Klinikum Coburg GmbH, Germany

Mahesh Krishnan, MD, MPH, FASN

DaVita Healthcare Partners

Benjamin L. Laskin, MD

The Children's Hospital of Philadelphia

Kristen Miller, PharmD

Office of Medical Policy, CDER, FDA,
FDA Point of Contact

Joseph Muldoon

FAST BioMedical

Carolyn Y. Neuland, PhD

CDRH, FDA

Uptal D. Patel, MD

Gilead Sciences, Inc

Meda E. Pavkov, MD, PhD

Centers for Disease Control

Jesse Roach, MD

Centers for Medicare & Medicaid
Services

**Prabir Roy-Chaudhury, MD, PhD,
FASN**

University of Arizona,
ASN Co-chair for KHI

James P. Smith, MD, MS

CDER, FDA

Robert A. Star, MD

National Institute of Diabetes and
Digestive and Kidney Diseases, NIH

Wendy L. St. Peter, PharmD, FASN

University of Minnesota College of
Pharmacy

Aliza Thompson, MD

CDER, FDA

Katherine R. Tuttle, MD, FASN

University of Washington/Providence
Health Care

Katrin Uhlig, MD, MS

Keryx Biopharmaceuticals

Roberta L. Wager, MSN, RN

Boerne, TX

David White

Chair, KHI PFPC

Celia Witten, MD, PhD

CBER, FDA

Alex Yevzlin, MD, FASN

University of Michigan

KHI Members

One of the greatest strengths of KHI is its diverse and broad membership that includes all major stakeholders in the field of kidney diseases. KHI thanks its more than 90 members for their participation and support.

Achillion Pharmaceuticals, Inc.	Home Dialyzors United
Akebia Therapeutics, Inc.	Humacyte Inc.
Allena Pharmaceuticals, Inc.	InnAVasc Medical
Alnylam Pharmaceuticals	International Society for Peritoneal Dialysis
American Association of Kidney Patients	IQVIA
American College of Clinical Pharmacy- Nephrology Practice and Research Network	Janssen Research & Development, LLC
American Kidney Fund	JDRF International
American Nephrology Nurses Association	Karolinska Institute
American Society for Apheresis	Keryx Biopharmaceuticals, Inc.
American Society of Diagnostic and Interventional Nephrology	Kidney Disease: Improving Global Outcomes
American Society of Pediatric Nephrology	Kidney Research Institute at the University of Washington
American Society of Transplantation	Laminare Medical Technologies Ltd.
Amgen, Inc.	MediBeacon, LLC
Antaros Medical	Medtronic, Inc.
Arbor Research Collaborative for Health	National Institute of Diabetes and Digestive Kidney Diseases NIDDK
AstraZeneca	National Kidney Foundation
Avenu Medical, Inc.	National Renal Administrators Association
B. Braun Medical	NephCure Kidney International
Baxter Healthcare Corporation	Nephrology Nursing Certification Commission
Bayer AG	North American Pediatric Renal Trials and Collaborative Studies
BioPorto	NxStage Medical, Inc.
C. R. Bard, Inc.	Oxalosis and Hyperoxaluria Foundation
Calliditas Pharmaceuticals AB	OxThera AB
Center for Dialysis Innovation	PatientMpower
Centers for Disease Control, CDC	PeaceHealth Dialysis Center
Centers for Medicare and Medicaid Services, CMS	Pfizer, Inc.
ChemoCentryx Inc.	PKD Foundation
Clinipace Worldwide	Proteon Therapeutics, Inc.
Collaborative Study Group	pulseData
Corvidia Therapeutics	Relypsa, Inc.
Covance Global CRO	Renal Physicians Association
DaVita, Inc.	Retrophin, Inc.
Dialysis Clinic, Inc.	Sanofi US Services Inc., A Sanofi Company
Duke Clinical Research Institute	The George Institute
Dutch Kidney Foundation	The Rogosin Institute
European Renal Association-European Dialysis & Transplant Association	TVA Medical
Expedition Therapeutics	University of North Carolina Kidney Center
FAST BioMedical	Vascular Access Society
Food and Drug Administration, FDA	Vascular Access Society of the Americas
Foundation imec Netherlands	Vascular Therapies
Fresenius Medical Care	Vasculitis Foundation
Gilead Sciences, Inc.	Vertex Pharmaceuticals
Goldfinch Bio	Veterans Health Administration
GraftWorx	Visterra Inc.
Hansa Medical AB	W.L. Gore & Associates
	Wearable Artificial Organs, Inc.

The Kidney Health Initiative is part of the American Society of Nephrology.



KIDNEY HEALTH INITIATIVE

1510 H Street NW | Suite 800
Washington, DC 20005
khi@asn-online.org
www.kidneyhealthinitiative.org