


Annual Review 2016



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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Pioneer and General Members

One of the greatest strengths of KHI is its diverse and broad membership, including all major stakeholders in the field of kidney diseases. KHI thanks its more than 75 members, especially its Pioneer members, for their participation and support.



Leading European Nephrology

Pioneer and General Members



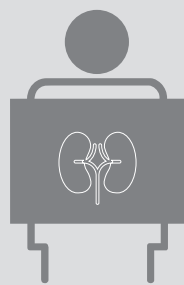
Kidney Diseases Statistics

Kidney Diseases



More than **20 million**¹ Americans have kidney disease, the ninth leading cause of death in the United States in 2014².

Chronic Kidney Disease (CKD)



Overall prevalence of CKD in the general population is approximately 14%³.

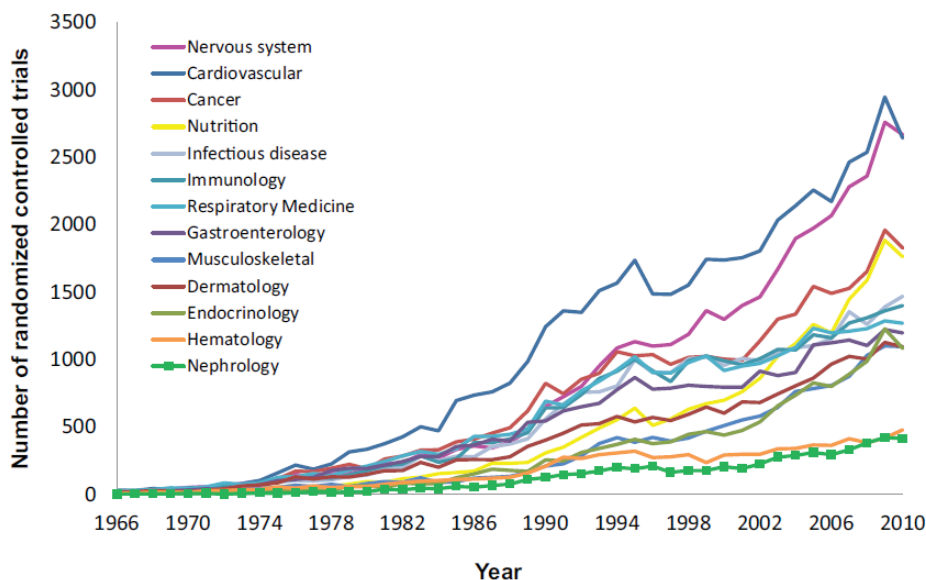
Patient awareness is less than 10% for those with CKD⁴.

Kidney Failure



Approximately **100,000** patients are currently on the wait-list for a kidney transplant⁵.

Therapeutic Pipeline⁶



Nephrology publishes the lowest number of randomized clinical trials among the internal medicine specialties.

References

- 1] <http://www.cdc.gov/diabetes/pubs/factsheets/kidney.htm>, Centers for Disease Control and Prevention (CDC). National Chronic Kidney Disease Fact Sheet: General Information and National Estimates on Chronic Kidney Disease in the United States, 2014. Atlanta, GA: US Department of Health and Human Services, Centers for Disease Control and Prevention; 2014.
- 2] <http://www.cdc.gov/nchs/fastats/deaths.htm>
- 3] https://www.usrds.org/2015/view/v1_01.aspx (Chp 1 CKD, USRDS)
- 4] https://www.usrds.org/2015/view/v1_00.aspx
- 5] <https://optn.transplant.hrsa.gov/data/view-data-reports/national-data/#>
- 6] Palmer et al, AJKD 2011. Vol. 58, Issue 3, p. 335
- 7] 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.

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.

Since its establishment in 2012, KHI has initiated 15 projects that encompass a number of key priority areas.

Advancement of Patient and Family Partnership and Funding of Kidney Diseases

- Workshop to Elucidate Role of Patient Preferences in Support of CDRH Regulatory Actions in Kidney Disease
- Advancing Technologies to Facilitate Remote Management of Patient Self-Care in Renal Replacement Therapy
- Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions

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 Approval in the US: Tools to Assess the Process and Foster Device Development for Patients with Kidney Disease
- Development of a Roadmap for Innovations in Renal Replacement Therapy
- Overcoming Barriers to Drug Development in Children with CKD

Clinical Trial Endpoint and Design

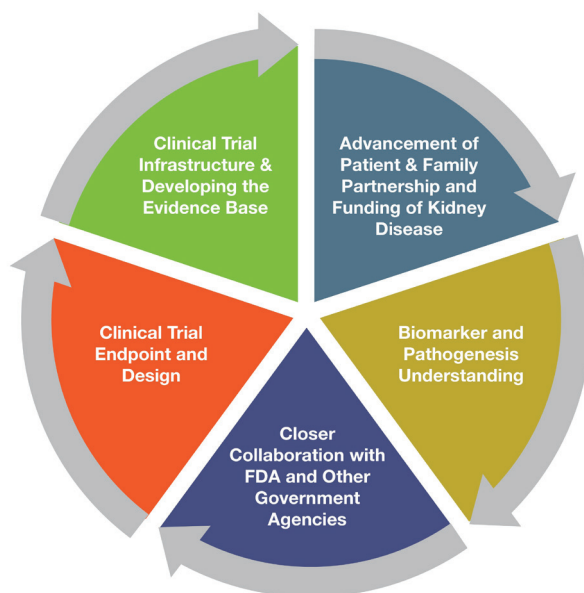
- Pharmacokinetics in Patients Receiving Continuous Renal Replacement Therapy
- Outcome Measures in Lupus Nephritis
- Clinical Trial Endpoints for Dialysis Vascular Access
- Pragmatic Trials in Dialysis: Challenges and Opportunities
- Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy
- Understanding and Overcoming the Exclusion of Patients with Kidney Disease from Cardiovascular Trials

Clinical Trial Infrastructure and Developing the Evidence Base

- Data Harmonization in Kidney Transplant
- Data Standards in Diabetic Kidney Disease

KHI Innovation⁷

To disrupt the stagnation of innovation in nephrology, the KHI “Promoting Kidney Health and Innovative Treatments for Kidney Disease: Barriers and Potential Solutions” workgroup developed the framework for KHI’s strategic priorities illustrated below.



2016 Highlights

Highlights from KHI are included below and more detailed updates on projects and other KHI activities are available in KHI's Project Portfolio or on the KHI website at www.kidneyhealthinitiative.org.

Completed Projects

KHI acknowledges and congratulates two workgroups for publishing their deliverables in the *Journal of the American Society of Nephrology*:

“Overcoming Barriers in Kidney Health – Forging a Platform for Innovation,” which identified five strategic priority areas for KHI and its future projects.

“Pragmatic Trials in Maintenance Dialysis: Perspectives from the Kidney Health Initiative” which presented the potential use of pragmatic trials to answer important clinical questions and recommended solutions to operational, regulatory, and business issues that may hinder the conduct of clinical trials in the provision of clinical care.

New Projects

KHI looks forward to working on the following new projects that were endorsed by the KHI Board of Directors in the past year:

- Development of a Roadmap for Innovations in Renal Replacement Therapy
- Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy
- Overcoming Barriers to Drug Development in Children with CKD
- Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions
- Understanding and Overcoming the Exclusion of Patients with Kidney Disease from Cardiovascular Trials

Patients & Family

The Patient and Family Partnership Council has continued to ensure that the patient's voice is maintained throughout KHI by:

- Designing and leading the “Patient and Care Partner Engagement: Gaining Wisdom through Collaboration” session at the Fourth Annual KHI Stakeholders Meeting in May 2016;
- Participating in the FDA's Patient Focused Drug Development Meeting for Patients Who Have Received an Organ Transplant on September 27, 2016;
- Contributing to the following KHI projects
 - Clinical Trial Endpoints for Dialysis Vascular Access
 - Overcoming Barriers to Drug Development in Children with CKD
 - Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions
 - Workshop to Elucidate Role of Patient Preferences in Support of CDRH Regulatory Actions in Kidney Disease

Patient and Family Partnership Council (PFPC)

PFPC was established in 2015 to work with the KHI Board of Directors and staff to advise on KHI's projects and overall efforts so that the patient's voice, experience, and involvement in KHI workgroups is meaningful and effective.

PFPC has been charged with:

1. Advising KHI members regarding patient involvement in their project proposals
2. Outlining opportunities for patients to serve once a project has been endorsed
3. Identifying patients to serve on project workgroups
4. Collaborating on developing patient centered project(s) to submit for KHI endorsement

Current members of the PFPC and their terms are listed below:

Celeste Castillo Lee, Chair and Liaison to KHI Board of Directors (2018)

Pamela M. Duquette (2018)

Denise Eilers, BSN, RN (2017)

Richard D. Fissel (2016)

Kevin J. Fowler (2016)

Nichole M. Jefferson (2018)

Terry F. Litchfield (2016)

Roberta L. Wager, MSN, RN (2017)

David M. White, Vice-Chair (2018)

Caroline Wilkie (2017)

PFPC would like to acknowledge Sam Pederson, who rotated off the PFPC in 2016, and thank him for his efforts in helping establish the Council.

Thank you for your support

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



Project and Workgroup Selection

To maintain its portfolio of innovative, collaborative, and member-driven projects, KHI administers an inclusive project submission process.

KHI members are involved in the execution of a variety of endorsed projects by co-authoring white papers; collaborating to leverage previously conducted and ongoing clinical studies, research infrastructure, and databases; and promoting dialogue on kidney health pertaining to drug, device, biologics, as well as food product development, evaluation, and safety.



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)

Daniel C. Brennan, MD

Washington University
(Standards in Transplantation)

Patrick Brophy, MD

University of Iowa
(Remote Patient Monitoring)

Dolph Chianchiano, JD, MPH

KHI Board of Directors
National Kidney Foundation
(Patient Preferences)

Laura M. Dember, MD

University of Pennsylvania
(Pragmatic Trials in Dialysis)

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, American Society of Pediatric
Nephrology
(Pediatrics with CKD)

Richard Haynes, DM, MRCP

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

Frank Hurst, MD, FASN

Center for Devices and Radiological
Health (CDRH), FDA
(Patient Preferences)

Susie Lew, MD

George Washington University
(Remote Patient Monitoring)

Peter G. Linde, MD, FASN

KHI Board of Directors
Accelaron Pharma, Inc.
(Barriers to Innovation)

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 Chapel Hill
(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.
(Pediatrics with CKD)

Monnie Wasse, MD, MPH, FASN

Rush University
(Vascular Access)

KHI accomplishes its mission by endorsing projects completed by multidisciplinary workgroups that cross all major stakeholders in nephrology. The composition of all of KHI's active workgroups is depicted below.

WORKGROUP COMPOSITION



103
HEALTHCARE
PROFESSIONAL



34
INDUSTRY



30
GOVERNMENT



7
PATIENTS, PATIENT
ORGANIZATIONS,
FOUNDATIONS

Milestones

KHI is proud of the accomplishments and deliverables since its establishment four years ago. With more than 75 members, the initiation of 15 projects, and the establishment of a Patient and Family Partnership Council, KHI strives to meet its mission of fostering innovation and enhancing patient safety for people with kidney diseases.

2012



KIDNEY HEALTH INITIATIVE

KHI Established September 2012

Ronald J. Falk, MD, FASN (former ASN President) and Margaret Hamburg, MD (former FDA Commissioner) sign a Memorandum of Understanding (MOU) between ASN and FDA to establish KHI.

2013

Pilot Projects Endorsed March 2013

- Outcome Measures in Lupus Nephritis
- Pharmacokinetics in Patients Receiving Continuous Renal Replacement Therapy
- Promoting Kidney Health and Innovative Treatments for Kidney Disease: Barriers and Potential Solutions

2013



"Fostering Innovation, Advancing Patient Safety: The Kidney Health Initiative" Publication September 2013

KHI leadership describe the mission of KHI to strive to foster innovation in therapies while ensuring patient safety through an article in the *Clinical Journal of the American Society of Nephrology*.

2013



First Annual Stakeholders Meeting – September 11, 2013

The keynote address was delivered by Margaret Hamburg, MD (Former FDA Commissioner) and Douglas Throckmorton, MD (Deputy Center Director for Regulatory Programs, Center for Drug Evaluation and Research, FDA). Closing remarks provided by Barry M. Straube, MD (former Chief Medical Officer for CMS).

2013

First Member Proposed Projects Endorsed October 2013

- Clinical Trial Endpoints for Dialysis Vascular Access
- Workshop to Elucidate Role of Patient Preferences in Support of CDRH Regulatory Actions in Kidney Disease

2013



61 Pioneer Members Join KHI December 31, 2013

Designation received by member organization that joined KHI by December 31, 2013.

2014

Second Annual Stakeholders Meeting – June 11-12, 2014

Mark B. McClellan, MD, PhD (Senior Fellow and Director of the Health Care Innovation and Value Initiative at the Brookings Institution, and former FDA Commissioner, and former Administrator of CMS) delivered the keynote speech.

2014

KHI Awarded 5-year Renewable FDA R18 Grant September 2014

2014

Standardized Pharmacokinetics Assessment in Patients Receiving CRRT Workshop November 11, 2014

Workshop presented recommendations of a KHI workgroup for the standardized assessment of pharmacokinetics and development of corresponding drug dosing recommendations in critically ill patients with AKI receiving CRRT.

2015

"Pharmacokinetic Assessment in Patients Receiving Continuous RRT: Perspectives from the Kidney Health Initiative" Publication January 2015

KHI workgroup's manuscript published in CJASN and evaluated key considerations in the assessment of pharmacokinetics and drug dosing in CRRT and the constraints to conducting pharmacokinetic studies in critically ill patients.

2015



Animated Video Released to Encourage Patient Involvement – March 2015

KHI workgroup releases an animated video that features a kidney patient encouraging individuals to attend one of three educational webinars hosted in April in 2015.

2015



Series of Educational Webinars for Patients Held April 2015

A KHI workgroup hosted three webinars to educate patients on the product lifecycle and the role of the FDA, specifically the Center for Device and Radiological Health, while increasing patients' interest in participating in a workshop held in August 2015.

2015

Patient and Family Partnership Council Established May 2015

To further patient involvement in KHI, the PFPC was created to work closely with the KHI Board of Directors and staff in advising and making recommendations on member project proposals, KHI projects and overall efforts of KHI.

2015

Third Annual Stakeholders Meeting – May 20-21, 2015

The keynote presentations were given by Robert Temple, MD (Deputy Director for Clinical Science, Center for Drug Evaluation and Research, FDA), Julia K. Inrig, MD, FASN (Global Medical Director and Therapeutic Strategy Lead in Nephrology for Quintiles Global CRO) and Robert Califf, MD (previously with Duke University, current FDA Commissioner).

2015



Understanding Patient Preferences Workshop August 12-13, 2015

The workshop convened the FDA, clinicians, device industry, and patients, care partners and patient advocacy groups to discuss patient preferences and how patients can influence device design, assist in the planning and implementation of clinical trials.

2015

Term of Inaugural Board of Directors Ends December 2015

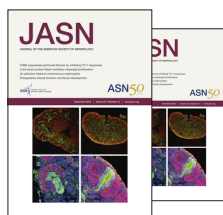
2016



Fourth Annual Stakeholders Meeting – May 25-26, 2016

KHI's Annual Stakeholder Meeting continues to grow and attract diverse participants from across the KHI membership. Of this year's meeting participants, nearly one-third represented FDA and government agencies, one-third were affiliated with industry, and one-third represented patients and health professionals.

2016



"Overcoming Barriers in Kidney Health – Forging a Platform for Innovation" Publication July 2016

A KHI workgroup's manuscript describing the strategic priorities for KHI and future projects was published in the Journal of the American Society of Nephrology (JASN).

2016

"Pragmatic Trials in Maintenance Dialysis: Perspectives from the Kidney Health Initiative" e-Publication July 2016

A KHI workgroup's manuscript was e-published ahead of print and presented the potential use of pragmatic trials to answer important clinical questions and recommended solutions to barriers that may hinder the conduct of clinical trials in the provision of clinical care in JASN.

2017



Fifth Annual Stakeholders Meeting in Silver Spring, MD May 24-25, 2017

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 completed their term in 2016 – Patrick Archdeacon, MD, Dolph Chianchiano, JD, MPA, and Alan Kliger, MD. In particular, we would like to acknowledge Founding Co-Chair Patrick Archdeacon, MD, for his leadership and guidance, which were instrumental in establishing KHI.

Co-Chairs

Patrick Archdeacon, MD

Office of Medical Policy, Center for Drug Evaluation and Research (CDER), FDA

Prabir Roy-Chaudhury, MD, PhD, FASN

American Society of Nephrology

Members

Matthew D. Breyer, MD, FASN (2017)

Eli Lilly and Company

Dolph Chianchiano, JD, MPA (2016)

National Kidney Foundation

Paul T. Conway (2018)

American Association of Kidney Patients

Mark Costanzo (2019)

Fresenius Medical Care North America

Ronald J. Falk, MD, FASN (2018)

University of North Carolina Chapel Hill

Michael Flessner, MD, PhD (2018)

National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)

Stuart L. Goldstein, MD (2017)

Cincinnati Children's Hospital

Jula K. Inrig, MD, FASN (2017)

Duke University, Quintiles Global Clinical Research Organization

Reshma Kewalramani, MD, FASN (2017)

Amgen

Alan S. Kliger, MD (2016)

Yale University

Mahesh Krishnan, MD, MPH, MBA, FASN (2019)

DaVita Healthcare Partners

Celeste Castillo Lee (2018)

Vasculitis Foundation

Peter G. Linde, MD, FASN (2017)

Accelaron Pharma, Inc.

Joe Muldoon (2019)

FAST Biomedical, Inc.

Carolyn Y. Neuland, PhD (2018)

Center for Devices and Radiological Health (CDRH), FDA

Meda E. Pavkov, MD, PhD (2018)

Epidemiology and Statistic Branch (ESB), Centers for Disease Control (CDC)

Jesse Roach, MD (2018)

Centers for Medicare and Medicaid Services

James A. Sloand, MD, FASN (2017)

Baxter International Inc.

James P. Smith, MD, MS (2018)

Center for Drug Evaluation and Research (CDER), FDA

Wendy L. St. Peter, PharmD, FASN (2018)

University of Minnesota

Katherine R. Tuttle, MD, FASN, FACP (2019)

University of Washington

Roberta L. Wager, MSN, RN (2018)

Fresenius Kidney Care

Celia Witten, PhD, MD (2018)

Center for Biologics Evaluation and Research (CBER), FDA

Alexander S. Yezlin, MD, FASN (2018)

University of Wisconsin

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

KHI Leadership and Staff

Founding Co-Chair

Patrick Archdeacon, MD

Medical Officer, Office of Medical Policy
Center for Drug Evaluation and Research (CDER)
Food and Drug Administration (FDA)

Active Co-Chair

Prabir Roy-Chaudhury, MD, PhD, FASN

Chief of Division of Nephrology
University of Arizona College of Medicine
Email: proychaudhury@deptofmed.arizona.edu

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Elle Silverman

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