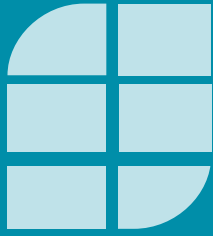




2023 ANNUAL REPORT

kidneyhealthinitiative.org



The Kidney Health Initiative (KHI) is a public-private partnership among the American Society of Nephrology (ASN), the US Food and Drug Administration (FDA), and with more than 70 member companies and organizations committed to catalyzing innovation and the development of safe and effective patient-centered therapies for people with kidney diseases.

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MESSAGE FROM THE CHAIR OF KHI'S BOARD OF DIRECTORS

As we reflect on the past year, we are filled with immense gratitude for the unwavering dedication and commitment demonstrated by each of you towards the mission of the Kidney Health Initiative (KHI). Your collective efforts have been instrumental in driving forward our shared vision of catalyzing innovation and advancing the development of safe and effective patient-centered therapies for individuals with kidney diseases.

2023 was a busy year with many highlights. The KHI convened its Annual Meeting, featuring keynote speaker FDA Commissioner Robert M. Califf, MD, and hosted in-person workshops for projects focused on APOL1 kidney disease and pediatric trials. Additionally, KHI hosted two standing-room only sessions at ASN Kidney Week 2023, contributed to the design of several other sessions, and hosted an Open House.

Throughout the year, KHI hosted virtual events including webinars, roundtables, and networking mixers. KHI also co-hosted, moderated, or presented at convenings across the kidney community. KHI submitted comments to FDA guidance documents, published roadmaps, and launched a new project. Finally, KHI also communicated its efforts through several publications and videos, emphasizing patient perspectives and collaborative achievements.

As we commemorate the milestones of the past 10 years, we extend our deepest appreciation to all who have contributed to KHI's success. Your commitment and collaboration have been instrumental in shaping the trajectory of our kidney health initiatives.

Looking ahead, we are filled with hope and optimism for the future. With continued collaboration and determination, we are confident in our ability to overcome challenges together and make meaningful strides towards our shared goal of improving the lives of people with kidney diseases.

Thank you for your support and dedication towards improving kidney health.

Sincerely,

Uptal D. Patel, MD

Chair, Kidney Health Initiative

Board of Directors



MESSAGE FROM THE CHAIR OF KHI'S PATIENT AND FAMILY PARTNERSHIP COUNCIL

In 2023, the Kidney Health Initiative's Patient and Family Partnership Council's (PFPC) priority was to establish our value proposition to the kidney community. We redefined our Vision Statement to highlight our collective skills, talents, and expertise as:

"The PFPC strives to be the foremost leader in incorporating lived experience into kidney research, driving person-centered innovation throughout the development of medical devices, drugs, and biologics."

Along with Vice Chair Jack Lennon, MBA, the PFPC continued to build on our Patient Advisory Board project that honored Celeste Castillo Lee, the mother of Patient Advisory Boards. Inspired by her vision and dedication, our objective is to underscore the importance of strategically integrating the advice and experiences of patient advocates and their care partners in advisory boards, committees, and boards of directors of research organizations and companies.

Leading by example, members of the PFPC provided their own strategic advice in review panels for KidneyX competitions and contributed as speakers at several sessions at the ASN Kidney Week 2023.

As our first community tool, PFPC members published an instructional video series to teach other people with lived experiences of kidney diseases on how to integrate their voices and advise in the development of much needed medical devices, biologics and drug therapies, and in the design of clinical trials for these innovations. The PFPC also hosted a discussion on Patient Advisory Boards with representatives from the National Kidney Foundation, American Kidney Fund, and American Association of Kidney Patients at the KHI Annual Meeting in Washington, DC.

Amongst these wins, Derek L. Forfang, one of PFPC's most dedicated members, unexpectedly passed away on October 20, 2023. His untimely death left us momentarily paralyzed, but the PFPC rallied in unity with the whole kidney community to honor his legacy on October 26 with a virtual celebration of his life. Amidst our grief and despair, the professional and patient kidney community shared thoughts, memories, and prayers with the Forfang family. This gathering provided solace and healing for all with the PFPC vowing to channel the pain of Derek's loss into preserving his legacy.

2023 was an important milestone for the PFPC as we established our identity to lead within the Kidney Health Initiative. We used this year as a time of regrouping, self-reflection, and doing more with fewer resources. We now focus our efforts on doing more in 2024 on pediatric kidney disease, transplantation, and home dialysis. We are eager to tackle these issues through a lens that includes our own lived experiences, those of our caregivers and care partners, and with a need to create Health Equity.

Sincerely,

Patrick O. Gee, Sr., PhD, JLC

Chair, Kidney Health Initiative

Patient and Family Partnership Council



2023 IMPACT & HIGHLIGHTS



Convene

- 1.** The Annual Meeting in September reunited the kidney community after Kidney Health Initiative's (KHI) last in-person meeting in 2019. This event celebrated the inaugural decade and set the trajectory for KHI's next decade. Distinguished speakers, including U.S. Food and Drug Administration (FDA) Commissioner Robert M. Califf, MD, inspired the kidney community through his keynote, which was followed by a series of panel discussions and presentations highlighting numerous opportunities for collaborating to accelerate the development of innovative biologics, devices, and drugs for people with kidney diseases.
- 2.** As a supplement to the Annual Meeting, KHI hosted workshops to gather input on two projects. The first workshop solicited feedback on the draft KHI Roadmap on APOL1 Kidney Disease Clinical Trial Awareness and Screening, which was launched shortly after the Annual Meeting. The second workshop solicited feedback as KHI expands its efforts to strengthen clinical trials for the pediatric kidney population.
- 3.** At ASN Kidney Week 2023 in Philadelphia, PA, KHI organized and moderated two standing-room only sessions.
 - Clinical Trials in Pediatric CKD: Trial with SGLT2 Inhibitors
 - Treatment Development in IgA Nephropathy
- 4.** In addition to KHI hosted sessions, KHI experts contributed to the design of other Kidney Week sessions:
 - APOL1 and Kidney Transplantation
 - Health Care Equity in Education, Clinical Care, and Community Engagement
 - Implementation: Breaking Barriers to Reach the Bedside
- 5.** An ASN Innovation Tournament at Kidney Week featured several KHI leaders.
- 6.** The KHI Community also convened at Kidney Week at a dedicated Open House.

2023 IMPACT & HIGHLIGHTS

- 7. In addition to in-person convenings, KHI also hosted several topical webinars, virtual roundtables and workshops for the member community:**
 - Sickle Cell Nephropathy: Therapeutic Landscape and Unmet Needs
 - The State of Kidney Precision Medicine
 - Regulatory Considerations for Human Factors Studies In Dialysis Devices
- 8. KHI also launched a series of virtual Networking Mixers:**
 - Meet the KHI Patient and Family Partnership Council (PFPC) to assess the community needs to strengthen patient advisory boards
 - Meet the FDA Liaisons to KHI, including representatives from FDA CBER, CDER, and CDRH
 - Meet the KHI Board of Directors
 - Meet the Patient Advocacy Organizations that are KHI members to highlight research resources available to kidney researchers
 - Scientific Opportunities to Advance Drug Development through Data Sharing, to highlight data sharing opportunities and challenges for kidney researchers
 - Patient Perspectives on Innovations for Vascular Access
- 9. As a collaboration with KHI member, NephCure, KHI co-hosted an in-person workshop on SGLT2 inhibitors in pediatric populations.**
- 10. KHI leadership also co-organized, moderated, or presented at convenings hosted by the kidney community:**
 - American Association of Kidney Patients (AAKP) Annual Global Summit of Kidney Innovations
 - American Kidney Fund (AKF) Unknown Causes of Kidney Disease Project
 - Kidney Innovation Accelerator (KidneyX) Summit
 - National Kidney Foundation (NKF) Spring Clinical Meeting
 - NephCure Health Equity Conference
 - NephCure Membranous Nephropathy Scientific Workshop

Catalyze

- 1. Submitted a response to the FDA Request for Comment on Increasing Patient Access to At-Home Use Medical Technologies.**
- 2. Published Roadmaps**
 - Human-Centered Design Toolkit (v2.0)
<https://khi.asn-online.org/pages/?ID=80>
 - Roadmap for APOL1 Kidney Disease
<https://khi.asn-online.org/projects/project.aspx?ID=91>
- 3. Launched a project to frame clinical/regulatory pathways for Sickle Cell Kidney Disease (SCKD), building synergies for Sickle Cell Disease (SCD) and kidney treatments approved or in development. This project focuses on: understanding natural history of SCKD and identifying SCD patients at higher risk of progression to kidney disease; identifying and evaluating endpoints, metrics, and effect sizes; and recommending trial populations based on biomarkers.**

Communicate

1. Publications

- Overcoming Barriers to Drug Development in Children with CKD as a Feature Article in the Clinical Journal of the American Society of Nephrology
https://journals.lww.com/cjasn/fulltext/2023/08000/overcoming_barriers_to_drug_development_in.21.aspx
- Patient Perspectives of Skeletal Muscle Cramping in Dialysis
<https://journals.lww.com/kidney360/pages/articleviewer.aspx?year=2023&issue=06000&article=00008&type=Fulltext>
- Dialysis was Hell to Me, highlighting the Chair of KHI PFPC
www.kidneynews.org/view/journals/kidney-news/15/5/article-p17_8.xml
- End Point Considerations for Clinical Trials in Enteric Hyperoxaluria
<https://journals.lww.com/cjasn/pages/articleviewer.aspx?year=2023&issue=12000&article=00021&type=Fulltext>
- Xenotransplantation and the Role of the Patient Voice
https://journals.lww.com/kidney360/fulltext/2024/01000/xenotransplantation_and_the_role_of_the_patient.14.aspx

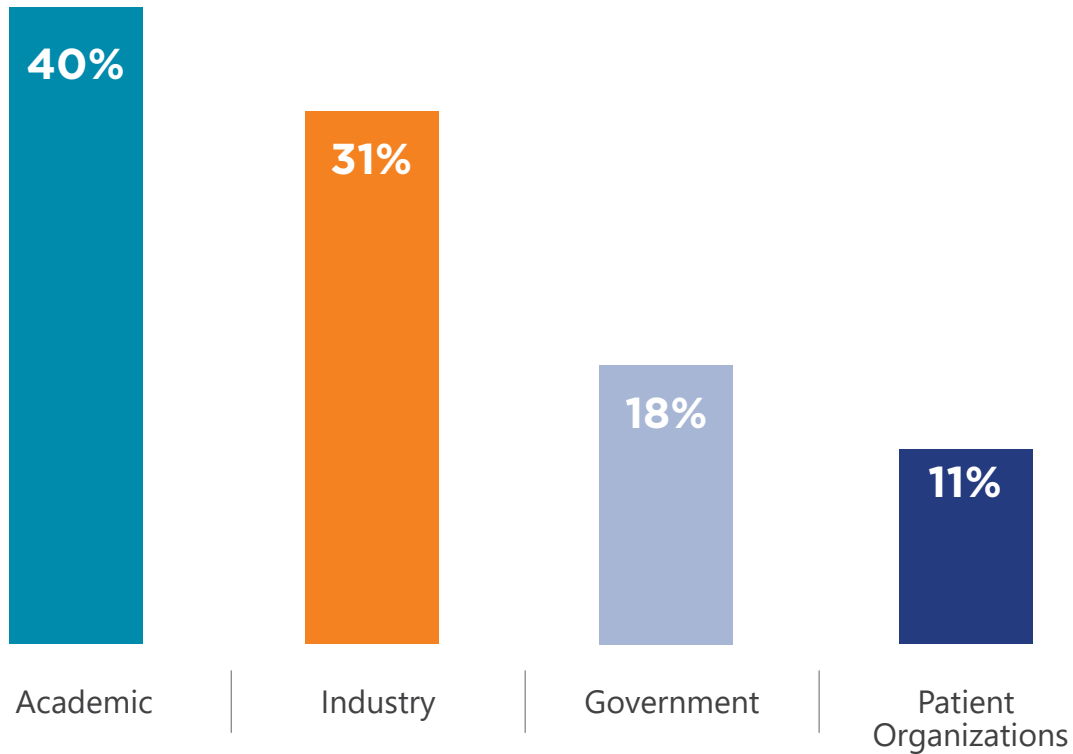
2. Videos

- An Introduction to Patient Advisory Boards a series of instructional videos for patients and care partners who are recruited to join Patient Advisory Boards
<https://www.youtube.com/watch?v=t7JBq62xO8k>
- 10 Years of the Kidney Health Initiative
<https://www.youtube.com/watch?v=gJGSmWCxtfl>

STATISTICS

Volunteers

In 2023, 67 dedicated volunteers collaborated within KHI projects to lend their expertise and dedication. These volunteers played a crucial role in advancing KHI's mission and objectives and represented diverse backgrounds that include patient organizations, academia, government, and industry. Their contributions were instrumental in advancing initiatives such as Endpoints for C3 Glomerulopathy, Roadmap for APOL1 Kidney Disease, and Clinical Trials in pediatric IgAN.



	3 New Member Organizations		3 Completed Projects
	67 Volunteers Participating in Workgroups		5 Publications

2023 NEW KHI MEMBER ORGANIZATIONS

Learn more about how these organizations are working towards improving the lives of people with kidney diseases.



BMI OrganBank

A developer of medical devices intended to preserve a wide variety of organs and tissues ex-vivo to transform the world of organ transplant.



Hansa Biopharma AB

A commercial-stage biopharmaceutical company supporting the development and commercialization of innovative treatments for patients with rare diseases.



Vera Therapeutics

A biologics company with a mission to change the standard of care for people with immunological diseases

CATALYZING DRUG DEVELOPMENT

In 2023, several projects were completed with goals to drive drug development innovation for rare kidney diseases, under the direction and oversight of the Drugs Committee of KHI's Board of Directors.



Completed Projects

Drug Development in Children with Kidney Diseases

This project published recommendations to foster drug development for children with kidney diseases. The work group included key opinion leaders including patients, health care professionals, researchers, professional organizations, industry partners, and regulators.

Endpoints for Enteric Hyperoxaluria Clinical Trials

This project published an evaluation of potential endpoints for assessing the efficacy of treatments for hyperoxaluria. The work group included patients, families, advocacy organizations (Oxalosis and Hyperoxaluria Foundation), clinicians, scientists, pharmaceutical companies, and the FDA.

Roadmap to increase disease awareness and clinical trial participation of APOL1 Kidney Disease (APOL1 KD)

This project published the first version of a “living” roadmap with three core goals: increase APOL1 KD awareness, increase access to and awareness of genetic testing and associated counseling, and empower people at higher risk of developing APOL1 KD to make informed decisions about participating in clinical studies. APOL1 KD is one driving force behind disproportionate kidney health outcomes for those of recent African ancestry. KHI aims to further align collaborations within the community to strengthen the potential of treatments being developed for APOL1 KD.



Continuing Projects

Endpoints for Focal Segmental Glomerulosclerosis (FSGS)

This project aims to identify surrogate endpoint(s) for use in FSGS interventional clinical trials. Despite being associated with a relatively rapid decline in kidney function, there are no approved drugs and no accepted endpoints for registration of investigational products for FSGS. The project goals include: review published literature and analyze data from cohorts/completed trials to evaluate candidate surrogates for endpoints, identify risk factors for progression, critically assess the candidate measures, and identify gaps in the data.

Endpoints for C3 Glomerulopathy (C3G)

This project's scope was revised due to feasibility constraints for developing a central repository of shared data. The new focus is to evaluate strengths and limitations of current endpoints for C3G and provide insights for research and trials. C3G remains an aggressive renal disease with high risk for progression to kidney failure, with recurrence in transplants being common. The rarity of the disease limits a precise, systematic description of its natural history, and current treatment approaches have unpredictable effects on outcomes, with no targeted therapeutics available to patients.

Clinical Trials in Pediatric Patients with IgA Nephropathy

This project assesses the current literature and trial landscape to obtain community consensus on a path forward for developing therapies for children with IgA Nephropathy. This project includes the broader community who host clinical trial or registry data, resources that could inform natural history of disease research or identify biomarkers candidates and outcome measurements that advance therapies for children at-risk or living with IgA Nephropathy.

CATALYZING DEVICE DEVELOPMENT

In 2023, KHI's device portfolio focused on the home dialysis device development community. This focus was driven by needs expressed by member organizations and emphasized by the Devices Committee of KHI's Board of Directors, which hosted two activities:

Monthly Mixer: What is ideal and is it possible to have “Perfect” Vascular Access?

This informal conversation provided people with kidney failure an opportunity to share their requirements for improved hemodialysis vascular access, and for entrepreneurs and product developers to interact with patients and share barriers they face in product development. The objectives were to: 1) hear the patient perspective on the ideal vascular access, 2) facilitate conversation between innovators and patients on requirements and barriers, and 3) define specific problem areas that could be addressed in a pre-competitive forum. The event attracted 49 participants including 14 representatives from vascular access companies, nine people with kidney failure, and five representatives from the FDA Centers for Devices and Radiological Health (CDRH).

Roundtable: Regulatory considerations for Human Factors Studies in Dialysis Devices

This public roundtable meeting provided a venue among FDA CDRH's Human Factors and Dialysis teams and the device industry to exchange thoughts on pre-competitive and product agnostic questions to improve human factors studies for novel dialysis devices. The roundtable involved 59 participants including representatives from 10 dialysis device manufacturers.

CATALYZING BIOLOGICS DEVELOPMENT

In 2023, KHI completed its inaugural project in the Biologics portfolio under oversight of the Biologics Committee of the KHI Board of Directors. The project, “Assessing Knowledge and Perception of Xenotransplantation”, brought together FDA-liaisons from FDA Center for Biologics Evaluation and Research (CBER) and a diverse group representing industry, patients, health care professionals, and researchers. This project resulted in a qualitative survey of patients, care partners, and health care professionals on their perceptions of kidney xenotransplantation, which were summarized in two publications authored by the patient advocates who led this project.

Beyond xenotransplantation, there is significant potential for creating new treatment options through biologics-based therapies. This includes cellular therapy and products developed through genetic and cellular engineering, but despite the approvals of gene and cell therapies for inherited diseases affecting other organs, none have specifically focused on kidney health.

A newly launched project is evaluating pathways for sickle cell kidney disease, and proposed projects in the pipeline include toolkits that communicate benefits, risks, and science for potential participants of investigational studies on emerging technologies.

MEMBER ORGANIZATIONS

FOUNDATIONS AND PATIENT ORGANIZATIONS



RESEARCH INSTITUTIONS



HEALTH CARE PROFESSIONAL ORGANIZATIONS



NON-PROFITS



MEMBER ORGANIZATIONS

DEVICE MANUFACTURERS AND DIAGNOSTICS COMPANIES



PHARMACEUTICAL AND BIOTECH COMPANIES



KIDNEY CARE AND DIALYSIS PROVIDERS



CONTRACT RESEARCH ORGANIZATIONS



FEDERAL PARTNERS



ACKNOWLEDGMENTS

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IN FOND REMEMBRANCE OF
DEREK L. FORFANG
A MAN OF ADVOCACY AND
LASTING LEGACY

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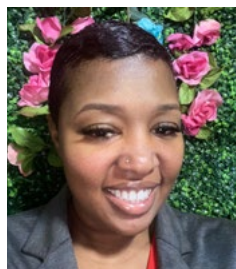
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LOOKING AHEAD

The Kidney Health Initiative will continue to advance its mission of catalyzing innovation and the development of safe and effective patient-centered therapies for people with kidney diseases. Established by the Strategy Committee of KHI's Board of Directors, KHI's objectives in 2024 focus on the following:

1. Convene and communicate a culture of innovation.

- a. Develop toolkits that communicate benefits, risks, and science to potential clinical trial participants.
- b. Serve as a neutral forum that defines challenges and identifies collaboration opportunities that strengthen pipeline of kidney innovations.

2. Support design and conduct of clinical trials.

- a. Integrate perspectives, preferences, and other measurements from people with kidney diseases and care partners in clinical trials.
- b. Evaluate clinical trial endpoints that could accelerate development of candidate products for rare kidney diseases.
- c. Increase participation of people with kidney diseases in clinical trials while also addressing participant diversity and underrepresentation.
- d. Increase participation of nephrologists who promote clinical trials as a treatment option.

3. Address innovation opportunities for pediatric kidney populations and people with kidney transplants.

NOTES

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