Member Update

June 10, 2020

KHI KIDNEY HEALTH INITIATIVE

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

Kristen Miller, PharmD
FDA Point of Contact

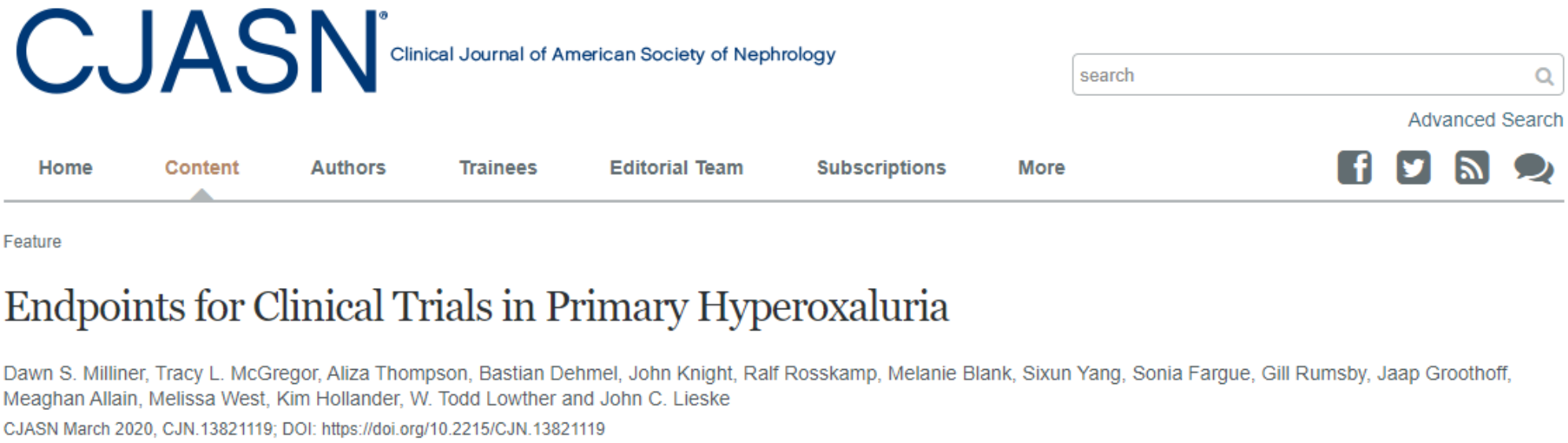
Melissa West
ASN Acting Vice President
Research, Discovery
and Innovation

Welcome & Opening Statement


2019
2020

Endpoints for Clinical Trials in Primary Hyperoxaluria

Led by: Dawn Milliner and John Lieske



CJASN Clinical Journal of American Society of Nephrology

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Feature

Endpoints for Clinical Trials in Primary Hyperoxaluria

Dawn S. Milliner, Tracy L. McGregor, Aliza Thompson, Bastian Dehmel, John Knight, Ralf Rosskamp, Melanie Blank, Sixun Yang, Sonia Fargue, Gill Rumsby, Jaap Groothoff, Meaghan Allain, Melissa West, Kim Hollander, W. Todd Lowther and John C. Lieske

CJASN March 2020, CJN.13821119; DOI: https://doi.org/10.2215/CJN.13821119

https://cjasn.asnjournals.org/content/early/2020/03/11/CJN.13821119

KHI KIDNEY HEALTH INITIATIVE

Patient-friendly Roadmap

Led by: David M. White and the Roadmap Patient Advisory Committee

https://khi.asn-online.org/uploads/KHI_RRTTechnologyRoadmap_PatientEdition.pdf

Fluid Management Supplement to Roadmap

Led by: Derek Forfang and Yossi Chait

https://khi.asn-online.org/uploads/KHI_InnovationsInFluidManagement.pdf

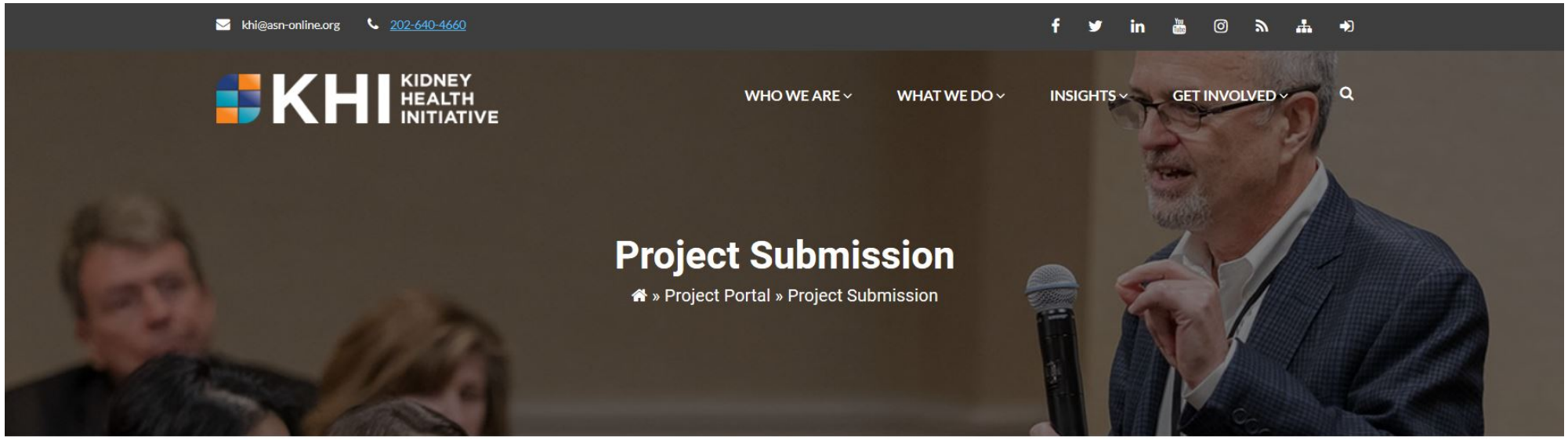
Patient-Reported Outcome Measures for Novel Renal Devices

Led by: Jennifer Flythe

https://cjasn.asnjournals.org/content/early/2020/04/09/CJN.00110120

KHI KIDNEY HEALTH INITIATIVE

KHI PROJECT SUBMISSION PROCESS



PROJECTS

CURRENT PROJECTS

PROJECT PROPOSALS

PROJECT SUBMISSION

CALL FOR WORKGROUPS

KHI Project Submission

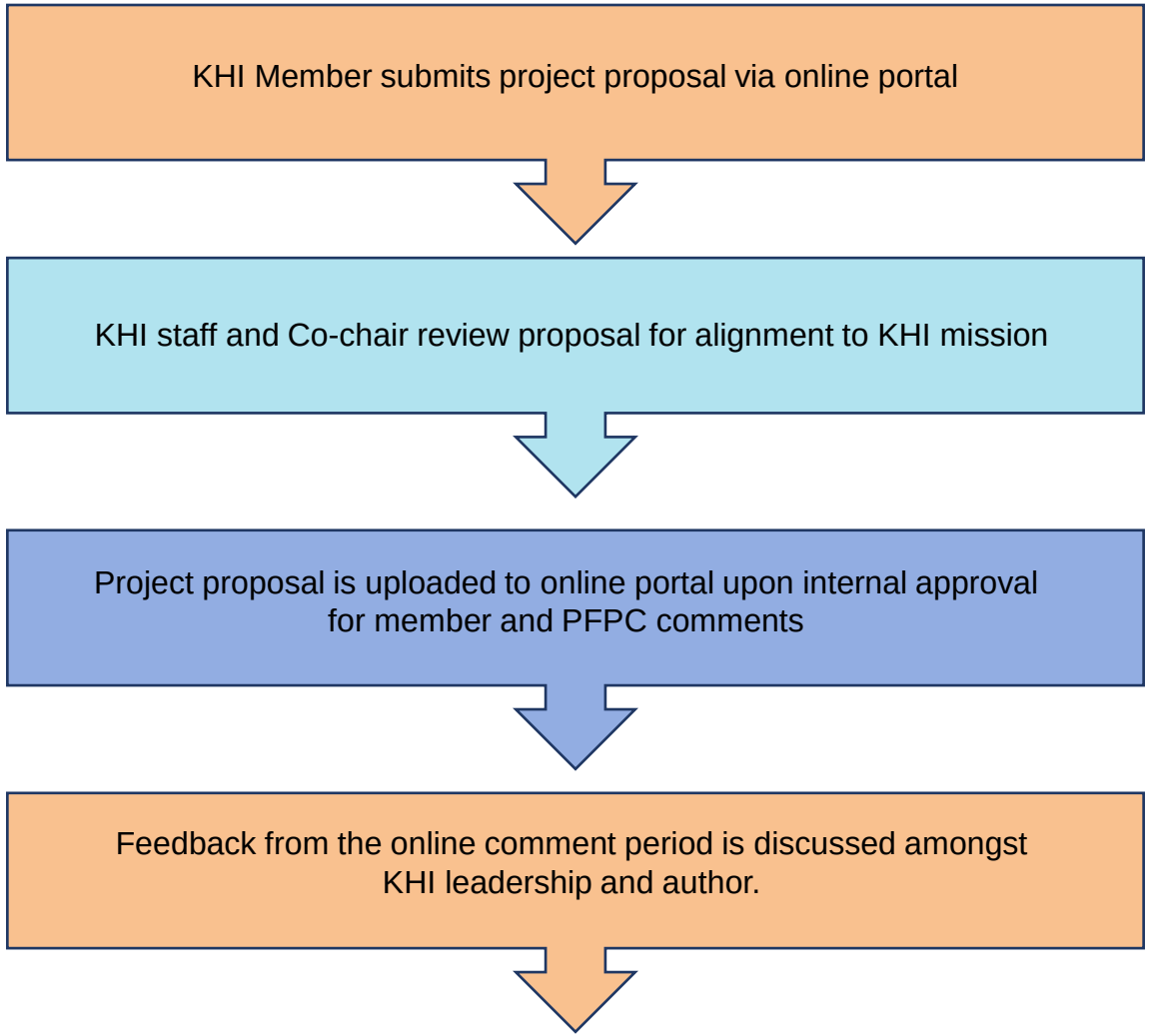
Welcome to the Kidney Health Initiative Project Submission Portal. KHI seeks to advance its mission through innovative, collaborative projects that seek input from and bring together members across all areas of the kidney community.

We encourage KHI members to submit their ideas for projects via this portal. The portal is open a rolling basis for submissions. In order to review and comment on current proposals please visit the Project Proposal page.

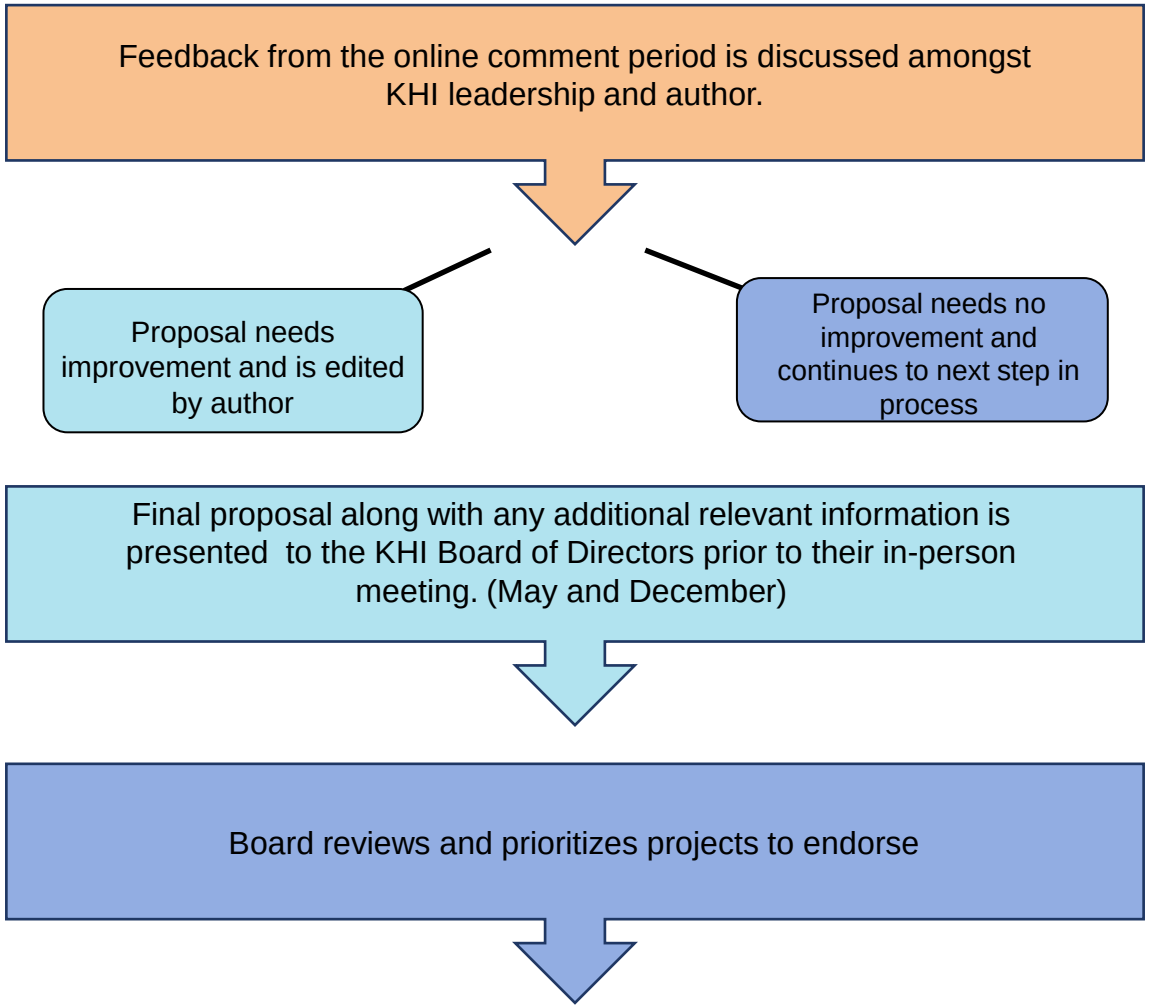
https://khi.asn-online.org/projects/submission.aspx

KHI KIDNEY HEALTH INITIATIVE

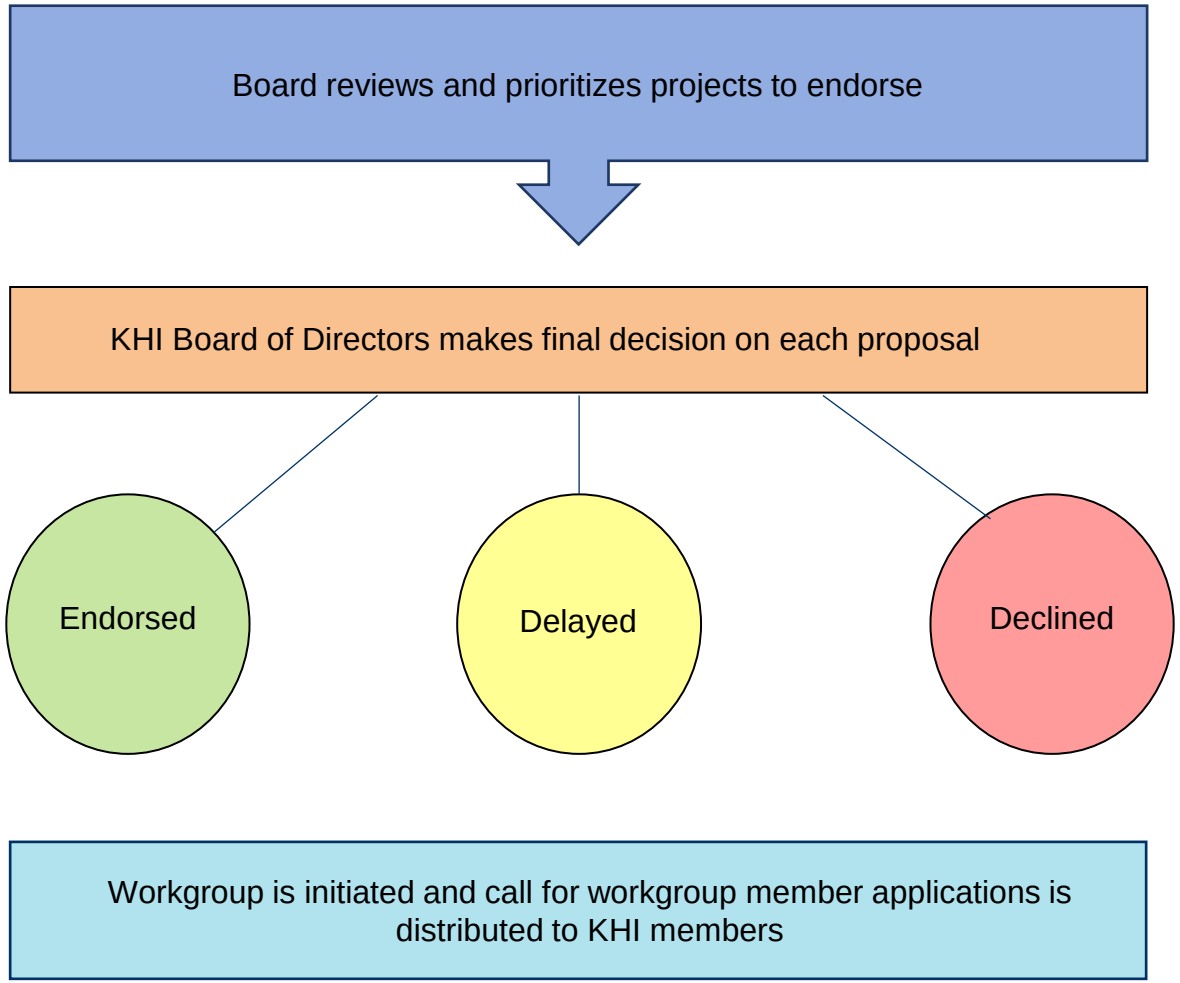
KHI PROJECT SUBMISSION PROCESS



KHI PROJECT SUBMISSION PROCESS



KHI PROJECT SUBMISSION PROCESS



GOVERNMENT PARTNERS

MEMBER ORGANIZATIONS

FOUNDATIONS AND PATIENTS ORGANIZATIONS

Alport Syndrome Foundation
AAKP American Association of Kidney Patients
American Kidney Fund
Dutch Kidney Foundation
HDU Home Dialyzors United
JDRF
National Kidney Foundation
NEPHCURE Kidney International
Oxalosis & Hyperoxaluria Foundation
PKD Foundation Polycystic Kidney Disease
Vasculitis Foundation

RESEARCH INSTITUTIONS

Arbor Research Collaborative for Health
Center for Dialysis Innovation
Duke Clinical Research Institute
Kidney Research Institute
A collaboration between Northwest Kidney Centers and UW Medicine
Massachusetts General Hospital Kidney Research Center
The George Institute for Global Health
NAPRTCS
UNC Health Care Kidney Center

HEALTH CARE PROFESSIONAL ORGANIZATIONS

accp American College of Clinical Pharmacy
ANNA American Nephrology Nurses Association
ASEA
ASDIN
ASN American Society of Nephrology
American Society of Pediatric Nephrology
AST American Society of Transplantation
era edta
NRAA
nncc Nephrology Nursing Certification Commission
RPA Renal Physicians Association
termis
VASA Vascular Access Society of the Americas
Access Excellence

NON-PROFITS AND DIGITAL HEALTH/ AI COMPANIES

KDIGO Kidney Disease Improving Global Outcomes
mec
patientMpower empowering better outcomes
pulseData

KHI Kidney Health Initiative

MEMBER ORGANIZATIONS

DEVICE MANUFACTURERS AND BIOTECH COMPANIES

advent access, Antaros Medical, ARTARA THERAPEUTICS, AVENU MEDICAL, B BRAUN SHARING EXPERTISE, Baxter, BD, bioporto DIAGNOSTICS, DIALITY, FAST BioMedical, graftworx, HUMACYTE, INNAVASC InnAVasc Medical, Inc., inRegen, Laminate Medical Technologies, MediBeacon, Medtronic, Vascular Therapies, vexev vascular, Galanthus Pte Ltd, Wearable Artificial Organs, Inc., GORE Creative Technologies Worldwide

PHARMACEUTICAL COMPANIES

ARDELYX, Akebia THERAPEUTICS, Allena PHARMACEUTICALS, Alnylam PHARMACEUTICALS, AMGEN, angion, AstraZeneca, BAYER, Boehringer Ingelheim, calliditas THERAPEUTICS, ChemoCentryx, CHINOOK THERAPEUTICS, Complexa, Dicerna, Dimerix, GILEAD, Goldfinch Bio the kidney company, HANSA BIOPHARMA, janssen Johnson & Johnson, KBP BIOSCIENCES, Mallinckrodt, NOVARTIS, Otsuka, Oxidien Pharmaceuticals, OxThera, Pfizer, REATA PHARMACEUTICALS, relypsa A Vifor Pharma Group Company, Retrophin, SANOFI, VERTEX, VIELABIO

DIALYSIS PROVIDERS

CVS Health, DaVita, DCI Dialysis Clinic, Inc. A Non-Profit Corporation, FRESENIUS MEDICAL CARE, PeaceHealth St. John Medical Center, Rogosin Institute, SATELLITE HEALTHCARE DIALYSIS · WELLBOUND · RESEARCH, SOMATUS REVOLUTIONIZING KIDNEY CARE

CONTRACT RESEARCH ORGANIZATIONS (CROs)

Clinipace CLINICAL RESEARCH, COVANCE SOLUTIONS MADE REAL, IQVIA

KHI KIDNEY HEALTH INITIATIVE

NEW MEMBER ORGANIZATIONS

DEVICE MANUFACTURERS AND BIOTECH COMPANIES

DIALITY

Galanthus Pte Ltd

inRegen

PHARMACEUTICAL COMPANIES

ARDELYX

Boehringer Ingelheim

CHINOOK THERAPEUTICS

Dicerna

Oxidien Pharmaceuticals

DIALYSIS PROVIDERS

CVS Health

CONTRACT RESEARCH ORGANIZATIONS (CROs)

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- Mr. Derek Forfang
- Mr. Patrick O. Gee
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- Mr. Jack Lennon
- Ms. Glenda Roberts
- Mr. David M. White, Chair

https://khi.asn-online.org/pages/?ID=1

KHI KIDNEY HEALTH INITIATIVE

KHI PATIENT AND FAMILY PARTNERSHIP COUNCIL

KHI KIDNEY HEALTH INITIATIVE

February 28, 2020

RADM Richardae Araojo
Associate Commissioner for Minority Health – Office of Minority Health and Health Equity
US Food and Drug Administration
10903 New Hampshire Ave., Bld. 32
Silver Spring, MD 20993

Re: Office of Minority Health and Health Equity Strategic Priorities Request for Comment

Dear RADM Araojo,

The members of the Kidney Health Initiative (KHI) Patient and Family Partnership Council (PFPC) would like to thank the US Food and Drug Administration (FDA) for the opportunity to submit recommendations on establishing strategic priorities for the Office of Minority Health and Health Equity (OMHHE), and we congratulate OMHHE on celebrating its 10th anniversary in 2020.

KHI, a public-private partnership between the American Society of Nephrology (ASN), the FDA, and over 100 companies and organizations, is committed to catalyzing innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases. The KHI PFPC is a group of people living with kidney diseases or serving as care partners who ensure that the desires and perspectives of patients and care partners are

Comment Letter:
FDA Office of Minority Health and Health Equity Strategic Priorities

KHI KIDNEY HEALTH INITIATIVE

May 1, 2020

Wendy Selig
Medical Device Innovation Consortium (MDIC)
1501 Wilson Blvd. Suite 910
Arlington, VA 22209

Re: Medical Device Innovation Consortium Maximizing Patient Input in the Design and Development of Medical Device Clinical Trials Request for Comment

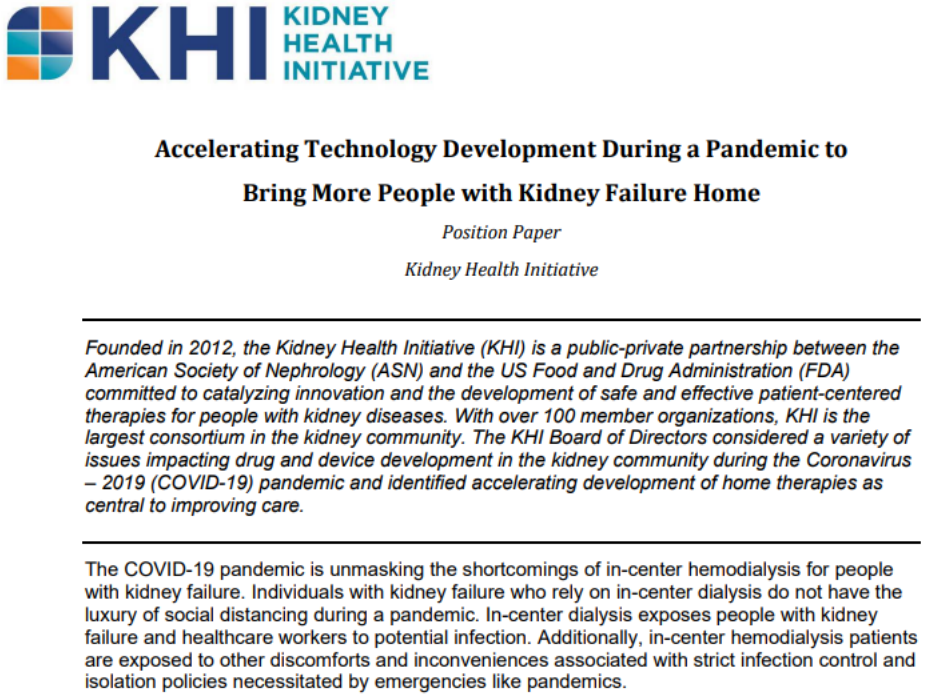
Dear Ms. Selig,

The members of the Kidney Health Initiative (KHI) Patient and Family Partnership Council (PFPC) would like to thank the Medical Device Innovation Consortium (MDIC) for the opportunity to submit comments on establishing evidence-based recommendations for the inclusion of patient choice into medical device development and trials. Patient-centeredness is the foundation of the KHI PFPC, and we commend your work.

KHI, a public-private partnership between the American Society of Nephrology (ASN), the US Food and Drug Administration (FDA), and over 100 member organizations, is the largest consortia in the kidney community committed to catalyzing innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases. The KHI PFPC is a group of people living with kidney diseases who ensure that the desires and perspectives of patients and care partners are honored in every stage of the kidney medical product development life cycle. Promoting patient engagement is critical for the development of

Comment Letter:
Medical Device Innovation Consortium
“Maximizing Patient Input in the Design and Development of Medical Device Clinical Trials”

Accelerating Technology Development During a Pandemic to Bring More People with Kidney Failure Home



KHI KIDNEY HEALTH INITIATIVE

Accelerating Technology Development During a Pandemic to Bring More People with Kidney Failure Home

Position Paper

Kidney Health Initiative

Founded in 2012, the Kidney Health Initiative (KHI) is a public-private partnership between the American Society of Nephrology (ASN) and the US Food and Drug Administration (FDA) committed to catalyzing innovation and the development of safe and effective patient-centered therapies for people with kidney diseases. With over 100 member organizations, KHI is the largest consortium in the kidney community. The KHI Board of Directors considered a variety of issues impacting drug and device development in the kidney community during the Coronavirus – 2019 (COVID-19) pandemic and identified accelerating development of home therapies as central to improving care.

The COVID-19 pandemic is unmasking the shortcomings of in-center hemodialysis for people with kidney failure. Individuals with kidney failure who rely on in-center dialysis do not have the luxury of social distancing during a pandemic. In-center dialysis exposes people with kidney failure and healthcare workers to potential infection. Additionally, in-center hemodialysis patients are exposed to other discomforts and inconveniences associated with strict infection control and isolation policies necessitated by emergencies like pandemics.

https://khi.asn-online.org/uploads/AcceleratingHomeDialysisTechnology_May2020_KHIPositionPaper.pdf

KHI KIDNEY HEALTH INITIATIVE

Making the Case for Change: Including People with Kidney Diseases in COVID-19 Trials

KHI KIDNEY HEALTH INITIATIVE

Making the Case for Change:

Including People with Kidney Diseases in COVID-19 Trials

Clinical trials often exclude people with kidney diseases. This means that 37 million people in the United States are rarely represented in the kind of research that advances change in treatment and care. The challenges that result from such exclusion are highlighted by the current COVID-19 crisis.

Preliminary studies indicate that people with kidney diseases demonstrate an increased risk (two- to sixteen-fold) for developing severe COVID-19 symptoms. These data support US statistics that show acute kidney injury occurs in up to 5% of hospitalized COVID-19 patients and 50-90% of patients in the intensive care unit. (reference) Responding effectively to the COVID-19 pandemic should include people with kidney diseases.

https://khi.asn-online.org/uploads/IncludingPeopleWithKidneyDiseasesInCOVID19Trials_KHIBoDStatement_051520.pdf

In Development
Ensuring the Resilience of Kidney Trials

- Maintain the investment and continue innovation in the kidney space.
- Provide resources for investigators and sponsors to be innovative and novel in continuing their research.

ONGOING ACTIVITIES - 2020

Endpoints

2020

- FSGS
- Enteric Hyperoxaluria

Completed

Moving Points in Nephrology

Recommended Clinical Trial End Points for Dialysis Catheters

Michael Allon, Deborah J. Brouwer-Maier, Kenneth Abreo, Kevin M. Baskin, Kay Bregel, Deepa H. Chand, Andrea M. Eason, Leonard Mermel, Michele H. Mokrzycki, Priti R. Patel, Prabir Roy-Chaudhury, Surendra Shenoy, Rudolph P. Valentini, and Haimanot Wasse

Moving Points in Nephrology

Definitions and End Points for Interventional Studies for Arteriovenous Dialysis Access

Gerald A. Beathard, Charmaine E. Lok, Marc H. Glickman, Ahmed A. Al-Jaishi, Donna Bednarski, David L. Cull, Jeffery H. Lawson, Timmy C. Lee, Vandana D. Nayyar, Donna Syracuse, Scott O. Trerotola, Prabir Roy-Chaudhury, Surendra Shenoy, Margo Underwood, Haimanot Wasse, Karen Woo, Theodore H. Yuo, and Thomas S. Huber

Establishing Surrogate Kidney End Points for Lupus Nephritis Clinical Trials: Development and Validation of a Novel Approach to Predict Future Kidney Outcomes

Meggan Mackay, Maria Dall'Era, Joanna Fishbein, Kenneth Kalunian, Martin Lesser, Jorge Sanchez-Guerrero, Deborah M. Levy, Earl Silverman, Michelle Petri, Cristina Arriens, Edmund J. Lewis, Stephen M. K., Fabrizio Conti, Vladimir Tesar, Zdenka Hruskova, Eduardo F. Borba, Eloisa Bonfa, Manish Rathi, K. L. Gupta, Vivekanand Jha, Sarfaraz Hasni, Melissa R. West, Frederic A. Houssiau, Juanita Romero-Diaz, Juan Mejia-Vilet, and Brad H.

Proteinuria Reduction as a Surrogate End Point in Trials of IgA Nephropathy

Aliza Thompson, Kevin Carroll, Lesley A. Inker, Jürgen Floege, Vlado Perkovic, Sonia Boyer-Suavet, Rupert W. Major, Judith I. Schimpf, Jonathan Barratt, Daniel C. Cattran, Barbara S. Gillespie, Annamaria Kausz, Alex W. Mercer, Heather N. Reich, Brad H. Rovin, Melissa West, and Patrick H. Nachman

KHI KIDNEY HEALTH INITIATIVE

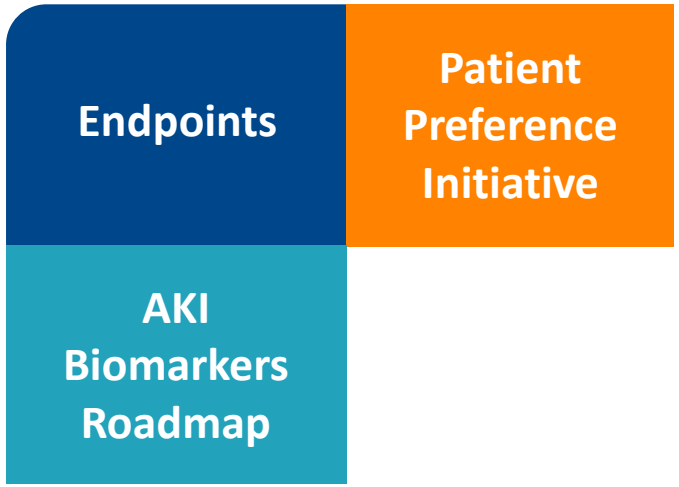
Endpoints | Patient Preference Initiative

Building Capacity to Incorporate Patient Preferences into the Development of Innovative RRT

To support the FDA and *Advancing American Kidney Health*'s goals, the deliverables of this project are:

- Prioritization of patient's perspective on benefits and risks of innovative wearable devices.
- Inventory of benefit – risk levels for device attributes.
- Results from a pilot study of patient preferences for innovative wearable devices.
- Develop a strategy to capture patient preference information as real world evidence.
- Publications on best practices and lessons learned from developing the survey.

Target End Date: 2021



Developing a Technology Roadmap to Catalyze the Development of Biomarkers for Acute Kidney Injury (AKI)

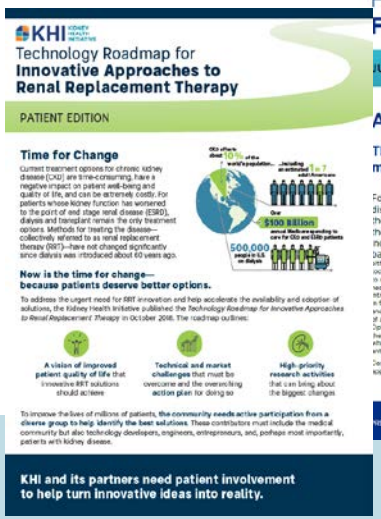
The roadmap is anticipated to include:

1. The Unmet Need for AKI Biomarkers
   a) Overview of challenges
   b) Trends, drivers, and opportunities for AKI biomarker use
   c) Contexts of Use
2. Gaps / Challenges to Advancing AKI Biomarkers
3. Proposed Activity Timelines and Activities to Catalyze the Development

Target End Date: Q1 2021

ONGOING ACTIVITIES - 2020

Endpoints	Patient Preference Initiative
AKI Biomarkers Roadmap	RRT Roadmap



2020 Updates

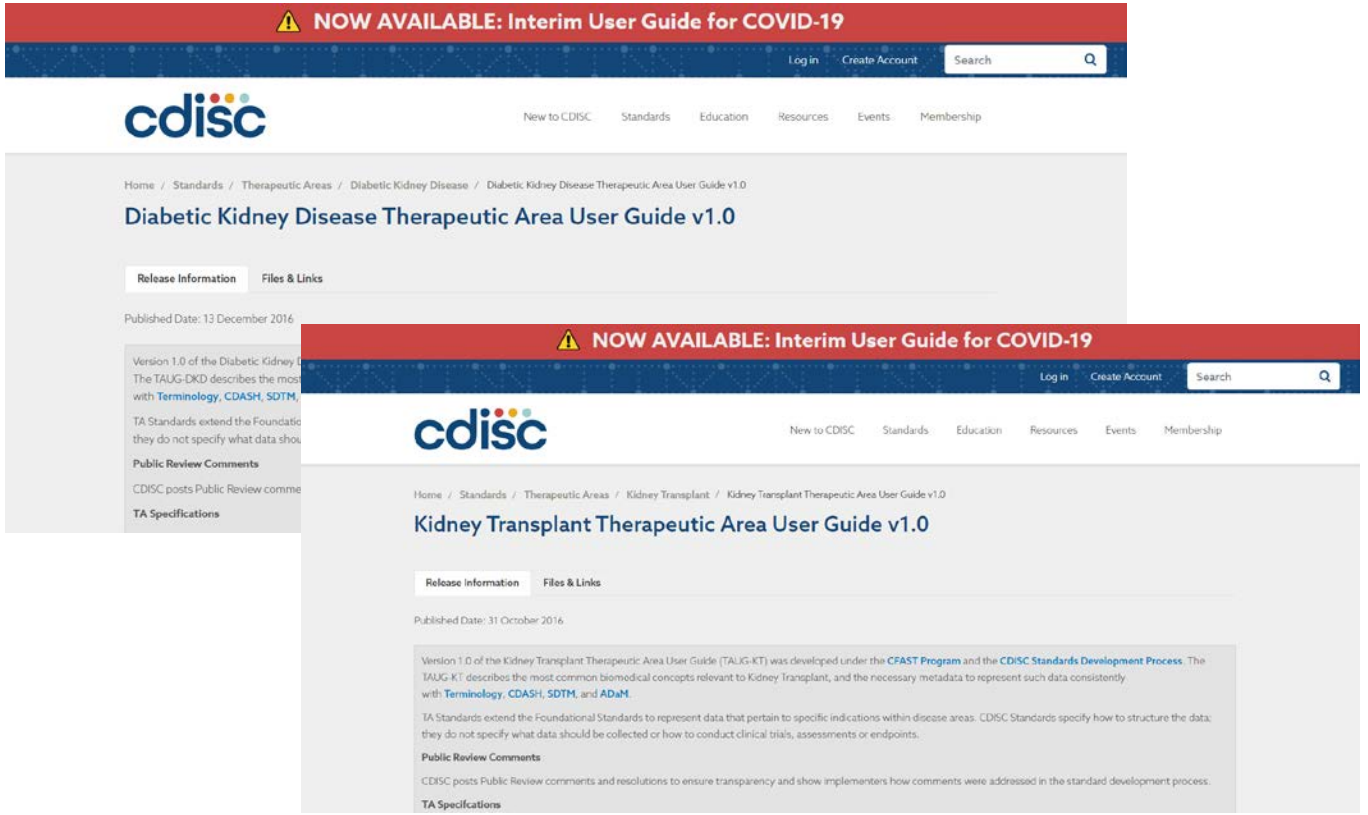
1. Community Feedback on Topics for Additional Supplements
2. Proposed Definitions for Portable, Wearable, Implantable Kidneys (Based on Regulatory Pathways)
3. Clinical Trial Design
4. Stakeholder Understanding of Xenotransplantation

Target End Date: Oct 2020

KHI KIDNEY HEALTH INITIATIVE

ONGOING ACTIVITIES - 2020

Endpoints	Patient Preference Initiative
AKI Biomarkers Roadmap	RRT Roadmap
Clinical Trial Design	



2020: End Stage Renal Disease

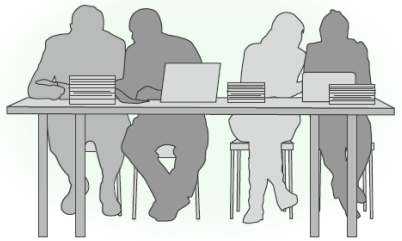
KHI KIDNEY HEALTH INITIATIVE

Endpoints	Patient Preference Initiative
AKI Biomarkers Roadmap	RRT Roadmap
Clinical Trial Design	

Understanding and Overcoming the Challenges to Involving Patients with Kidney Disease in Cardiovascular Trial

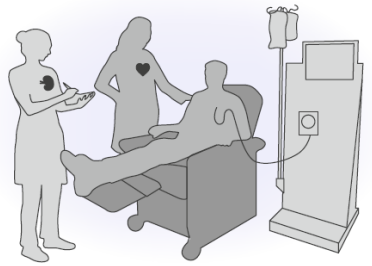
Building the Business Case

- Incentives



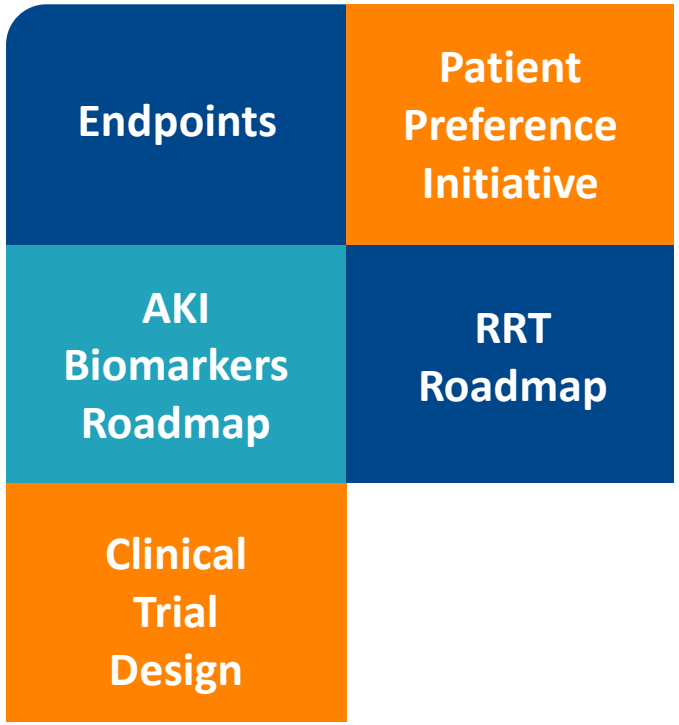
Study Design and Implementation

- Safety
- Protocol design



Changing Research Culture

- Collaboration
- Engagement



Kidney Pediatric Accelerator Trial Clearing House (Kidney-PATCH) Pilot Program

Goals

- Enable feasibility assessment in terms of the available patient populations through data sharing and access to CKD pediatric registries
- Facilitate assessment of the capacity of various pediatric kidney clinical trial organizations
- Assist with identification of expertise that can provide consultation on study planning

ONGOING ACTIVITIES - 2020

Endpoints	Patient Preference Initiative
AKI Biomarkers Roadmap	RRT Roadmap
Clinical Trial Design	Patient Reported Outcomes

Feature

Symptom Prioritization among Adults Receiving In-Center Hemodialysis
A Mixed Methods Study

Jennifer E. Flythe, Tandrea Hilliard, Graciela Castillo, Kourtney Ikeler, Jazmine Orazi, Emaad Abdel-Rahman, Amy Barton Pai, Mathew B. Rivara, Wendy L. St. Peter, Steven D. Weisbord, Caroline Wilkie, and Rajnish Mehrotra

Abstract

Background and objectives Individuals receiving in-center hemodialysis experience a high symptom burden that detrimentally affects their quality of life. There are few evidence-based interventions for symptom relief in this population. To stimulate innovation in symptom management, data on patient symptom prioritization and treatment preferences are needed. We undertook this study to (1) identify patient-prioritized symptoms for the development of symptom relief therapies and (2) elicit preferences for treatments among individuals receiving hemodialysis.

Design, setting, participants, & measurements We conducted a mixed methods study that included focus groups in Carrboro, North Carolina; Tucson, Arizona; and Seattle, Washington and a nationally distributed online survey. Focus group transcripts were analyzed for patterns, and the highest priority symptoms were determined on the basis of frequency and report severity. We used focus group findings to inform survey items. Focus group and survey results were cross-validated and synthesized for final symptom prioritization.

Results There were 32 participants across three focus groups and 87 survey respondents from 27 states in the United States. The physical symptoms of insomnia, fatigue, muscle cramping, and nausea/vomiting and the mood symptoms of anxiety and depressed mood were reported by participants in all focus groups. Among survey respondents, fatigue (94%), cramping (79%), and body aches (76%) were the most common physical symptoms, and feeling depressed (66%), worried (64%), and frustrated (63%) were the most common mood symptoms. The top-prioritized symptoms were consistent across focus group and survey participants and included the physical symptoms insomnia, fatigue, and cramping and the mood symptoms anxiety, depression, and frustration. Participants indicated that symptom frequency, duration, unpredictability, and social and financial effects factored most heavily into symptom prioritization.

Conclusions Patients prioritized the physical symptoms of insomnia, fatigue, and cramping and the mood symptoms of anxiety, depression, and frustration as the top symptoms for which to find new therapies.

Clin J Am Soc Nephrol 13: 735–745, 2018. doi: https://doi.org/10.2215/CJN.10850917

Introduction

More than 400,000 people with ESKD receive in-center hemodialysis in the United States, and they experience exceptionally high rates of morbidity and poor quality of life relative to individuals with other chronic diseases (1–3). Existing data suggest that individuals on hemodialysis have, on average, 11 symptoms, and this high symptom burden contributes to poor outcomes (4). Patients on dialysis have identified symptom relief as a top research priority (5), and a recent international consensus-based prioritization initiative named fatigue, among other symptoms, as a high priority outcome for clinical trials (6). However, there have been few efforts to understand how and why patients prioritize symptoms. The first essential steps in fostering innovation in symptom relief are identifying the symptoms that patients feel are the most important to address and understanding which treatment strategies are most desired by patients.

Prior research has identified common symptoms among individuals on dialysis, including fatigue, sadness, difficulty falling or staying asleep, itching, muscle cramps, pain, and sexual dysfunction (4,7–9). There is a significant association between higher symptom burden and quality of life impairment (2,4). Thus, it is reasonable to posit that symptom amelioration may lead to meaningful improvement in patient-reported outcomes and/or quality of life. However, there are few drugs or devices that have been approved by the US Food and Drug Administration (FDA) that yield improvement in any patient-reported outcome among individuals on dialysis. Moreover, there has been little research on symptom prioritization and treatment preferences in this population.

Feature

Fostering Innovation in Symptom Management among Hemodialysis Patients
Paths Forward for Insomnia, Muscle Cramps, and Fatigue

Jennifer E. Flythe,[1,2] Tandrea Hilliard,[3] Elena Lumby,[3] Graciela Castillo,[3] Jazmine Orazi,[3] Emaad M. Abdel-Rahman,[4] Amy Barton Pai,[5] Matthew Bertrand Rivara,[6,7] Wendy L. St. Peter,[8,9] Steven Darrow Weisbord,[10,11,12] Caroline M. Wilkie,[13] and Rajnish Mehrotra,[6,7] for the Kidney Health Initiative Prioritizing Symptoms of ESRD Patients for Developing Therapeutic Interventions Stakeholder Meeting Participants

Abstract

Individuals receiving in-center maintenance hemodialysis bear a high burden of both physical and mood symptoms. More than half of patients on hemodialysis report sleep disturbance, muscle cramps, and fatigue. Patients describe symptoms as having a deleterious effect on their quality of life, suggesting that symptom alleviation may meaningfully improve patient-reported outcomes. Moreover, patients on hemodialysis have identified symptom management as a key area for research and innovation, prioritizing symptom alleviation over other health outcomes such as mortality and biochemical indices. Despite the importance of symptoms to patients, there has been little research explicitly geared toward improving patient symptoms, and therefore minimal innovation in symptom management. In general, the physiologic underpinnings of symptoms are poorly understood, hampering the development of targeted therapies. In fact, there have been few drugs or devices approved by the US Food and Drug Administration for the indication of improving any patient-reported outcomes for patients on hemodialysis. Recognizing this gap in innovation, the Kidney Health Initiative, a public–private partnership between the American Society of Nephrology and US Food and Drug Administration, convened a workgroup to first prioritize symptoms for the development of therapeutic interventions, and then identify near-term actionable research goals for the prioritized physical symptoms of insomnia, muscle cramps, and fatigue. This paper summarizes the pathophysiology of the three prioritized symptoms, identifies key knowledge gaps, acknowledges factors that challenge development of new therapies, and offers the nephrology community actionable research goals for insomnia, muscle cramps, and fatigue.

Clin J Am Soc Nephrol 14: 150–160, 2019. doi: https://doi.org/10.2215/CJN.07670618

Introduction

Individuals receiving in-center maintenance hemodialysis experience exceptionally high rates of morbidity and poor quality of life compared with individuals with other chronic diseases (1,2). A high burden of both physical and mood symptoms is strongly associated with these poor outcomes. More than half of patients on hemodialysis report sleep disturbance, cramping, and fatigue (3). Patients describe symptoms as substantially affecting their quality of life because of interference with social relationships, financial stability, and overall well-being (4,5). These data suggest that symptom alleviation may meaningfully improve patient-reported outcomes.

Moreover, patients on hemodialysis have identified symptom management as a key area for research and innovation, prioritizing symptoms over other health outcomes such as mortality and biochemical indices (6,7). Despite the importance of symptoms to patients, there has been little research geared toward improving patient symptoms, and therefore minimal innovation in symptom management over the years. Additionally, payment models focus largely on biochemical rather than patient-centered outcomes, missing an opportunity to incent symptom-focused care delivery. In general, the physiologic underpinnings of symptoms are poorly understood, hampering the development of targeted therapies. In fact, there have been few drugs or devices approved by the US Food and Drug Administration (FDA) for the indication of improving any patient-reported outcomes for patients on hemodialysis. Innovation in symptom management is essential to meet the needs of individuals receiving maintenance hemodialysis.

Kidney Health Initiative Hemodialysis Symptom Project

Overview

In 2016, the Kidney Health Initiative (KHI), a public–private partnership between the American Society of Nephrology and FDA, assembled an interdisciplinary workgroup to (1) conduct a study to prioritize symptoms to target for therapeutic development among

Feature

CJASN ePress. Published on April 10, 2020 as doi: 10.2215/CJN.00110120

Toward Patient-Centered Innovation
A Conceptual Framework for Patient-Reported Outcome Measures for Transformative Kidney Replacement Devices

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Abstract

Individuals with dialysis-dependent kidney failure experience considerable disease- and treatment-related decline in functional status and overall wellbeing. Despite these experiences, there have been few substantive technological advances in KRT in decades. As such, new federal initiatives seek to accelerate innovation. Historically, integration of patient perspectives into KRT product development has been limited. However, the US Food and Drug Administration recognizes the importance of incorporating patient perspectives into the total product life cycle (*i.e.*, from product conception to postmarket surveillance) and encourages the consideration of patient-reported outcomes in regulatory-focused clinical trials when appropriate. Recognizing the significance of identifying patient-reported outcome measures (PROMs) that capture contemporary patient priorities, the Kidney Health Initiative, a public–private partnership between the American Society of Nephrology and US Food and Drug Administration, convened a workgroup to (*1*) develop a conceptual framework for a health-related quality of life PROM; (*2*) identify and map existing PROMs to the conceptual framework, prioritizing them on the basis of their supporting evidence for use in the regulatory environment; and (*3*) describe next steps for identifying PROMs for use in regulatory clinical trials of transformative KRT devices. This paper summarizes the proposed health-related quality-of-life PROM conceptual framework, maps and prioritizes PROMs, and identifies gaps and future needs to advance the development of rigorous, meaningful PROMS for use in clinical trials of transformative KRT devices.

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Introduction

More than 700,000 Americans receive KRT—dialysis or kidney transplantation—costing the Medicare system $35 billion in 2016 (1). Despite this investment, individuals receiving dialysis experience considerable disease- and treatment-related declines in functional status and overall wellbeing. Dialysis is highly burdensome, with many individuals treated with in-center hemodialysis, a therapy that is disruptive to daily life and often compounded by debilitating side effects such as cramping, fatigue, poor sleep, and depression (2–4).

To address this state of affairs, initiatives such as KidneyX, an innovation accelerator supported by a public–private partnership between the US Department of Health and Human Services and the American Society of Nephrology (ASN), and the Executive Order, Advancing American Kidney Health, seek to disrupt existing approaches to kidney care and incentivize innovation in KRT (5,6). Next-generation KRT devices are likely to encompass a spectrum of technologies, from portable to wearable to implantable bioengineered products, many with the potential to revolutionize the patient experience. Historically, integration of patient perspectives into KRT product development has been limited. However, the US Food and Drug Administration (FDA) recognizes patient perspectives as essential to safe, effective medical product development and evaluation, and the Center for Devices and Radiologic Health named partnering with patients as a strategic priority in 2016 (7).

Using patient-reported outcomes (PROs) in defining clinical trial end points is one opportunity to encourage more patient-centered innovation and evaluation of medical products (7). Existing patient-reported outcome measures (PROMs) may not adequately reflect patient priorities (8). The Standard Outcomes in Nephrology (SONG) Initiative found that patients prioritize relief of fatigue, ability to work and travel, and more dialysis-free time as outcomes for clinical trials, concepts not specifically captured in existing PROMs (2). PROMs that better capture patient priorities would focus innovators and regulators on the treatment effects most meaningful to patients.

Kidney Health Initiative Transformative KRT Devices PROM Project

Overview

PROMs used as outcome assessments in regulatory trials must meet rigorous criteria and be sensitive in detecting treatment (*e.g.*, KRT device) effects and discriminating between scores in a clinical trial's treatment and nontreatment arms (9,10). As such, all PROMs used in clinical practice may not be appropriate for

- Patient Reported Outcomes for Dialysis Vascular Access
- Patient Reported Outcomes for Muscle Cramping in Patients on Dialysis

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KHI KIDNEY HEALTH INITIATIVE

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Q & A