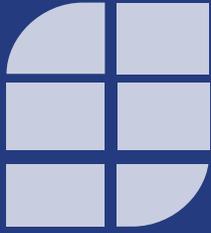


20
22



**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 almost 90 member companies and organizations committed to catalyzing innovation and the development of safe and effective patient-centered therapies for people with kidney diseases.

CONTENTS

- 4** **Message from the Chair**
- 5** **Message from the Chair of the Patient and Family Partnership Council**
- 6** **2022 Impact & Highlights**
- 10** **Catalyzing Drug Development**
- 10** **Catalyzing Device Development**
- 11** **Catalyzing Biologics Development**
- 12** **Member Organization**
- 14** **KHI New Member Organizations 2022**
- 15** **Acknowledgments**

MESSAGE FROM THE CHAIR

It has been an eventful year for the Kidney Health Initiative. I am completing my term as chair of the Board of Directors and am delighted to pass the baton to Uptal Patel, M.D. We have also realigned the Board of Directors to more closely respond to the needs and concerns of KHI members with interests in drugs, biologics or devices.

KHI has undertaken a number of important projects during the past year that align with its mission and strategic priorities, including projects relating to knowledge and perception assessment of xenotransplantation, a human centered design toolkit for kidney failure, and endpoints for Complement 3 Glomerulopathy and for pediatric IgA Nephropathy, to name but a few. It has been my honor to be associated with KHI and I look forward to its continued success.

Sincerely,



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



MESSAGE FROM THE CHAIR OF THE PATIENT AND FAMILY PARTNERSHIP COUNCIL

In 2022, KHI's Patient and Family Partnership Council (PFPC) focused on strengthening their framework and processes to ensure that the PFPC will remain strong, continue to grow, and carry-on Celeste Castillo Lee's legacy. There was a large focus on updating governance and developing guidelines to firmly ground the PFPC and continue to bolster itself in the community. The PFPC continued to evolve the Patient Advisory Board (PAB) working group by planning and developing content so that the nephrology innovation community can continue to embed the patient voice into all aspects of care.

The PFPC mission continues to strengthen and advance. At the beginning of 2022, we welcomed Austin Lee and Curtis Warfield, MS, as new members to the PFPC. Both have strong backgrounds in kidney transplantation. Patrick O. Gee, Sr., PhD, became our Vice Chair. Patrick, Curtis, and Austin's passion and tenacity will help further the mission of the PFPC.

Thank you to Mary Baliker, Curtis, and Jack Lennon, MBA, for devoting so much time and energy into the PFPC Governance Task Force. The Governance Task Force analyzed, revised, and created streamlined or new processes for the nomination period, new members, current members, and Chair and Vice Chair roles. All members voted on these processes to ensure they were fair, transparent, and thoughtful. All PFPC members continued to work on the PAB working group. The PFPC also had a large presence at the KHI's meeting. Curtis spoke at our Annual Stakeholder Meeting and Leigh-Ann Williams, MS, MPH, Glenda V. Roberts, and Patrick presented at ASN Kidney Week in Orlando, Florida.

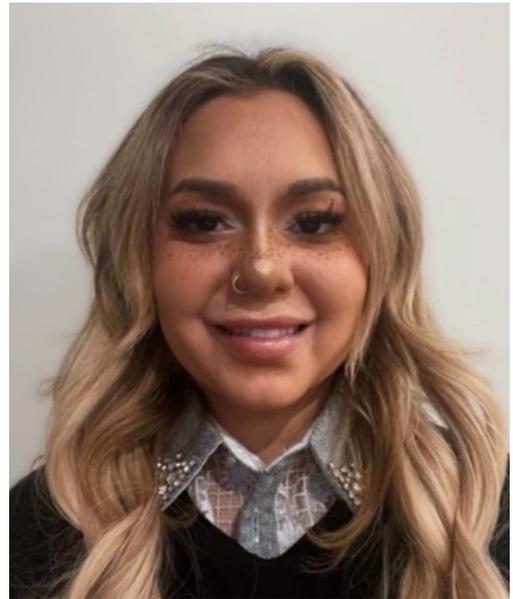
Thank you, Patrick, for operationalizing the 2022-year mission. Thank you to all PFPC members for their time, dedication, and hard work.

As my final year with the PFPC comes to an end, I'd like to thank all members of the PFPC, KHI staff, the FDA, and all KHI partner organizations. It was an honor to work with each and every one of you. I look forward to watching the PFPC continue to reach their goals and seeing what they will achieve in the future.



Amanda Grandinetti, MPH

Chair, Patient and Family Partnership Council



2022 IMPACT & HIGHLIGHTS



KHI Hosts Stakeholder Meeting Highlighting Clinical Trial Diversity

The Stakeholder Meeting to **Break Down Clinical Trial Barriers to Achieve Health Equity** was held virtually on September 28, 2022. This meeting included presentations, panel discussions with live Q&A, and a networking event that focused on clinical trial diversity and community led initiatives to overcome barriers. The full recording from the event can be viewed [here](#).



KHI Holds Three Sessions at ASN Kidney Week in Orlando, FL

1. Advancing Clinical Trials for People with Kidney Diseases: An Overview

This session was hosted by KHI and included Barbara Gillespie, MD, FASN (KHI Board of Directors), Glenda V. Roberts (KHI Patient and Family Partnership Council), Aliza Thompson, MD, MS (FDA Liaison to KHI Board of Directors), and Opeyemi Olabisi, MD, PhD (Duke University) to discuss strategies for clinical trials. The presentations included overviews on: surrogate endpoints and regulatory pathways for drug development, the importance of well-designed Patient Advisory Boards, and community engagement strategies for increasing clinical trial participation.

2. Building Capacity to Collect Patient Preferences Information for Innovative Kidney Devices

This session was moderated by ASN's Melissa West with Board of Directors Member Jennifer E. Flythe, MD, MPH, FASN, Carol Mansfield, PhD, and Michelle Tarver, MD, PhD as speakers.

This session discussed the FDA guidance for collecting patient preference information, reviewed methodological approaches, formative qualitative research, results of the research and discussed the risk and best practices for online surveys.

3. Future Shock: Transformational Therapies for Kidney Failure

This session was moderated by Board of Directors Chair Raymond C. Harris, MD, FASN, and ASN's Melissa West. Jonathan Himmelfarb, MD, FASN, Shuvo Roy, PhD, Jeremy S. Duffield, MBChB, MD, PhD, and PFPC member Leigh-Ann Williams, MS, MPH.

2022 IMPACT & HIGHLIGHTS



Patient Preferences in Novel Kidney Device Development Completed

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 completion of a three-year contract between the FDA and KHI to collect patient preference information on a hypothetical wearable dialysis device. Several publications will highlight the study results and best practices.



Publications



Click on any publication to open and read the link.

1. Development of a Patient Preference Survey for Wearable Kidney Replacement Therapy Devices
2. Kidney Health Initiative ESKD Data Standards Project
3. Conceptual Framework for Patient-Reported Outcome Measures in Clinical Trials of Skeletal Muscle Cramping Experienced in Dialysis
4. Biomarkers Reported to be Useful in Acute Kidney Injury
5. Roadmap for Accelerating the Development of Biomarkers for Acute Kidney Injury
6. Reducing the Risks of Home Dialysis Innovation and Uptake
7. Patient Perspective on Xenotransplantation



**5 New
Member
Organizations**



**4
Completed
Projects**

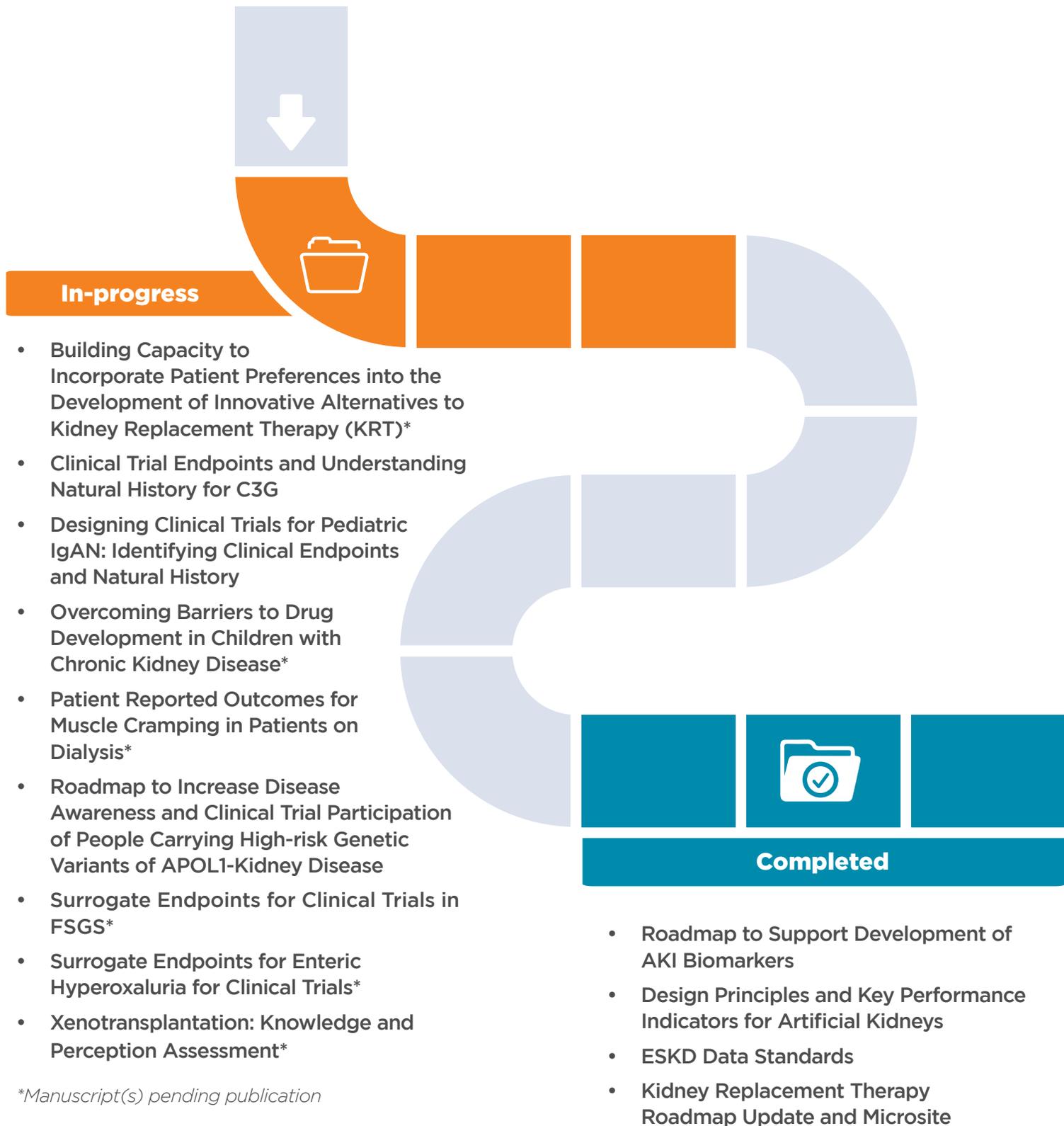


**51 Volunteers
Participating in
Workgroups**



**7
Publications**

2022 IMPACT & HIGHLIGHTS



2022 IMPACT & HIGHLIGHTS

New projects launched

1. Clinical trials in pediatric patients with IgA Nephropathy (IgAN)

In recent years, there has been a dramatic increase in drug development for IgAN; however, clinical trials are largely being conducted in adults. At this time, it is not clear whether the efficacy and safety of these treatments should also be assessed in children with IgAN, and, if so, how best to design studies to obtain the data needed to support use of novel therapies in this population. KHI's project approaches this issue by engaging subject matter experts to assess the current literature and trial landscape to formulate a community consensus on a path forward for developing therapies for children with IgA Nephropathy. Additionally, the project team will engage stakeholders who have clinical trial or registry data that can be used to inform natural history of disease research or identify biomarkers candidates and outcome measurements that advance therapies for children living with IgA Nephropathy. This data would then be shared to a central repository for analysis as a potential partnership.

2. Roadmap to increase disease awareness and clinical trial participation of people carrying high risk genetic variants of APOL-1 associated nephropathy

People with recent West African ancestry have an increased risk for developing kidney disease, with an estimated 4 in 10 Black Americans on dialysis having kidney failure caused by APOL1. Genetic risk factors include two high-risk variants of the APOL1 gene, which are found almost exclusively in people with recent West sub-Saharan African ancestry. 10%–15% of the Black population have a high-risk APOL1 genotype and up to 3% of the Black population may be affected.

This project will develop a roadmap that coordinates cross-sector partnerships to increase disease awareness and participation in APOL1-associated nephropathy clinical trials. Inclusivity of the entire community at-risk of APOL1 associated nephropathy, particularly those who may receive care in community health clinics, is an important principle that informs the design and utility of this project. Planned for completion in 2023, this resource will describe a collaborative APOL1 clinical trial engagement agenda that outlines: a) roles and key contributions of each stakeholder, b) approaches for engaging each stakeholder into an APOL1 focused collaboration, c) partnerships between stakeholder groups to support and sustain efforts that provide awareness and encourage APOL1 clinical trial participation, and c) resources that are needed for each stakeholder or can be shared by other stakeholders to strengthen outreach and engagement efforts. The intended audience of this roadmap includes trial sponsors, trial principal investigators, patient advisory boards, clinical care teams, community health clinics and other community organizations. This roadmap could also serve as a framework for other kidney clinical trial efforts that focus on communities of color.

CATALYZING DRUG DEVELOPMENT

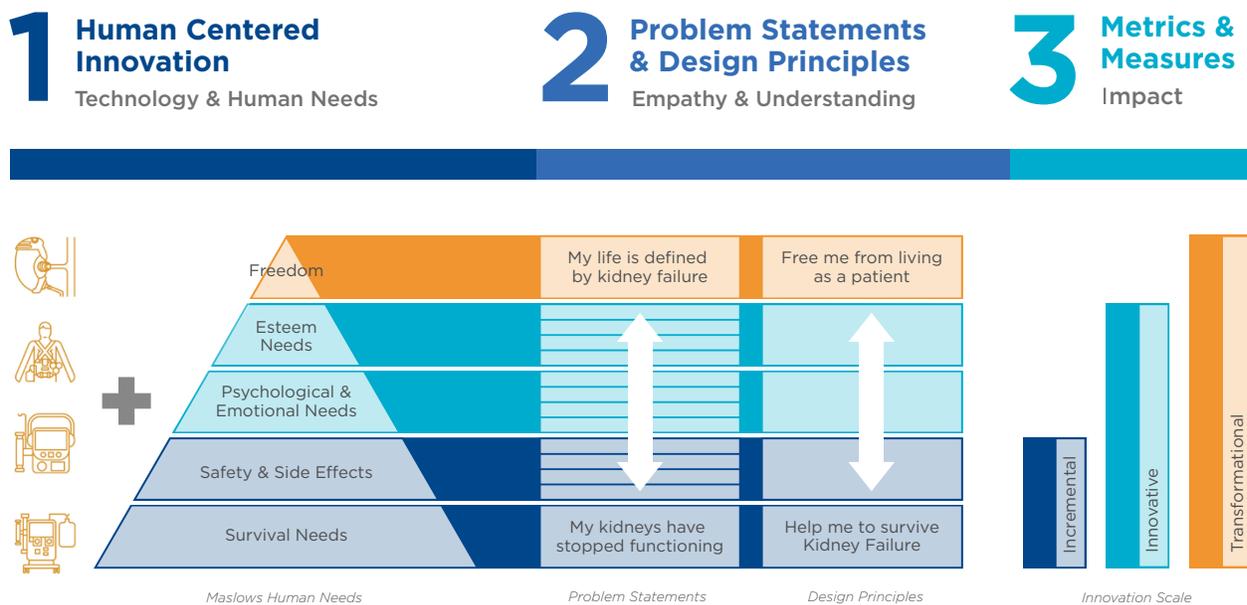
The community continues to make strides in drug development for people with kidney diseases. KHI continues to focus on clinical trial design, clinical trial endpoints, and biomarkers as integral areas for community alignment. In 2022, KHI continued its focus on the natural history for a rare kidney disease to identify key endpoint candidates for clinical trials and a consensus building effort to prioritize areas of interest for pediatric kidney diseases.

CATALYZING DEVICE DEVELOPMENT

Human-Centered Design Toolkit for Kidney Failure

Changing a person with kidney failure’s story to one of hope is the purpose of kidney replacement therapy innovations. To do that, product developers and researchers need to understand these stories. Human-centered design is a tool for gleaning needs and problem statements from consumer’s stories and integrating them into the design of products. The [Human-Centered Design Toolkit for Kidney Failure](#) provides innovators developing kidney replacement therapies with empathy tools to facilitate their interactions with people with kidney failure to enable market segmentation and provide a framework for a customer requirements document.

The Toolkit is intended to help build better alternatives to conventional dialysis by better understanding the problems people with kidney failure face.



CATALYZING BIOLOGICS DEVELOPMENT

Biologics are often not easily identified or characterized, and many are manufactured using biotechnology, including blood components and derivatives, vaccines, allergenics, cellular and gene therapies, and tissues for transplantation. These include numerous products that are living microorganisms, cells or tissues from humans or animals, and products that are derived from them, such as stem cells and genetically engineered immune cells.

These products frequently represent cutting-edge biomedical research and, in time, may offer the most effective means to treat a variety of medical illnesses and conditions that presently have few or no other treatment options.

KHI's inaugural project for Assessing Knowledge and Perception of Xenotransplantation represents the first step in addressing stakeholder needs. Future KHI initiatives may focus on communicating the risk/benefits of new biotechnologies that currently have limited clinical information.

MEMBER ORGANIZATIONS

FEDERAL PARTNERS



U.S. Department of Veterans Affairs

DEVICE MANUFACTURERS, DIAGNOSTICS COMPANIES, BIOTECH COMPANIES, AND DIGITAL HEALTH



Vascular Therapies



Wearable Artificial Organs, Inc.

KIDNEY CARE AND DIALYSIS PROVIDERS



PHARMACEUTICAL COMPANIES



MEMBER ORGANIZATIONS

NON-PROFITS



HEALTH CARE PROFESSIONAL ORGANIZATIONS



CONTRACT RESEARCH ORGANIZATIONS



FOUNDATIONS AND PATIENT ORGANIZATIONS



RESEARCH INSTITUTIONS



NEW KHI MEMBER ORGANIZATIONS 2022

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



Invizius: Invizius' proprietary H-Guard® biotechnology aims to transform care on dialysis and other extra-corporeal treatments by reducing inflammation.



Maze Therapeutics: Maze Therapeutics uses human genetics, functional genomics and data science to inform treatment for patients with severe rare and common diseases. They combine their COMPASS platform with drug and technology innovation to develop medicines for these diseases.



Pulse Data: Pulse Data's AI technology advances diagnosis and prediction of adverse effects for cardiorenal patients.



Sanofi: Sanofi is an innovative healthcare company focused on creating life-changing treatments and vaccines for millions of people globally.



Talaris Therapeutics: Talaris is a late-clinical stage cell therapy company developing new therapies for organ transplantation and severe immune and blood disorders.

ACKNOWLEDGMENTS

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

BOARD OF DIRECTORS AND FEDERAL LIAISONS

Paul T. Conway

American Association of Kidney Patients

Bruce Culleton, MD

CVS Kidney Care

Jennifer E. Flythe, MD, MPH, FASN

University of North Carolina Kidney Center

Kevin J. Fowler

Voice of the Patient, Inc.

Barbara S. Gillespie, MD, FASN

Labcorp

Nicolas J. Guzman, MD

AstraZeneca

Raymond C. Harris, MD, FASN

Vanderbilt Center for Kidney Disease
KHI Chair

Meg Jardine, MBBS, PhD

University of Sydney

Annamaria T. Kausz, MD, MS

ATK Pharma Strategies LLC

Jeffrey Lawson, MD, PhD

Duke University

Vandana Dua Niyayar, MD, FASN

Emory University

Thomas D. Nolin, PharmD, PhD

University of Pittsburgh

Ikenna Okezie, MD, MBA

Somatus, Inc.

Uptal Patel, MD

AstraZeneca

Alain Romero, PhD, PharmD

Global Blood Therapeutics

Amit Sharma, MD, FASN

Bayer Pharmaceuticals

Howard Trachtman, MD, FASN

University of Michigan

Amy Young

DaVita Clinical Research

Frank P. Hurst, MD, FASN

Center for Drug Evaluation and Research (CDER), FDA

Meda E. Pavkov, MD, PhD

Centers for Disease Control (CDC)

Prabir Roy-Chaudhury, MD, PhD FASN

University of North Carolina Kidney Center

ASN Council Liaison

Kristen Miller, PharmD

Office of Medical Policy, CDER, FDA
FDA Point of Contact

James P. Smith, MD, MS

Center for Drug Evaluation and Research (CDER), FDA

Robert A. Star, MD

National Institute of Diabetes and Digestive Kidney Diseases (NIDDK)

Aliza M. Thompson, MD, MS

Center for Drug Evaluation and Research (CDER), FDA

Celia Witten, MD, PhD

Center for Biologics Evaluation and Research (CBER), FDA

EX OFFICIO

Amanda Grandinetti, MPH

KHI Patient and Family Partnership Council Chair

PROJECT WORKGROUP CHAIRS AND STEERING COMMITTEE MEMBERS

Ogo Egbuna, MD

Vertex Pharmaceuticals

Jennifer E. Flythe, MD, MPH, FASN

University of North Carolina Kidney Center

Patrick O. Gee, Sr., PhD, JLC

KHI Patient and Family Partnership Council, APOLLO Community Advisory Committee, Evidation Health

Stuart Goldstein, MD

Cincinnati Children's Hospital Medical Center

Keisha Gibson, MD, MPH, FASN

University of North Carolina Kidney Center

Barbara Gillespie, MD, MS

Labcorp, University of North Carolina

Raymond C. Harris, MD, FASN

Vanderbilt University

Eduardo K. Lacson, Jr., MD, MPH

Dialysis Clinic, Inc.

Christine Lee, PhD, PharmD

FDA-liaison, Office of Minority Health and Health Equity

John C. Lieske, MD, FASN

Mayo Clinic

Laura Mariani, MD, MS

University of Michigan

Dawn S. Milliner, MD

Mayo Clinic

Susanne B. Nicholas, MD, PhD

ASN Health Care Justice Committee (Clinical Care and Innovation Team); University of California at Los Angeles

Carla M. Nester, MD, MS, FASN

University of Iowa

Uptal D. Patel, MD

AstraZeneca

Michelle M. Richardson, PharmD, BCPS

Tufts Medical Center

Glenda V. Roberts

APOLLO Community Advisory Committee, Center for Dialysis Innovation, Kidney Research Institute

Michelle Tarver, MD, PhD

FDA-liaison, Center for Devices and Radiological Health

Aliza M. Thompson, MD, MS

FDA-liaison, Center for Drug Evaluation and Research

Joshua M. Thurman, MD

University of Colorado

Howard Trachtman, MD, FASN

University of Michigan

Katrin Uhlig, MD, MS

Agios Pharmaceuticals

Eric W. Young, MD

Arbor Research Collaborative for Health

ACKNOWLEDGMENTS

PATIENT & FAMILY PARTNERSHIP COUNCIL



Mary Baliker



Vanessa A. Evans



Derek Forfang



Patrick O. Gee, Sr., PhD
Vice Chair



Amanda Grandinetti, MPH
Chair



Austin Lee



Jack Lennon, MBA



Glenda V. Roberts



Curtis Warfield, MS



Leigh-Ann Williams, MS, MPH

STAFF



Mark D. Lim, PhD, PMP
Vice President,
Research, Discovery,
and Innovation



Lynee Galley
Director, Research,
Discovery, and
Innovation



Cesia Portillo, MPH, CHES
Program Coordinator,
Kidney Health Initiative



Meaghan Malley
Program Manager,
Kidney Health Initiative



Zachary Cahill
Artificial Kidney
Product Specialist,
Research, Discovery, and
Innovation



Abigail Martin
Program Associate,
Research, Discovery,
and Innovation



Elisheba Wilson
Administrative Associate,
Research, Discovery,
and Innovation

This page is intended to be blank.



kidneyhealthinitiative.org

1401 H St NW, Suite 900
Washington, DC, 20005
khi@asn-online.org