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**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 over 100 member companies and organizations committed to **catalyzing innovation and the development of safe and effective patient-centered therapies** for people living with kidney diseases.

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Even in the face of the ongoing pandemic, the Kidney Health Initiative has continued to serve as an active and important part of an innovation community, working with the FDA to identify transformative issues related to kidney diseases and helping to provide solutions that overcome roadblocks to the development of impactful therapies for people with kidney diseases. In partnership with the ASN, KHI has worked to protect people with kidney failure and dialysis clinic staff from infection with COVID-19. In addition, KHI continues to work closely with FDA, industry, researchers, and people with kidney diseases to develop new opportunities and approaches for clinical trial design and implementation. In addition to our partnership with the FDA, these efforts are the result of strong leadership from the Board of Directors, the commitment of our workgroup chairs, expertise from workgroup members, and participation from our member companies and organizations. During the past year, we have also realigned the Board of Directors and the Patient and Family Partnership Council to better meet the goals of KHI. Board subgroups focused on drugs, devices or biologics have helped to address specific issues and provide direction for ongoing and future KHI activities.



2021 has been an active year for KHI. We hosted a successful virtual Eighth Annual Stakeholder Meeting that attracted 235 participants. We also completed six projects and launched four new projects. We continued to have active volunteer participation in our workgroups, with 130 participants in 2021, and nine new publications representing KHI activities. We foresee that during 2022, we will complete projects that align closely with the FDA's efforts to incorporate patient preferences in development of novel devices for kidney replacement therapy and provide the community with a roadmap to support development of biomarkers for acute kidney injury. In addition, KHI will convert our technology roadmap for innovative kidney replacement treatments into a dynamic website and is working with the pediatric nephrology community to prioritize and develop a pipeline of projects.

We have recently seen some real successes in developing therapies that can slow the progression of kidney diseases, and KHI will continue to work with all its members to continue the fight to prevent, treat and cure kidney diseases. To support this effort, we have developed five strategic priorities for the upcoming year. First is to increase participation of people with kidney diseases in clinical trials, both trials that are directed specifically at kidney diseases and other trials for which people with kidney diseases have been systematically excluded in the past. Our second priority is to promote the need for inclusion of patient preferences into trial design and product development and help to develop tools to aid in their effective use. We will also continue to catalyze development of therapies for rare diseases that can affect the adult or pediatric population. Finally, we will convene the patient, research, government and kidney care communities to address important unmet needs. To better support innovators, KHI will use a variety of channels to communicate with members and the broader community. Thank you for being a part of the important work that KHI is doing. Without the commitment, ingenuity, and persistence of its members, KHI could not meet the demand for patient-centered therapies for people with kidney diseases.

Sincerely,



Raymond C. Harris, MD, FASN
Chair, Kidney Health Initiative

In 2021 the Patient and Family Partnership Council (PFPC) launched new initiatives to strengthen the voice of the patient amongst the innovation community including efforts to advance equity in kidney health and the building and utilization of effective patient advisory boards. Most notably, the PFPC became a communication platform that the kidney community will be able to use to convene and lead patient-driven collaborations with kidney care stakeholders for years to come.

Amongst the nine members of the PFPC there has been a vast number of activities highlighting our commitment to patient-centered innovation. During KHI's Eighth Annual Stakeholder's Meeting "Meeting Patients Where They Are," Patrick O. Gee Sr., PhD, JLC, represented the PFPC in the plenary session "2020 and Beyond: Integrating the community to accelerate innovation." Next, we introduced our signature project – Promoting the expanded development and utilization of patient advisory boards to improve kidney patients' clinical outcomes and quality of life – in our session "Designing a patient advisory board to strengthen clinical trials and research." Vanessa A. Evans, Glenda V. Roberts, and Amanda Grandinetti, MPH's expert presentations were followed by concurrent brainstorming breakout discussions. The following day, Nichole M. Jefferson shared her thoughtful insights in the session "Leap of Faith: First-in-Human Clinical Trials for Potentially Transformative Products in CBER" which highlighted the need for patient choice when it comes to treatment options.

In December, the PFPC followed up on the success of the Stakeholder Meeting's patient-driven focus by delivering the first installment of the Celeste Castillo Lee Legacy Webinar Series "Who Do You See When You See Me." Ms. Grandinetti, Dr. Gee, Ms. Roberts, and Dr. Jonathan Himmelfarb, MD, FASN's reiterated the importance of honoring the humanity of every person that the medical community touches. This webinar series will ensure that Celeste's voice and vision continue to guide KHI's work.



Several members made impactful individual contributions to KHI projects in 2021 including, Mary Baliker, Vanessa A. Evans, Amanda Grandinetti, Glenda V. Roberts, Derek L. Forfang, Jack Lennon, and Patrick O. Gee. The entire PFPC provided invaluable input during the developmental phase of the Artificial Kidney Project.

The PFPC continues to grow as we take on larger initiatives. In the beginning of this year, we welcomed Ms. Evans and Leigh-Ann Williams, MS, MPH, both home hemodialysis patients and champions, to our ranks. In November, accomplished kidney transplant recipients Austin Lee and Curtis Warfield, MS, accepted invitations to join the growing council. The addition of these members in 2022 will be crucial to advancing our mission.

As my final year on the PFPC comes to an end, I would like to thank all of my PFPC colleagues and KHI's Board of Directors members for your support, encouragement, and fellowship during my tenure. Serving with all of you was an honor and a privilege that I will always cherish. I also wish to thank KHI's members and partner organizations as well as FDA and all of the Federal agencies that have supported and continue to support our work.

Sincerely,

David M. White

Chair, Patient and Family
Partnership Council



Stakeholder's Meeting Returns Virtually Highlighting Meeting Patients Where They Are and Health Equity

The Eighth Annual Stakeholder's Meeting was held virtually on June 23-24 centered around the theme of "Meeting Patients Where They Are." During this two-day event, KHI heard from members of the community who are focused on breaking down the barriers to health equity and creating patient-centered development programs. Additionally, we heard from our partners at each FDA center about advances in kidney innovations and the journey three members took to commercialize their transformative innovations. Recordings from the meeting can be viewed [here](#).



Patient Preferences in Novel Kidney Device Development Nears Completion

Access to scientifically rigorous patient preference information could inform the decisions of industry and regulators in the design and evaluation of new devices for people with kidney failure. This year marked the second year of a three-year contract between the FDA and KHI to collect patient preference information on a hypothetical wearable dialysis device. Survey fielding through patient partner organizations was completed in October 2021, with 559 completed surveys. Survey results, including the Maximum Acceptable Risk that patients are willing to take for the benefits of a new wearable dialysis device, will be available by the end of the 2Q 2022. Several publications will highlight the study results and best practices.



Design Principles and Key Performance Indicators for Artificial Kidneys Work Begins

Artificial kidneys continue to grow in prominence as alternatives to dialysis treatment. Despite this increased attention, regulators, payors, and investors do not have a framework for the clinical or quality of life needs that artificial kidneys could address. Understanding the needs of people with kidney failure in a way that informs product design can strengthen the value of an artificial kidney product by incorporating patient choices.

KHI launched a project to apply principles of user-centered design to the artificial kidney landscape. Through a workshop, qualitative interviews, and a quantitative survey of people with kidney failure, KHI developed patient-centered design principles for artificial kidneys to help innovators trace design specifications back to user needs. Additionally, the research generated from this project will provide the kidney community a way to understand the relationship between individual patient needs with tools for defining the market for artificial kidney solutions.



Assessing Knowledge and Perception of Xenotransplantation in the Kidney Community

The advent of genome editing techniques has empowered xenotransplantation researchers to potentially overcome the rejection of xenogeneic organs by the immune system. This has created an opportunity for using modified porcine kidneys as a potential source of viable organs for patients with end-stage kidney disease. But, the patient and health care communities have a diversity of perceptions on this approach for kidney replacement. A clear baseline of current perceptions is needed by the innovator community to design first-in-human studies that address these concerns and perceptions. KHI initiated a project that aims to inform first-in-human xenotransplantation trials by engaging the research and development communities, care partners, and patients to assess current knowledge and perceptions of this potentially powerful technology.

New projects launched

- 1** Endpoints for C3 Glomerulopathy (C3G)
- 2** Xenotransplantation: Knowledge and Perception Assessment
- 3** Design Principles and Key Performance Indicators for Artificial Kidneys
- 4** Designing a Patient Advisory Board to Strengthen Clinical Trials and Research



**12 New
Member
Organizations**



**9
Publications**

**130 Volunteers
Participating in
Workgroups**

















**Presented at
9 Meetings**



**6
Completed
Projects**



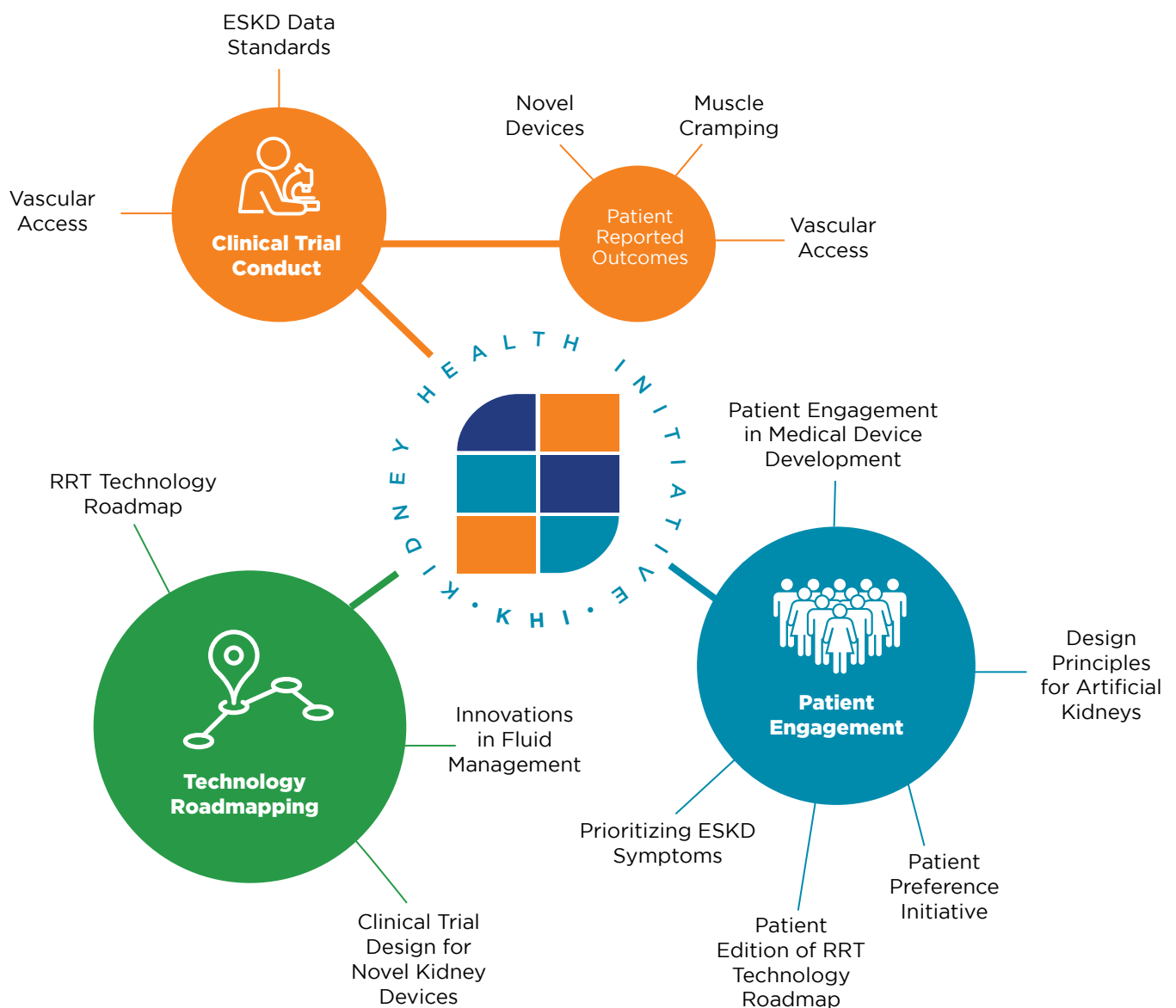
The community continues to make strides in drug development for people living with kidney disease. KHI continues to focus on clinical trial design, clinical trial endpoints, and biomarkers as integral areas for community alignment. In 2021, KHI launched its first project focused on the natural history for a rare kidney disease to identify key endpoint candidates for clinical trials and began work on a consensus building effort to prioritize areas of interest for pediatric kidney diseases.

	Status	First Therapy Approval
Rare Kidney Diseases		
Membranous Nephropathy	 2015	
Lupus Nephritis	 2018	 January 2021
IgA Nephropathy	 2019	 December 2021
Primary Hyperoxaluria	 2020	 November 2020
Enteric Hyperoxaluria		
C3 Glomerulopathy		
Focal Segmental Glomerulosclerosis		
Biomarkers		
Acute Kidney Injury		
Cross-cutting Community Barriers		
Prioritizing ESRD Symptoms	 2018	
Including People with Kidney Disease in Cardiovascular Clinical Trials	 2021	
Pediatric Drug Development		



KHI member organizations and companies at the center of transformative device innovation need to start with incorporating the perspective of people with kidney diseases. KHI's work elevating the patient perspective in device development informed the Technology Roadmap for Innovative Approaches to Kidney Replacement Therapy, an effort that connects subsequent clinical trial endpoints and patient reported outcomes (PROs) projects. In 2021, KHI continued its collaboration with the kidney community to understand patient perspectives for kidney replacement therapies and launched a new project to develop design principles and key performance indicators for artificial kidneys. Additionally, three projects were completed including PROs for dialysis vascular access, PROs for muscle cramping in patients on dialysis and end-stage kidney disease data standards.

Device Portfolio



MEMBER STAKEHOLDERS

Thank you to all our member stakeholders. Our stakeholders are part of an innovation community at the center of transformative issues that are catalyzing innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases.

FEDERAL PARTNERS



U.S. Department
of Veterans Affairs

DEVICE MANUFACTURERS AND BIOTECH COMPANIES



Vascular Therapies



KIDNEY CARE AND DIALYSIS PROVIDERS



PHARMACEUTICAL COMPANIES



NON-PROFITS AND DIGITAL HEALTH/AI COMPANIES



HEALTH CARE PROFESSIONAL ORGANIZATIONS



CONTRACT RESEARCH ORGANIZATIONS



FOUNDATIONS AND PATIENT ORGANIZATIONS



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Thank you to the Board of Directors, Project Workgroup Chairs, and the Patient & Family Partnership Council for making this work possible.

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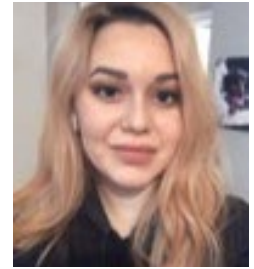
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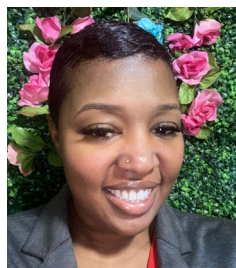
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