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.

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, MD, PhD, FASN
KHI Co-Chair, American Society of Nephrology
On behalf of the KHI Board of Directors
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.

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](http://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

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)

Stephen R. Ash, MD, FACP
Indiana University, HemoCleanse, Inc., and Ash Access Technology, Inc.
(CDRH Regulatory Policies)

Joseph V. Bonventre, MD, PhD, FASN
Brigham and Women’s Hospital
(RRT Roadmap)

Daniel C. Brennan, MD
Washington University
(Standards in Transplantation)

Patrick Brophy, MD
University of Iowa
(Remote Patient Monitoring)

Jennifer E. Flythe, MD, MPH, FASN
University of North Carolina Chapel Hill
(Symptoms of ESRD Patients)

Stuart Goldstein, MD
Cincinnati Children’s Hospital Medical Center
(Pediatric Drug Development)

Richard Haynes, DM, MRCP
Clinical Trial Service Unit and Epidemiological Studies Unit, University of Oxford
(Standards in Diabetic Kidney Disease)

Charles Herzog, MD, DACC, FAHA
University of Minnesota
(Cardiovascular Trials)

Julie Ishida, MD, MAS
University of California, San Francisco
(Cardiovascular Trials)

Susie Lew, MD
George Washington University
(Remote Patient Monitoring)

Roslyn B. Mannon, MD, FASN
University of Alabama at Birmingham
(Standards in Transplantation)

Rajnish Mehrotra, MD, FASN
University of Washington
(Symptoms of ESRD Patients)

Amy K. Mottl, MD, MPH
University of North Carolina Kidney Center
(Standards in Diabetic Kidney Disease)

Patrick Nachman, MD, FASN
University of North Carolina Kidney Center
(Surrogate Endpoints for IgAN)

Brad Rovin, MD, FACP, FASN
The Ohio State University
(Lupus Nephritis)

Surendra Shenoy, MD, PhD
Washington University
(Vascular Access)

Douglas M. Silverstein, MD
Center for Devices and Radiological Health (CDRH), FDA
(CDRH Regulatory Policies)

Aliza Thompson, MD
Center for Drug Evaluation and Research (CDER), FDA
(Surrogate Endpoints for IgAN)

Katrin Uhlig, MD
Keryx Biopharmaceuticals, Inc.
(Pediatric Drug Development)

Monnie Wasse, MD, MPH, FASN
Rush University
(Vascular Access)

KHI Workgroups
KHI accomplishes its mission by endorsing projects completed by multidisciplinary workgroups that cross all major stakeholders in nephrology.

KHI Workgroup Composition

- Healthcare Professionals
- Government
- Patients and Care Partners
- Industry
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.

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

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*
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, Jula K. Inrig, MD, FASN, Reshma Kewalramani, MD, FASN, Peter G. Linde, MD, FASN and James A. Sloand, MD, FASN.

David Baron, PhD
Matthew D. Breyer, MD, FASN
Paul T. Conway
Mark Costanzo
Ronald J. Falk, MD, FASN
Stuart L. Goldstein, MD
Jula K. Inrig, MD, FASN
Markus Ketteler, MD
Reshma Kewalramani, MD,FASN
Mahesh Krishnan, MD, MPH, MBA, FASN
Peter G. Linde, MD, FASN
Andrew S. Narva, MD, FASN
Kristen Miller, PharmD, FDA Point of Contact
Joseph Muldoon
Carolyn Y. Neuland, PhD
Meda E. Pavkov, MD, PhD
Jesse Roach, MD
Prabir Roy-Chaudhury, MD, PhD, FASN
ASN Co-Chair
James A. Sloand, MD, FASN
James P. Smith, MD, MS
Wendy L. St. Peter, PharmD, FASN
Aliza Thompson, MD
Katherine R. Tuttle, MD, FASN, FACP
Robert L. Wager, MSN, RN
David M. White
KHI Patient and Family Partnership Council Chair
Celia Witten, PhD, MD
Alexander S. Yevzlin, MD, FASN

PKD Foundation
Eli Lilly and Company
American Association of Kidney Patients
Fresenius Medical Care North America
University of North Carolina Chapel Hill
Cincinnati Children’s Hospital Medical Center
Quintiles/ IMS
Klinikum Coburg
Vertex Pharmaceuticals
DaVita Healthcare Partners
Acceleron Pharma, Inc.
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)
Center for Drug Evaluation and Research (CDER), FDA
FAST Biomedical, Inc.
Center for Devices and Radiological Health (CDRH), FDA
Epidemiology and Statistic Branch (ESB), Centers for Disease Control (CDC)
Centers for Medicare and Medicaid Services
University of Arizona
Baxter International Inc.
Center for Drug Evaluation and Research (CDER), FDA
University of Minnesota
Center for Drug Evaluation and Research (CDER), FDA
University of Washington
Boerne, TX
Hillcrest Heights, MD
Center for Biologics Evaluation and Research (CBER), FDA
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

Co-Chair
Prabir Roy-Chaudhury, MD, PhD, FASN
University of Arizona College of Medicine
Email: proychaudhury@deptofmed.arizona.edu

FDA Point of Contact
Kristen Miller, PharmD
Center for Drug Evaluation and Research (CDER), FDA
Email: Kristen.Miller@fda.hhs.gov

Staff
Melissa West
Project Director
Kidney Health Initiative
Email: mwest@asn-online.org

Rick Kempinski
Manager
Kidney Health Initiative
Email: rkempinski@asn-online.org

Elle Silverman
Project Associate
Kidney Health Initiative
Email: esilverman@asn-online.org

SAVE THE DATE
Sixth Annual KHI Stakeholders Meeting
May 14–15, 2018 | Silver Spring, Maryland

For more information visit, www.kidneyhealthinitiative.org.
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.