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KHI

KIDNEY
HEALTH
INITIATIVE

**ANNUAL
REVIEW**



The Kidney Health Initiative (KHI) has a history of convening a diverse community focused on a common goal. Our new logo reflects the variety of KHI's membership, whose individual perspectives and collective efforts create a comprehensive and meaningful result. This re-branding positions KHI as an integral part of the ASN Alliance for Kidney Health. To match our new logo, KHI has refined its mission to **catalyze innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases**. The refreshed mission and new logo build on past success and reflect our vision for the future.



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Dear Colleagues,

On behalf of the Board of Directors, thank you for your membership in KHI. The 2019 Annual Review highlights what your membership and involvement in the kidney community have made possible.

This has been an exciting year for KHI and the kidney community in the fight against kidney diseases. Our success would be impossible without the unique partnership we have with the US Food and Drug Administration (FDA). Thanks to our 105 members, we pursued a variety of projects in drug and device development and motivated the community through collaborations and convenings. These efforts serve our refined mission *to catalyze innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases*. In addition to highlighting KHI's accomplishments in 2019, this year's report emphasizes the contributions of our members in developing better treatment options for people with kidney diseases.

This year, KHI surpassed the 100-member mark, making us the largest consortia in the kidney community. Many of those member organizations were represented at the Seventh Annual Stakeholders Meeting, which enjoyed the largest attendance to date. Continuing our commitment to provide the community with the tools to bring new therapies to market, this year KHI produced a publication on clinical trial endpoints for IgA Nephropathy. We expect additional publications on appropriate endpoints for hyperoxaluria trials as well as recommendations for including people with kidney diseases in cardiovascular trials. Building off our signature initiative, the *Technology Roadmap for Innovative Approaches to Renal Replacement Therapy*, we produced a Patient Edition of the Roadmap and a report on innovations in fluid management. Beyond our traditional projects and meetings, KHI catalyzed innovation through involvement in the first KidneyX prize competition, supporting NephCure Kidney International's efforts related to clinical trials, and assisting a new ASN Task Force focused on diabetic kidney disease.



From our vantage point at KHI, we have seen the kidney community evolve significantly since our founding in 2012. More drug companies, large and small, are investing in kidney diseases. Both rare diseases, like IgA Nephropathy and FSGS, and common diseases like diabetic kidney disease, are receiving new attention from industry. Patient organizations are increasing their efforts

to engage sponsors and the FDA by hosting patient focused drug development meetings, creating patient registries, and sharing information that supports clinical trial enrollment. New innovators are being brought into the field through KidneyX's prize competitions in the quest to redesign dialysis.

This summer, the announcement of the US Department of Health and Human Service (HHS)'s *Advancing American Kidney Health* (AAKH) initiative brought national attention to the public health need that is kidney disease. This means for the first time in decades, kidney diseases received attention from the White House. The AAKH emphasized the importance of innovation and discovery in drugs and devices, specifically the artificial kidney. We appreciate the support from the federal government and look forward to catalyzing innovation with the community at large.

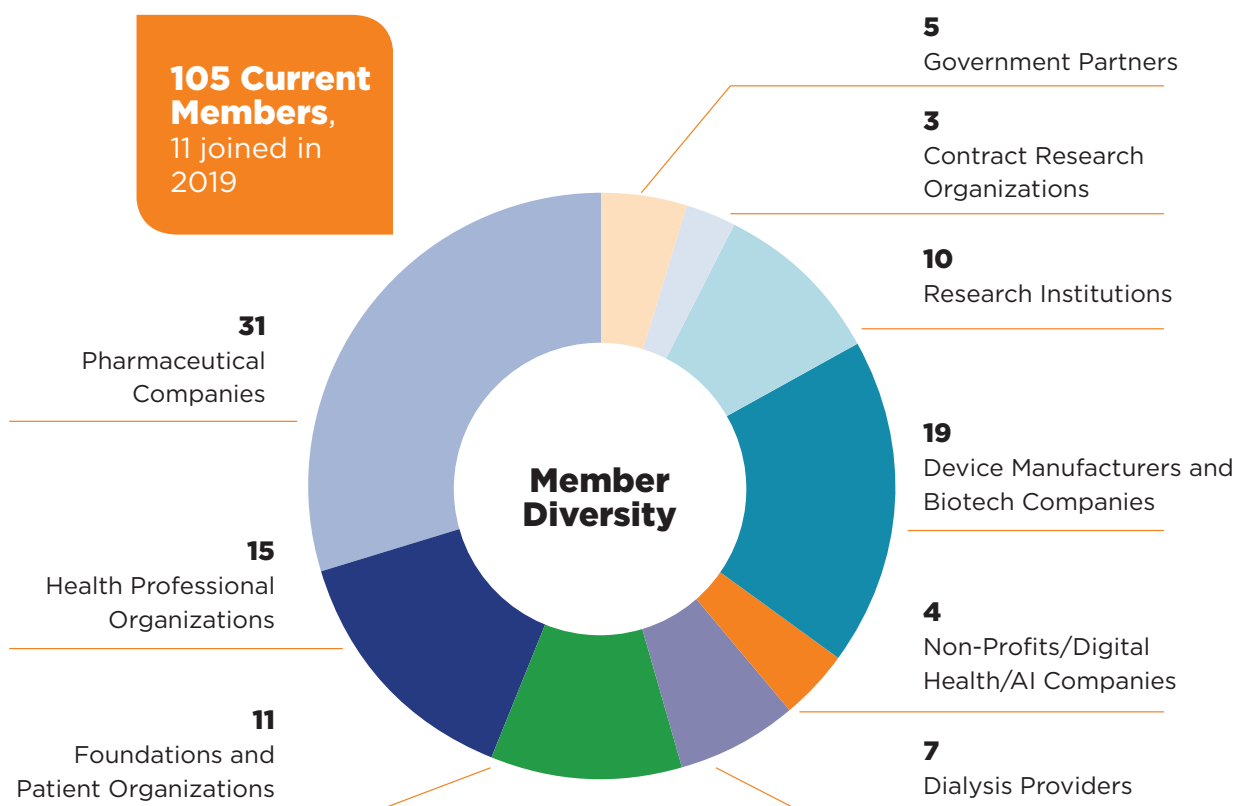
This is my first year as ASN Co-Chair for KHI and I look forward to continuing to refine our mission and build on our past successes in some key areas. First, including the patient perspective is foundational to our role in the community and I look forward to amplifying the work of the KHI Patient and Family Partnership Council. Second, the *Technology Roadmap for Innovative Alternatives to Renal Replacement Therapy* has been well received by the community and provides a model for KHI to replicate in other areas of need. Lastly, over the next year I anticipate KHI will capitalize on new opportunities to help translate basic science into new therapies.

Thank you to our members, Board of Directors, workgroup chairs, workgroup members, and the staff for making 2019 a success. I look forward to continuing our collaboration with you to improve the lives of people with kidney diseases.

Sincerely,

A handwritten signature in black ink that reads "Raymond Harris".

Raymond C. Harris, MD, FASN
ASN Co-Chair for KHI



152 Volunteers
Participating
in
Workgroups
and
Committees

14 Completed Projects
3 in 2019
8 Current Projects



Presentations
at 67 Meetings



19 Total Publications
2 in 2019



There is reason for optimism in drug development for kidney diseases. New collaborations among KHI members and within the broader community are creating an environment ripe for innovation. KHI is working alongside its members and the community by providing tools for industry and regulators to assess new drugs. These drugs will address the causes of kidney failure and progression of disease for the 37 million Americans with kidney diseases.

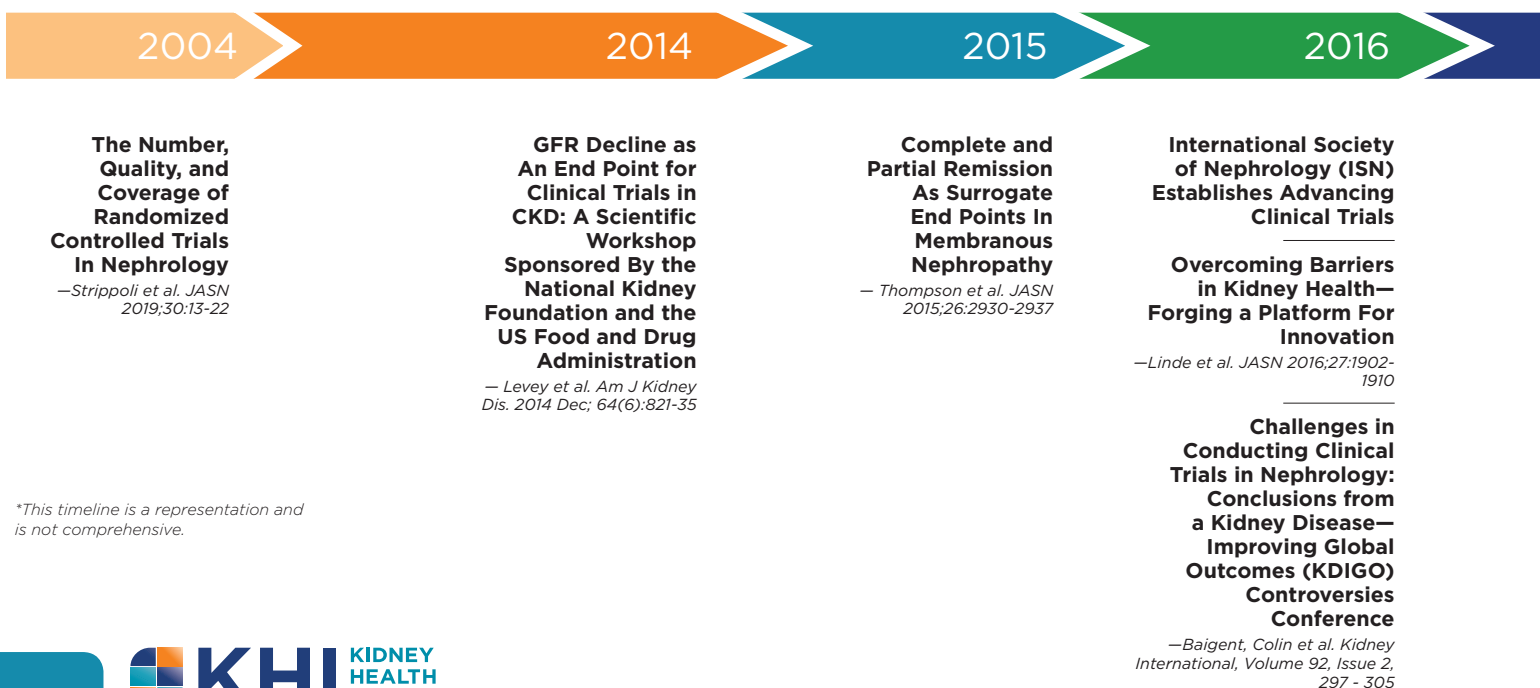
The landscape is changing. Industry, both small and large, is paying more attention to kidney diseases. Glomerular and rare diseases are attracting new attention, and renewed attention is being paid to diabetic kidney disease. Patient organizations are increasingly receiving an audience at the FDA, and are stepping up efforts to create patient registries, trial networks, and host collaborative scientific meetings.

These activities have occurred, in part, due to KHI's work in clinical trial endpoints and clinical trial infrastructure. As new trials are started and new molecules discovered, KHI will continue to support the community by bridging the gap between research and new therapies and building the relationships within the community to catalyze innovation.

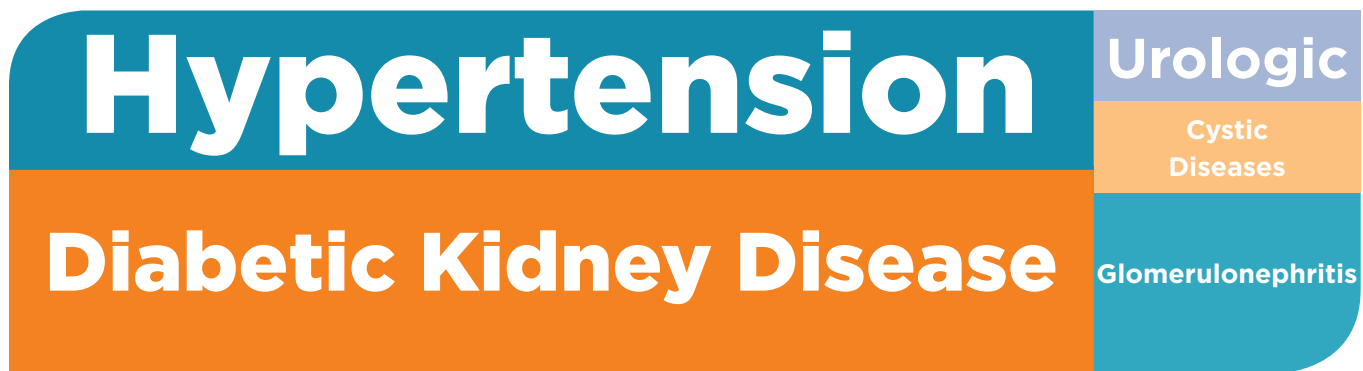
Pharmaceutical Company Members Conducting Publicly Disclosed Clinical Trials in Kidney Diseases

Achillion Pharmaceuticals, Inc.	Corvidia Therapeutics
Akebia Therapeutics, Inc.	Dimerix Bioscience
Allena Pharmaceuticals, Inc.	Goldfinch Bio
Alnylam Pharmaceuticals	Hansa Biopharma
Angion Biomedica Corporation	Janssen Research & Development, LLC
Amgen, Inc.	KBP Biosciences
AstraZeneca	Mallinckrodt Pharmaceuticals
Bayer AG	Otsuka America Pharmaceuticals, Inc.
Calliditas Pharmaceuticals AB	Oxthera AB
ChemoCentryx, Inc.	Pfizer, Inc.
Complexa, Inc.	Reata Pharmaceuticals, Inc.
	Retrophin, Inc.

Key Publications, Events, and Meetings*



Primary Causes of Kidney Failure (by proportion)



KHI Projects to Support Innovation

Project Topic	Current Project	Workshop	Publication
Prioritizing ESRD Symptoms			
IgA Nephropathy Surrogate Endpoints			
Lupus Nephritis Surrogate Endpoints			
Cardiovascular Disease Trials			 (Forthcoming)
FSGS Surrogate Endpoints			 (Forthcoming)
Hyperoxaluria Surrogate Endpoints			 (Forthcoming)
Pediatric Drug Development			 (Forthcoming)



Total Kidney Volume is a Prognostic Biomarker of Renal Function Decline and Progression to End-Stage Renal Disease in Patients With Autosomal Dominant Polycystic Kidney Disease

—Perrone et al. *Kidney International Reports*, Volume 2, Issue 3, 442 - 450

Global Kidney Health 2017 and Beyond: A Roadmap For Closing Gaps in Care, Research, and Policy

—Levin et al. *The Lancet*, Volume 390, Issue 10105, 1888 - 1917

NKF/Patient Centered Outcomes Research Institute (PCORI) Patient Meeting on Trial Participation

Kidney Disease Clinical Trialists Meeting

Change in Albuminuria and GFR as End Points for Clinical Trials in Early Stages of CKD: A Scientific Workshop Sponsored by the National Kidney Foundation in Collaboration with the US Food and Drug Administration and European Medicines Agency.

—Levey, Andrew S. et al. *American Journal of Kidney Diseases*, Volume 0, Issue 0

Cultivating a Research-Ready Dialysis Community

—Flythe et. al. *JASN* Mar 2019, 30 (3) 375-380; DOI: 10.1681/ASN.2018101059

NKF/Alport Syndrome Foundation/FDA Externally Led Patient-Focused Drug Development Meeting (PFDD) Meeting

NKF/IgA Nephropathy Foundation/FDA Externally Led Patient-Focused Drug Development Meeting

NKF Scientific Workshop: The Role of the Kidney and SGLT2 in Glucose Homeostasis and Kidney Disease

ASN Diabetic Kidney Disease Collaborative Launched

MEMBERSHIP

Thank you to all our members. Together, we represent the largest consortia in kidney diseases, working collaboratively to catalyze innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases.

CONTRACT RESEARCH ORGANIZATIONS



DEVICE MANUFACTURERS AND BIOTECH COMPANIES



Wearable Artificial Organs Inc. (WAO)

DIALYSIS PROVIDERS



PHARMACEUTICAL COMPANIES



GOVERNMENT PARTNERS



HEALTH PROFESSIONAL ORGANIZATIONS



NON-PROFITS, DIGITAL HEALTH, & AI COMPANIES



FOUNDATIONS AND PATIENT ORGANIZATIONS



RESEARCH INSTITUTIONS

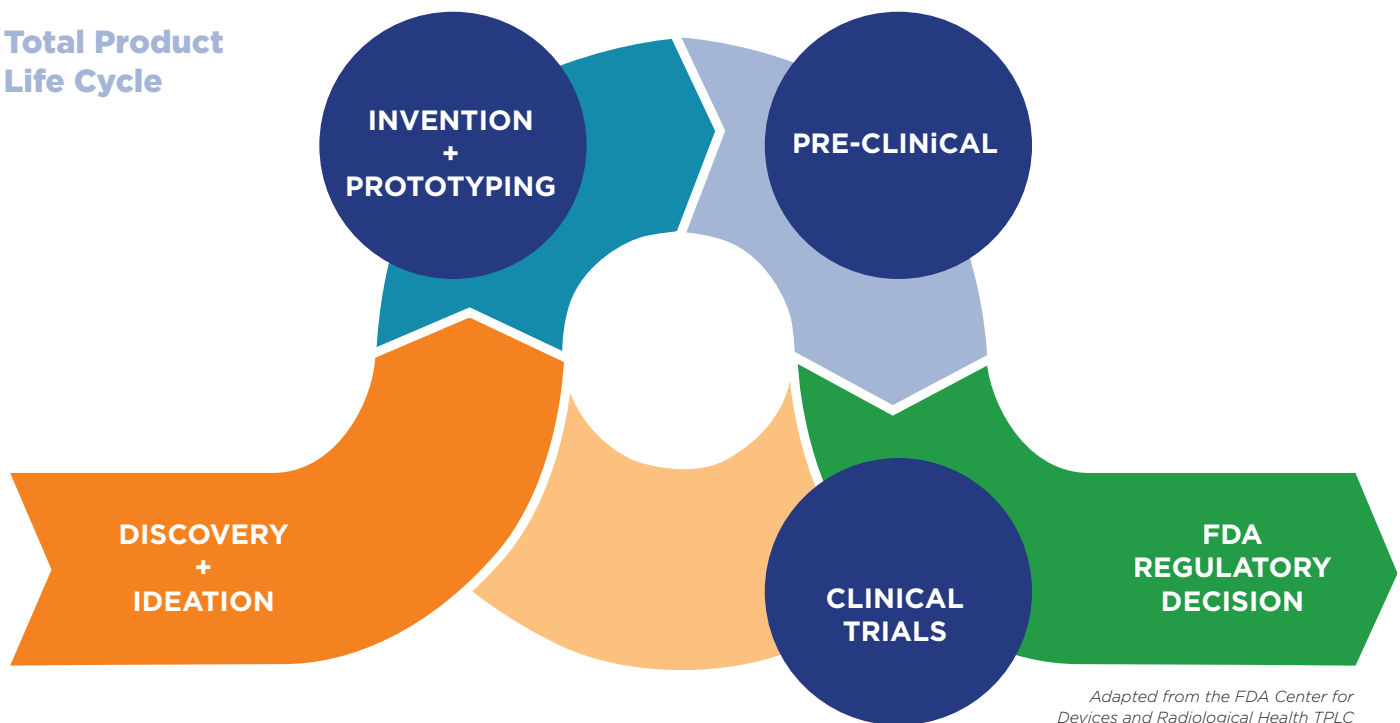


CATALYZING DEVICE DEVELOPMENT

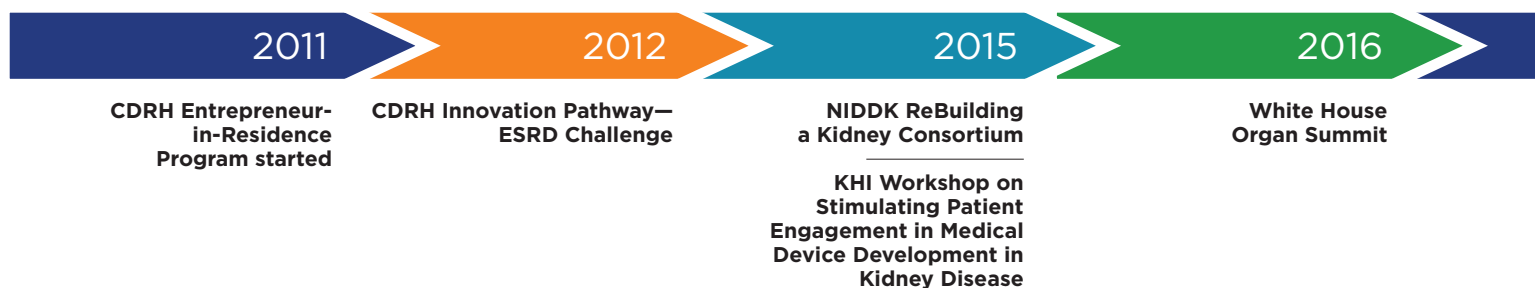
The environment around developing new devices for people living with kidney diseases is changing for the better. Today, more than a dozen member organizations are developing innovative devices and diagnostics. Through partnership with the FDA, KHI is addressing barriers to innovation and supporting people living with kidney diseases.

KHI has provided the community with resources to take advantage of this moment. Innovations in vascular access are essential to moving the artificial kidney forward. Publications on vascular access endpoints and trial design laid the foundation for important technologies underlining the artificial kidney. The *Technology Roadmap for Innovative Approaches to Renal Replacement Therapy* provides guidance for the community to move device development forward on many fronts and is being expanded to address new issues and to reach new audiences. Our patient reported outcomes and patient perspective projects help ensure that tomorrow's innovations meet the needs of people with kidney diseases.

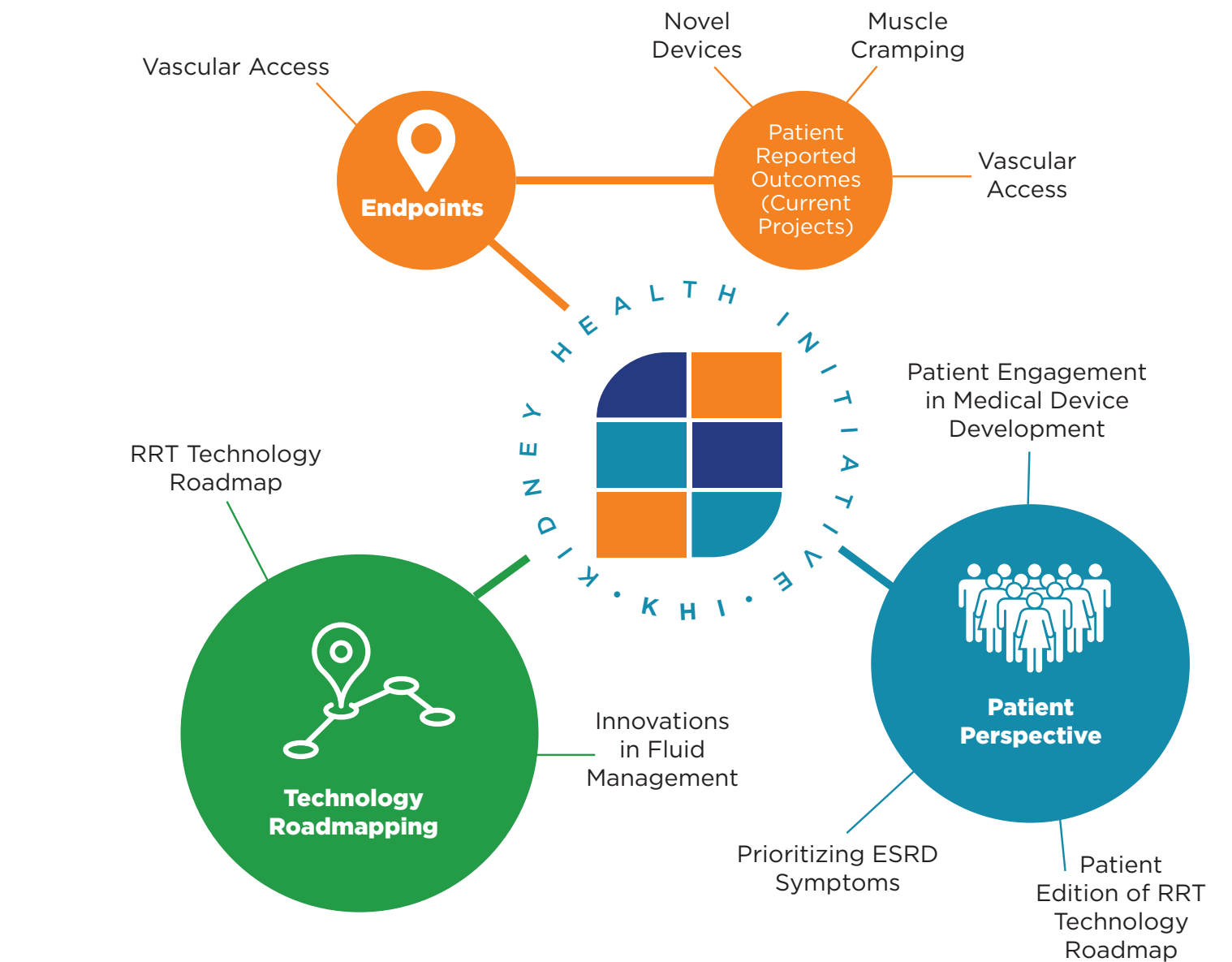
Total Product Life Cycle



Key Publications, Meetings & Events



KHI Projects to Support Innovation



2017	2018	2019
<p>Clinical Trial End Points for Hemodialysis Vascular Access —Shenoy S et al. CJASN March 2018, 13 (3) 490-4946</p> <p>Recommended Clinical Trial End Points for Dialysis Catheters —Allon M et al. CJASN March 2018, 13 (3) 495-500</p> <p>Definitions and End Points for Interventional Studies for Arteriovenous Dialysis Access —Beathard GA et al. CJASN March 2018, 13 (3) 501-512</p> <p>FDA Regulatory Perspectives for Studies on Hemodialysis Vascular Access Hurst FP, et al. CJASN Mar 2018, 13 (3) 513-518</p>	<p>KidneyX Innovation Accelerator Launch</p> <p>KHI RRT Technology Roadmap Release</p>	<p>KidneyX Redesign Dialysis Phase 1 and Phase 2 Prize Competitions</p>

Making progress in providing better treatments for people with kidney diseases requires including the voice of people with kidney diseases. KHI embeds that idea in everything it does. Patients and care partners vet all project proposals, serve on project workgroups, help plan the Annual Stakeholders Meeting, and lead on the Board of Directors. Signature initiatives, like the *Technology Roadmap for Innovative Approaches to Renal Replacement Therapy*, and other projects, are built around patient preferences and responding to the needs of people with kidney diseases. These efforts would not be possible without the support of KHI's members representing patient organizations and foundations across the community.

The KHI Patient and Family Partnership Council (PFPC) pushes the initiative to include the patient voice in drug and device development. This diverse group represents a variety of treatment experiences including home hemodialysis, in center hemodialysis and transplantation. The KHI PFPC also provides the patient perspective on nearly all of KHI's projects. The group provides extensive expertise on issues such as clinical trial operations, patient reported outcomes, patient engagement, and pediatric product development.

Thanks to these organizations for supporting the KHI PFPC

American Association of Kidney Patients
American Kidney Fund
Alport Syndrome Foundation
Dutch Kidney Foundation
Home Dialyzers United
JDRF International
National Kidney Foundation
NephCure Kidney International
Oxalosis and Hyperoxaluria Foundation
PKD Foundation
Vasculitis Foundation



**2019 KHI
Patient
and Family
Partnership
Council**

Left to right: Kevin J. Fowler (Vice-Chair), Patrick O. Gee, Mary Baliker, Pamela Duquette, Denise Eilers, Amanda Grandinetti, Caroline Wilkie, Nichole Jefferson, David M. White (Chair)
Not pictured: Richard Fissel.

Convening the community is one of KHI's strengths and an important benefit for members. The Seventh Annual Stakeholders Meeting revolved around the theme of "Collaborating for Innovation to Improve Patient Care and Outcomes." A Planning Committee led by Co-Chairs Paul T. Conway and Mahesh Krishnan, MD, FASN, developed a program that attracted nearly 200 registrants. The "think-tank" style meeting included onstage Q&A with government representatives, presentations from leaders in the kidney community, and member showcases highlighting innovations within the KHI membership.

KHI thanks the 2019 Planning Committee for their hard work and dedication

Paul T. Conway, *Co-Chair*

Mahesh Krishnan, MD, FASN, *Co-Chair*

Nieltje Gedney

Duvuru Geetha, MD, FASN

Nicolas J. Guzman, MD

Raymond C. Harris, MD, FASN

Nichole Jefferson

David Kuraguntla

Kristen Miller, PharmD

Joseph Muldoon

Jesse Roach, MD

Prabir Roy-Chaudhury, MD, PhD, FASN

Monnie Wasse, MD, MPH, FASN

Keynote Presentations



Amy Abernethy, MD, PhD, Principle Deputy Commissioner, FDA with Mahesh Krishnan, MD FASN (right)



Adam Boehler, Director of the Innovation Center at the Center for Medicare and Medicaid Services (CMS) with Paul T. Conway (right)



Eric Hargan, JD, Deputy Secretary at HHS

Thank you to the Board of Directors and Project Workgroup Chairs for making KHI's work possible.

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KHI Patient and Family Partnership Council

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Center for Biologics Evaluation and Research, FDA

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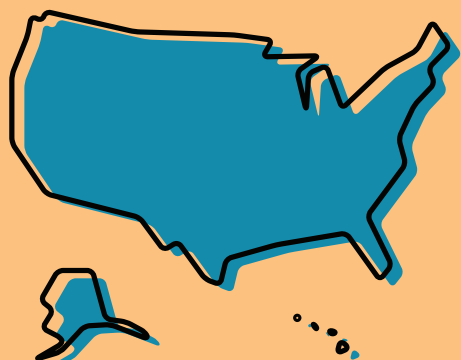
Agios Pharmaceuticals

Karen Woo, MD, MS

University of California, Los Angeles

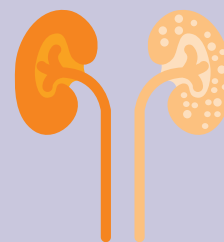
Eric W. Young, MD

Arbor Research Collaborative for Health



**37
million**

Americans
with kidney
diseases



726,000

people with
kidney failure



\$114 billion

spent by Medicare on
kidney diseases



58%

mortality
rate on
hemodialysis

“PEOPLE WITH KIDNEY FAILURE DIE EVERY DAY WITHOUT SEEING INNOVATIVE NEW DRUGS AND DEVICES. THE TIME IS NOW TO RENEW OUR COMMITMENT TO CATALYZE INNOVATION AND DELIVER A WORLD WITHOUT KIDNEY DISEASES.”

— Raymond C. Harris, MD, FASN, KHI Co-Chair



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