



2017 ANNUAL REVIEW



KIDNEY HEALTH INITIATIVE

Kidney Health Initiative (KHI) is a public-private partnership founded in September 2012 by the American Society of Nephrology (ASN) and U.S. Food and Drug Administration (FDA).

KHI was formed with the mission “to advance scientific understanding of the 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 FDA and the greater nephrology community can interact to optimize evaluation of drugs, devices, biologics, and food products.”



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Dear Kidney Health Initiative Member,

On behalf of the Board of Directors, I would like to thank you for your participation in the Kidney Health Initiative (KHI). 2017 has been a prolific year. With your support, KHI has administered numerous collaborative projects with multiple stakeholders to advance treatments and technologies for kidney-related diseases. We have also leveraged discussions and research in different settings to identify ways to develop safe, effective, and patient-centric therapies. We are thrilled to celebrate five years of progress in the fight against kidney diseases. We are especially grateful for the support and involvement of the U.S. Food and Drug Administration (FDA); they have been a true partner.

“We are thrilled to celebrate five years of progress in the fight against kidney diseases.”

Our Annual Stakeholders Meeting in May featured a keynote address by Eric Dishman, Director of National Institutes of Health's (NIH) *All of Us* Research Program, as well as presentations from Robert Star, MD, on the National Institute of Diabetes and Digestive and Kidney Diseases' Kidney Precision Medicine Project and Katherine Donigan, PhD, on the FDA's perspective on Next-Gen Sequencing. Thank you to everyone who was able to attend. Please save the date for the Sixth Annual KHI Stakeholders Meeting on May 14–15, 2018.

We would also like to acknowledge and thank our workgroup members for their effort and commitment. A number of projects closed out this year, resulting in workshops and white papers that we hope will facilitate the future passage of drugs, devices and biologics for the treatment of kidney diseases. We are also excited about three new projects that have just been endorsed which will focus on clinical trial endpoints for focal segmental glomerulosclerosis (FSGS) and hyperoxaluria; and on fluid management in hemodialysis. These highlights and others are featured on page 6.

KHI continues to grow since its establishment in September 2012. There are now over 80 members representing all the diverse stakeholders in the kidney community, including patient organizations, healthcare professional organizations, industry partners (pharmaceutical, biotechnology, device manufacturers, and dialysis organizations), research institutions, and federal agencies. The membership has been actively involved on multiple KHI projects to improve patient safety and promote the development of optimal therapies for kidney diseases that affect the quality of life of our patients.

As KHI continues to flourish, your continued engagement is invaluable. We appreciate your input on the project proposals submitted during our fall and spring submission cycles. Additionally, there are opportunities to become more active in KHI by serving on a committee or workgroup, or through a nomination to the Board of Directors. Please continue to share your KHI experiences with the community and prospective members.

Finally, I want to emphasize again that our greatest strength and asset is, without question, our membership. KHI is the platform where all the different stakeholders (all of you) can come together to focus on both innovation and patient safety; and by doing so, I truly believe we can make a difference for the millions suffering from kidney diseases.

Thank you again for all of your efforts and thank you for your leadership.



Sincerely,

Prabir Roy-Chaudhury

Prabir Roy-Chaudhury, MD, PhD, FASN

KHI Co-Chair, American Society of Nephrology

On behalf of the KHI Board of Directors

2017 Highlights

Fifth Annual Stakeholders Meeting

In May, our diverse membership gathered to focus on the emerging opportunity for personalized medicine to enhance kidney health.

Eric Dishman, Director, *All of Us Research Program* at NIH, outlined the initiative's dynamic cycle: patients contribute their biomedical data to a diverse field of researchers, who then use that information to accelerate medical breakthroughs for patients.

Robert Star, MD, Director, NIDDK described the Kidney Precision Medicine Program and how development of a Kidney Tissue Atlas from kidney biopsies can improve scientific knowledge of kidney disease, improve treatments, and recruit scientists into nephrology.

Katherine Donigan, PhD, Personalized Medicine Staff at FDA's Center for Devices and Radiological Health provided an overview of the regulatory challenges associated with Next-Gen Sequencing tests and precision medicine.



Preparing for the Opening Keynote during Fifth Annual KHI Stakeholders Meeting - Dr. Star, Mr. Dishman, Dr. Katherine Tuttle, and Dr. Prabir Roy-Chaudhury

Collaborating for Progress

In our ongoing commitment to advancing therapies, we brought diverse stakeholders together to address specific pre-competitive issues in product development. Their efforts gave rise to publications introducing state-of-the-art scientific models for use in future research:

- Recommended Clinical Trial Endpoints for Dialysis Catheters
- Definitions and Endpoints for Interventional Studies for Arteriovenous Dialysis Access
- FDA Regulatory Perspectives for Studies on Hemodialysis Vascular Access
- CDISC Therapeutic Area Data Standards User Guide for Diabetic Kidney Disease
- CDISC Therapeutic Area Data Standards User Guide for Kidney Transplant

Full text versions of these publications can be found at www.kidneyhealthinitiative.org.

The Voice of the Patient

Patients with kidney diseases are at the heart of KHI's mission. Through a variety of channels, we seek their perspective. Patient input was profoundly instrumental in two publications this year:

- 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

Innovative Alternatives to Renal Replacement Therapy (RRT): Developing a Roadmap

In spring of 2017, KHI launched a far-reaching initiative. The RRT Roadmap takes a big-picture approach to renal replacement therapy options. The goal is to design a roadmap that streamlines innovation of a bioartificial or bioengineered alternative to dialysis.

The project is complex, but the objective is simple. We are working to provide a catalyst for industry, academia, and other organizations to invest in RRT alternatives.

Strategic Priority Areas and Projects

KHI serves as a platform to enhance patient safety and overcome barriers to innovation in nephrology through its portfolio of collaborative, member-driven projects. KHI is currently administering nine projects that encompass a number of key priority areas.

A complete list of KHI projects can be found on pages 8-9.

Advancement of Patient and Family Partnership and Funding of Kidney Diseases

- Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions
- Fostering Innovation in Fluid Management

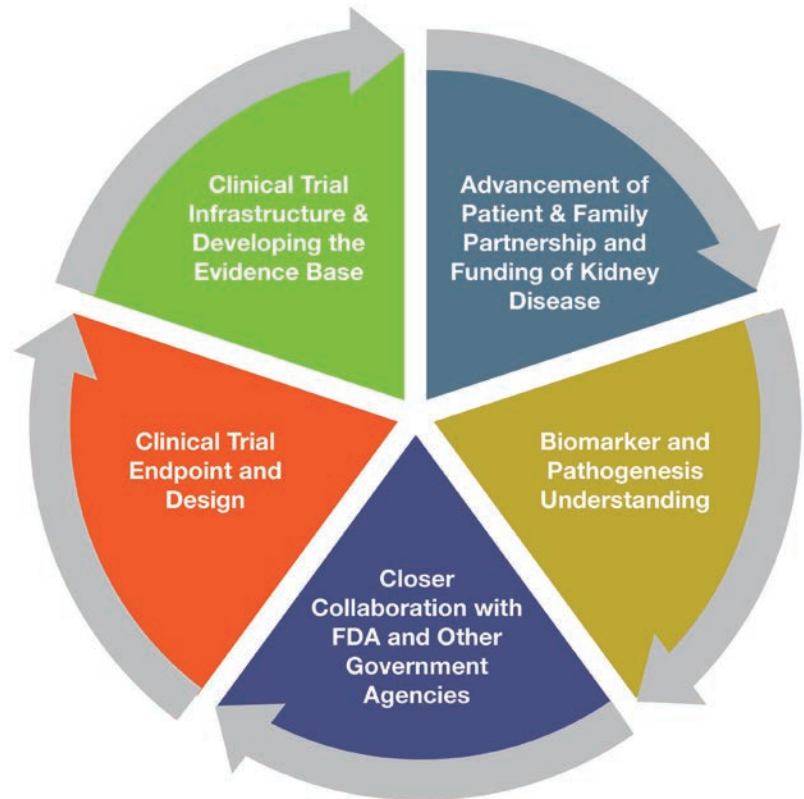
Closer Collaboration with the FDA and Other Government Agencies

- Development of a Roadmap for Innovations in Renal Replacement Therapy
- Overcoming Barriers to Drug Development in Children with CKD

Clinical Trial Endpoint and Design

- Outcome Measures in Lupus Nephritis
- Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy
- Understanding and Overcoming the Exclusion of Patients with Kidney Disease from Cardiovascular Trials
- Surrogate Endpoints for Focal Segmental Glomerulosclerosis (FSGS)
- Identification of Appropriate Endpoints for Clinical Trials in Hyperoxaluria

Strategic Priorities Areas¹



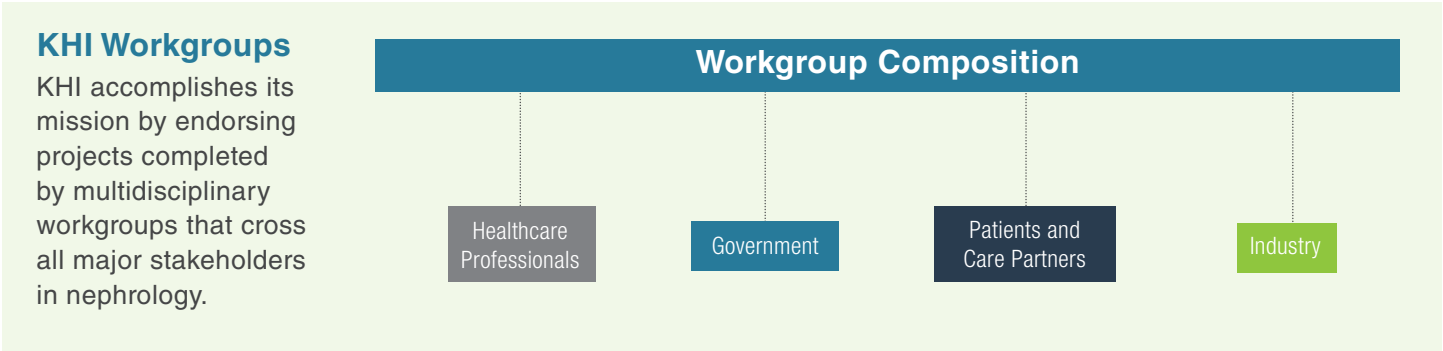
1] Linde P, Archdeacon P, Breyer M, et al: Overcoming Barriers in Kidney Health – Forging a Platform for Innovation. J Am Soc Nephrol 27: 1902-1910, 2016

Workgroup Leadership

The membership has worked diligently through KHI to improve patient safety and promote the development of optimal therapies for diseases that affect kidneys and the quality of life for millions of people.

The KHI Board of Directors would like to thank the chairs of each project workgroup for their leadership, hard work, and effort this past year.

Rita Alloway, PharmD, FCCP University of Cincinnati (Standards in Transplantation)	Richard Haynes, DM, MRCP Clinical Trial Service Unit and Epidemiological Studies Unit, University of Oxford (Standards in Diabetic Kidney Disease)	Patrick Nachman, MD, FASN University of North Carolina Kidney Center (Surrogate Endpoints for IgAN)
Stephen R. Ash, MD, FACP Indiana University, HemoCleanse, Inc., and Ash Access Technology, Inc. (CDRH Regulatory Policies)	Charles Herzog, MD, DACC, FAHA University of Minnesota (Cardiovascular Trials)	Brad Rovin, MD, FACP, FASN The Ohio State University (Lupus Nephritis)
Joseph V. Bonventre, MD, PhD, FASN Brigham and Women's Hospital (RRT Roadmap)	Julie Ishida, MD, MAS University of California, San Francisco (Cardiovascular Trials)	Surendra Shenoy, MD, PhD Washington University (Vascular Access)
Daniel C. Brennan, MD Washington University (Standards in Transplantation)	Susie Lew, MD George Washington University (Remote Patient Monitoring)	Douglas M. Silverstein, MD Center for Devices and Radiological Health (CDRH), FDA (CDRH Regulatory Policies)
Patrick Brophy, MD University of Iowa (Remote Patient Monitoring)	Roslyn B. Mannon, MD, FASN University of Alabama at Birmingham (Standards in Transplantation)	Aliza Thompson, MD Center for Drug Evaluation and Research (CDER), FDA (Surrogate Endpoints for IgAN)
Jennifer E. Flythe, MD, MPH, FASN University of North Carolina Chapel Hill (Symptoms of ESRD Patients)	Rajnish Mehrotra, MD, FASN University of Washington (Symptoms of ESRD Patients)	Katrin Uhlig, MD Keryx Biopharmaceuticals, Inc. (Pediatric Drug Development)
Stuart Goldstein, MD Cincinnati Children's Hospital Medical Center (Pediatric Drug Development)	Amy K. Mottl, MD, MPH University of North Carolina Kidney Center (Standards in Diabetic Kidney Disease)	Monnie Wasse, MD, MPH, FASN Rush University (Vascular Access)



Project Portfolio

KHI maintains a portfolio of projects in different stages of development. This portfolio aims to increase patient safety and foster the creation of novel therapies for diseases that affect the kidney.

Projects Listed by Categories	Concept
Advancement of Patient and Family Partnership and Funding of Kidney Diseases	
Advancing Technologies to Facilitate Remote Management of Patient Self-Care in Renal Replacement Therapy	●
Workshop to Elucidate Role of Patient Preferences in Support of CDRH Regulatory Actions in Kidney Disease	●
Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions	●
Fostering Innovation in Fluid Management	●
Closer Collaboration with the FDA and Other Government Agencies	
Promoting Kidney Health and Innovative Treatments for Kidney Disease: Barriers and Potential Solutions	●
Regulatory Policies and Positions Affecting Device and Drug Approval in the US	●
Overcoming Barriers to Drug Development in Children with CKD	●
Development of a Roadmap for Innovations in Renal Replacement Therapy (RRT)	●
Clinical Trial Endpoint and Design	
Pharmacokinetics in Patients Receiving Continuous Renal Replacement Therapy (CRRT)	●
Pragmatic Trials in Dialysis: Challenges and Opportunities	●
Clinical Trial Endpoints for Dialysis Vascular Access	●
Outcome Measures in Lupus Nephritis	●
Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy	●
Understanding and Overcoming the Exclusion of Patients with Kidney Disease from Cardiovascular Trials	●
Surrogate Endpoints for Focal Segmental Glomerulosclerosis (FSGS)	●
Identification of Appropriate Endpoints for Clinical Trials in Hyperoxaluria	●
Clinical Trial Infrastructure and Developing the Evidence Base	
Data Harmonization in Kidney Transplant	●
Data Standards in Diabetic Kidney Disease	●

Stage 1				Stage 2			Stage 3	
Concept Endorsed	Call for Workgroup	Workgroup Selected	Project Plan Finalized	Beginning	Middle	Near Completion	Completed	Evaluation / Assessment of Project Completed
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Patient and Family Partnership Council (PFPC)

In 2015, KHI created the Patient and Family Partnership Council. As an advisory body, the KHI PFPC brings the perspective of patients, their families, and care partners to all significant KHI endeavors.

The KHI PFPC works closely with KHI's Board of Directors and staff to:

Help KHI engage and network with other patients and patient organizations across the spectrum of kidney disease

Identify patients willing to participate in KHI workgroups and projects

Advise industry and research partners of the needs and preferences of patients to consider as they develop new products

Current members of the KHI PFPC:

David M. White, *Chair*

Kevin J. Fowler, *Vice Chair*

Pamela M. Duquette

Denise Eilers, BSN, RN

Richard D. Fissel

Nichole M. Jefferson

Terry F. Litchfield

Roberta L. Wager, MSN, RN

Caroline Wilkie



KHI PFPC members and staff at the FDA Patient Focused Drug Development Meeting, Sept. 2016

Thank you for your support

KHI would like to thank and acknowledge nine member organizations for their support of the PFPC:



In Memoriam



Celeste Castillo Lee

KHI Board of Directors Member and Chair of the KHI Patient and Family Partnership Council (2012-2017)

It is with great sadness that we announce the passing of Celeste Castillo Lee on Thursday, February 9, 2017. Ms. Lee worked tirelessly as a patient and care partner advocate and was an amazing and outstanding leader in patient engagement.

For more than 30 years, Celeste Castillo Lee lived with kidney failure. She had been on both peritoneal dialysis and hemodialysis, and had a transplant (for 10 years). Ms. Lee was committed to helping health systems, academic health centers, the biotechnology and pharmaceutical industries, and dialysis organizations partner with patients and their families to re-envision the future of health care. She served as a patient advisor, peer mentor and advocate in non-profit health organizations, governmental agencies, and research projects both nationally and internationally.

Ms. Lee was a friendly and familiar face at ASN Kidney Week, the Patient-Centered Outcomes Research Institute (PCORI), and other local and national events. Among other leadership roles, she served as a member of the KHI Board of Directors and as the Founding Chair of the KHI Patient & Family Partnership Council. Ms. Lee was awarded the 2017 Medal of Excellence by the American Association of Kidney Patients. Her tireless efforts reverberated throughout the nephrology community, and out across the entire healthcare sector. Her loss is profoundly felt by the community.

To honor her contributions and involvement in patient engagement, ASN is seeking to establish a Celeste Castillo Lee Endowed Lectureship. Held annually during ASN Kidney Week, the lectureship would provide an opportunity to recognize the patient as an integral member of the research and clinical team.

“Once patients are educated and engaged, they become empowered.”

▪ Celeste Castillo Lee

Board of Directors

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

A special thanks to members of the Board of Directors who complete their term in 2017:

Matthew D. Breyer, MD, PhD, FASN, Celeste Castillo Lee, Michael F. Flessner, MD, PhD, Stuart Goldstein, MD, Julia K. Inrig, MD, FASN, Reshma Kewalramani, MD, FASN, Peter G. Linde, MD, FASN and James A. Sloand, MD, FASN.

David Baron , PhD	PKD Foundation
Matthew D. Breyer , MD, FASN	Eli Lilly and Company
Paul T. Conway	American Association of Kidney Patients
Mark Costanzo	Fresenius Medical Care North America
Ronald J. Falk , MD, FASN	University of North Carolina Chapel Hill
Stuart L. Goldstein , MD	Cincinnati Children's Hospital Medical Center
Julia K. Inrig , MD, FASN	Quintiles/ IMS
Markus Ketteler , MD	Klinikum Coburg
Reshma Kewalramani , MD, FASN	Vertex Pharmaceuticals
Mahesh Krishnan , MD, MPH, MBA, FASN	DaVita Healthcare Partners
Peter G. Linde , MD, FASN	Accelaron Pharma, Inc.
Andrew S. Narva , MD, FASN	National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)
Kristen Miller , PharmD, FDA Point of Contact	Center for Drug Evaluation and Research (CDER), FDA
Joseph Muldoon	FAST Biomedical, Inc.
Carolyn Y. Neuland , PhD	Center for Devices and Radiological Health (CDRH), FDA
Meda E. Pavkov , MD, PhD	Epidemiology and Statistics Branch (ESB), Centers for Disease Control (CDC)
Jesse Roach , MD	Centers for Medicare and Medicaid Services
Prabir Roy-Chaudhury , MD, PhD, FASN <i>ASN Co-Chair</i>	University of Arizona
James A. Sloand , MD, FASN	Baxter International Inc.
James P. Smith , MD, MS	Center for Drug Evaluation and Research (CDER), FDA
Wendy L. St. Peter , PharmD, FASN	University of Minnesota
Aliza Thompson , MD	Center for Drug Evaluation and Research (CDER), FDA
Katherine R. Tuttle , MD, FASN, FACP	University of Washington
Roberta L. Wager , MSN, RN	Boerne, TX
David M. White <i>KHI Patient and Family Partnership Council Chair</i>	Hillcrest Heights, MD
Celia Witten , PhD, MD	Center for Biologics Evaluation and Research (CBER), FDA
Alexander S. Yevzlin , MD, FASN	University of Michigan

The KHI Nominating Committee holds an annual open call for nominations to the KHI Board of Directors each summer.

Leadership and Staff

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SAVE THE DATE

Sixth Annual KHI Stakeholders Meeting

May 14–15, 2018 | Silver Spring, Maryland

For more information visit, www.kidneyhealthinitiative.org.

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 80 members for their participation and support.







KIDNEY HEALTH INITIATIVE

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