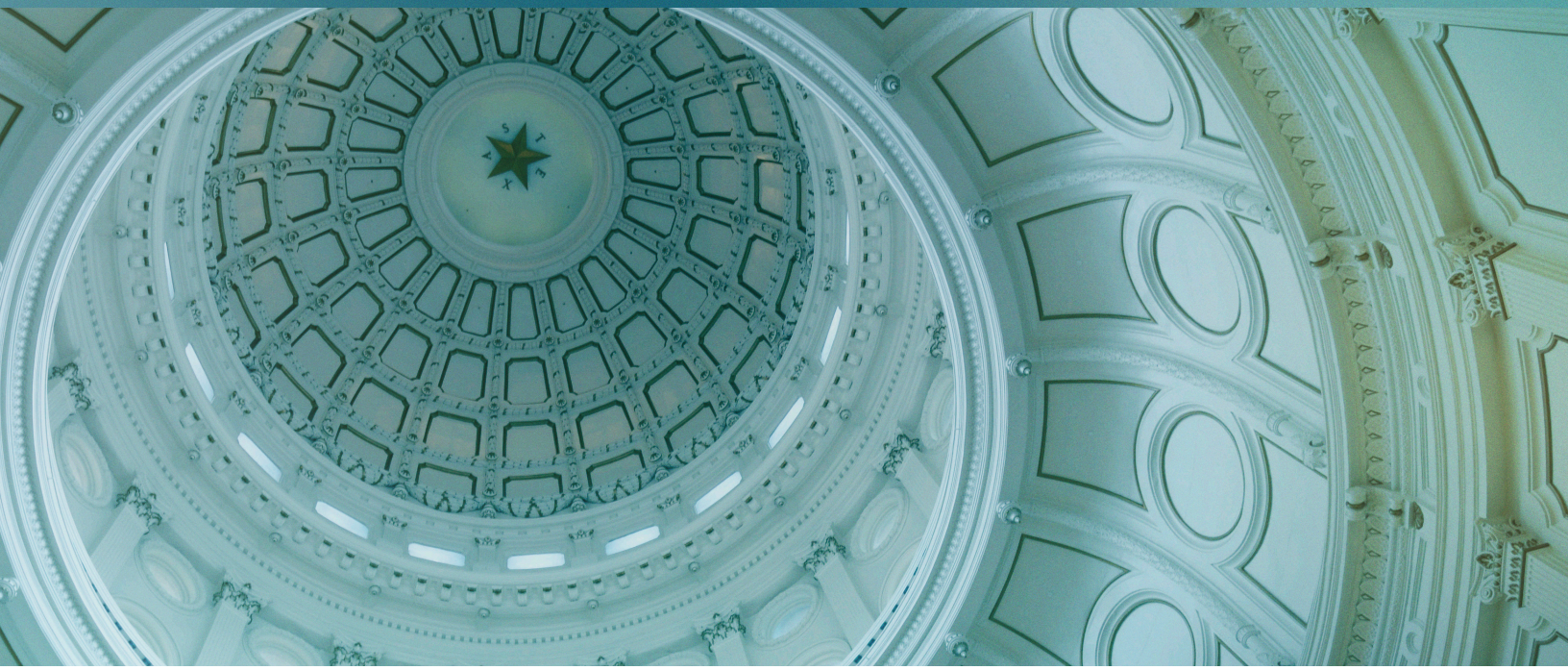
Rare Opportunities in State Policy to Prevent End-Stage Kidney Disease



INTRODUCTION

Rare kidney diseases represent a critical and often overlooked area of kidney health, often disproportionately impacting young and vulnerable populations. However, now is the time for bold action on rare kidney disease (RKD) education, diagnosis, treatment, and care – we are in a new era, the Rare Kidney Disease Revolution.

For too long, patients and families with RKD have endured long diagnostic journeys, limited and unfavorable treatment options, and systemic barriers to accessing specialized care. While nephrology has historically lagged behind other specialties in clinical trial activity and therapeutic development, we are now seeing more promising advances in research, the first-ever FDA approved therapies for RKD conditions like IgA nephropathy, a surge of clinical trials, and an energized patient community.

This momentum brought together over 40+ stakeholders in the fall of 2024 to discuss what opportunities exist in state policy to empower patients and providers to increase timely diagnosis and treatment of RKD and ultimately prevent end-stage kidney disease (ESKD), also known as kidney failure. While federal initiatives, including a 2020 policy roundtable and the subsequent introduction of related federal legislation entitled the New Era for Preventing End-Stage Kidney Disease Act have brought long-overdue attention to RKD, state-level policy action is essential to complement these efforts and build sustainable community-based solutions to address critical patient and provider challenges at the local level.

This white paper explores opportunities for states to lead in preventing ESKD among RKD patients by expanding screening programs, enhancing access to specialist care including through telehealth, launching targeted awareness campaigns, and ensuring RKD voices are represented and can impact state policymaking. With collaboration across stakeholders—including patients, healthcare providers, advocacy groups, industry, and government leaders—state-driven initiatives can help build national momentum and deliver transformative change for those living with rare kidney diseases.

> **The time is now for state action on RKD. Patients and families with rare kidney diseases do not have time to wait.**

EXECUTIVE SUMMARY

There are more than 150 different types of RKD including focal segmental glomerulosclerosis (FSGS), Alport syndrome, IgA nephropathy (IgAN), C3 glomerulopathy (C3G), and minimal change disease to name a few. RKD is a leading driver of chronic kidney disease, and in fact glomerulonephritis is the third leading cause of kidney failure in America. Data indicates that RKD patients represent only 5-10% of people with kidney disease, but account for more than 25% of patients receiving kidney replacement therapy.[1] With the recent development of new diagnostic and therapeutic tools for RKD, there is a real opportunity to improve the lives of those living with a disease that too often results in dialysis, transplant, or death. States have a landmark and unique opportunity to reduce dialysis and transplant costs if they address rare kidney diseases.

Patients and families impacted by RKD face long diagnostic journeys, barriers to accessing needed medical care, and other challenges that require collective state attention. Federal legislation, like the New Era for Preventing End-Stage Kidney Disease Act (H.R. 1518) (**"New Era Act"**), is bringing attention to the need to focus on early detection of RKD, bolster patient and provider education, and improve care coordination for RKD patients. Now, more than ever, state-level RKD initiatives must be leveraged to address local disparities in healthcare access and ensure resources are allocated where they are most needed.

In January 2024, NephCure launched the **New Era Coalition** to transform the landscape of research, treatment, and care for RKD and to advocate for federal and state policies that work to improve outcomes for RKD patients. To build on the promise of federal-level legislation action, like the **New Era Act**, at the state level, NephCure, with support from New Era Colaition advisory member Travere Therapeutics and other coalition stakeholders, facilitated a virtual event in October 2024 titled **"Rare Opportunities in State Policy to Prevent End-Stage Kidney Disease."** Over 40 attendees – representing patients, patient advocacy groups, caregivers, healthcare providers, researchers, government officials, and industry leaders – convened to discuss state-level policy challenges and opportunities related to RKD, as well as potential vehicles to advance those policies.

Participants shared their diverse perspectives in the following working group discussions:

The Patient Voice in RKD: Policy Engagement Opportunities at the State Leve

Opportunities to Advance Patient-and Provider-Focused Policies for RKD

Healthcare Coverage and Access for the Future of RKD Diagnosis and Treatment

Across each working group, participants highlighted opportunities to advance policies and activities to improve the patient journey for those impacted by RKD. The program was launched by two state leaders: Kelly Hancock, serving at the time as a Texas State Senator, and Brittany Hall, serving at the time as Delaware's Lieutenant Governor. As a person living with IgAN, Senator Kelly has worked in his state legislature to advance awareness and treatment for people living with RKD. As a clinician, Lt. Governor Hall knows first-hand the need for patient and provider education and the challenges facing those living with rare diseases, including rare kidney diseases. Both elected officials set the stage for all stakeholders on the importance of their input and deliberations.

Overall, four policy recommendations were identified:

01

Create an RKD pilot screening program in Medicaid or state health systems for at-risk populations to support earlier diagnosis to prevent end-stage kidney disease in at least two states.

Earlier screening was identified as a critical need across workshops. Today, there are FDA-approved treatments for some RKDs, and more are on the horizon. By catching RKD upstream, there is a real opportunity to save native kidneys and prevent kidney failure.

02

Support legislation that allows for telehealth flexibility, particularly for those living with rare diseases, so patients can access RKD specialists.

There is a shortage of nephrologists who routinely care for patients with RKD, or who specialize in RKD, especially in rural settings. Enabling patient access to RKD providers through telehealth can help solve this problem.

03

Establish a state awareness campaign to educate on RKD diagnosis, treatment, and care for patients served by, or care providers compensated by, state-regulated insurance.

There is a need to increase awareness of RKD in the context of state-regulated insurance. This can be accomplished by working with health departments, taskforces, and other relevant agencies to establish an awareness campaign to educate the general public, providers, and reach at-risk populations.

04

Ensure the RKD voice is present in state policy by increasing the role of our community in existing rare disease advisory councils (RDACs) and kidney taskforces, and work to increase the number of states which have one or both.

Consider building state RKD coalitions to ensure all existing and future RDACs and kidney taskforces advance the needs of the RKD community. Work to nominate members of the RKD community to serve on councils, participate in and amplify surveys with the RKD community, and participate in live/written public comment opportunities to share the impact of living with RKD.

Recommendation #1:

RKD Pilot Screening Program for Early Diagnosis in Medicaid Programs or Other State Health Systems

RECOMMENDATION:

Create an RKD pilot screening program in at least two states in their Medicaid program or other state health systems for at-risk populations to support earlier diagnosis to prevent end-stage kidney disease.

SUMMARY:

Earlier screening was identified as a critical need across all three workgroups. Today, there are FDA-approved treatments for some RKDs, and more on the horizon. By diagnosing RKD upstream, there is a real opportunity to save native kidneys and prevent kidney failure.

Early and accurate diagnosis is critical for those living with RKD, as many are not aware of their diagnosis until the disease has progressed to ESKD and they "crash" into dialysis. This phenomenon "crashing" into dialysis means starting dialysis unexpectedly or in an emergency situation. Each breakout session highlighted that delays in diagnosis, as well as misdiagnosis, remain a critical challenge for RKD patients. Diagnostic delays allow the disease to progress unnoticed, accelerating kidney failure, which results in the need for dialysis or a kidney transplant to sustain life.

In one RKD called focal segmental glomerulosclerosis (FSGS), it occurs in African American populations at a 4-5 times higher rate than white Americans.[2] Additionally, African Americans experience kidney failure at four times the rate of white individuals, and Asian Americans at 1.4 times the rate.[2] FSGS upstream diagnosis and care is critical to prevent the up to 40-50% of patients with FSGS progressing to kidney failure over 5-10 years, a rate much faster than other RKDs.[3] Barriers such as lack of patient and provider education, limited health literacy, language barriers, and lack of access to specialty care make early diagnosis challenging.

Additionally, misdiagnosis is also common, with patients frequently spending years consulting multiple healthcare providers before finally receiving an accurate diagnosis.[4] States are uniquely positioned to help promote early and accurate diagnosis which would improve patient outcomes and reduce public and private healthcare costs associated with treatment for kidney failure.[5]

To help facilitate access to earlier diagnosis, working group members identified the benefits of creating an RKD pilot screening program. Screening solutions such as quick, cheap, and effective urinalysis can be an incredibly effective tool to be deployed in community-based RKD screenings. Mobile health units could help improve access to education and screening. A targeted screening program could also include the use of newer diagnostic tools, including genetic testing, to identify patients with positive screening results. These efforts would promote equitable access to early screening and diagnosis for disproportionately affected communities.

Implementing a screening pilot program in at least two states would test whether RKD screening models through state and community engagement could improve health outcomes and provide savings in the form of reduced long-term healthcare costs for patients affected by RKD. This pilot initiative could also provide valuable data for state-run health programs about how to effectively increase screening and diagnosis at the state level, hopefully leading to wider adoption across states.

ACTION STEPS:
STATE-LEVEL ACTIVATIONS

1. Convene stakeholders to assess and identify two pilot states based on RKD prevalence, need, existing infrastructure, and population risk.
2. Develop guidelines for RKD screening and diagnosis in consultation with nephrologists, patient advocacy organizations, patients and families, industry, and others to deploy as part of the pilot program.
3. In the two identified states, pursue state legislation or administrative action to fund and authorize a Medicaid-based RKD screening pilot program. Engage state health departments, legislators, Medicaid administrators, and relevant advisory bodies to fund and authorize a pilot program in other state health systems.
4. Develop and distribute educational materials to family practices, pediatricians, and internal medicine practices about the importance of early screening and diagnosis, and to raise awareness about the pilot program.

Recommendation #2:
Advocate for Telehealth Flexibility for RKD

RECOMMENDATION:

Support state legislation or regulation that allows for telehealth flexibility, particularly for those living with rare diseases, so patients can access RKD specialists.

SUMMARY:

There is a shortage of nephrologists who routinely care for patients with RKD, including those who specialize in RKD, especially in rural settings. In some states, there are no pediatric nephrologists available at all, creating major gaps in care. Increasing the number of nephrologists as well as enabling patient access to RKD providers through telehealth can help solve this problem.

Nephrologists play a critical role in the early diagnosis and treatment of RKD, which is essential for avoiding ESKD and accessing new therapies. However, the United States has been facing a shortage of nephrologists, which is even more limited for populations disproportionately affected by RKD. For example, as one working group participant shared, at least five states do not have any pediatric nephrologists available to patients. Other barriers to nephrologists often include insurance coverage issues, long travel times, and out-of-pocket costs.

As a result of these access challenges, patients may need to wait extended periods or travel considerable distances to see a nephrologist. This can directly impact the quality of care patients with RKD of all ages receive. For individuals from underserved communities, these barriers can be even more pronounced.

To help combat known obstacles to accessing RKD providers, working group members highlighted an avenue to help address this potential access barrier – advancing legislation that allows for patients to see providers outside of their own state via telehealth. By leveraging telehealth technology like video conferencing for patient-provider visits, individuals living with RKD can face less challenges trying to access care. Rare kidney diseases are largely progressive diseases, and a patient's relationship with their specialist is one of the most important aspects of their care. This approach ensures that those who face significant challenges in accessing in-person medical services—such as long travel distances, limited provider availability, or mobility restrictions—can still address their evolving medical needs effectively and in a timely manner. Telehealth can be considered a tool that not only expands access to essential nephrology care but also helps bridge disparities for underserved populations. States have the authority to authorize nephrologists to practice across state lines, including by participating in the Interstate Licensure Medical Compact.

ACTION STEPS:
STATE-LEVEL ACTIVATIONS

1. Identify existing legislative or regulatory barriers states must address to expand access to telehealth services furnished by nephrologists. Many states started to expand these flexibilities as the result of the COVID-19 pandemic, which can be leveraged and built upon.
2. Engage with relevant state agencies and legislators to explore opportunities in their state and assess the need for legislation or regulation that would expand access to nephrologists via telehealth.
3. Partner with existing telehealth coalitions and advocates to launch legislative or regulatory efforts on a state by state basis, and measure if these initiatives reduce the cost of care patients with RKD, including by delaying or preventing end-stage kidney disease.

Recommendation #3:

Launch a State Awareness Campaign on RKD Diagnosis, Treatment, and Care

RECOMMENDATION:

Establish a state awareness campaign to educate patients and providers on RKD diagnosis, treatment, and care.

SUMMARY:

Despite the increase in innovative therapies to treat some RKD there is still a great lack of patient and provider awareness, especially in the context of state-regulated programs including Medicaid and the state-regulated insurance market. States are uniquely positioned to lead targeted education that can help close RKD gaps within the general public, providers, and at-risk populations by working with health departments, taskforces, and other relevant agencies.

More awareness about RKD is critical in order to reach populations at risk and ensure patients and their families are connected with the right resources to enable them to take the steps necessary to delay or prevent ESKD. A state-level public awareness campaign, with a focus on community impact, would serve a dual purpose: equipping patients and families with knowledge to recognize early signs and symptoms of RKD while empowering healthcare providers with the tools needed for timely diagnosis and effective management. By targeting patients served by, and care providers participating in, state-regulated insurance Medicaid programs, the campaign could foster early intervention, improve care quality, and reduce long-term health care costs.

Many stakeholders noted the presence of dialysis clinics in their communities, and cited the potential for community-based RKD programs to provide a better, more hopeful path for affected families. For patients, especially those in underserved and minority communities, such a campaign could bridge gaps in awareness and access to care. Early education about RKD symptoms in both pediatric and adult populations can lead to timely interventions, potentially preventing progression to ESKD. Culturally relevant outreach, coupled with partnerships with community organizations and local healthcare providers, can help overcome barriers like limited access to specialists and low health literacy. By utilizing digital media and in-person strategies, the campaign could effectively reach vulnerable populations and promote better health outcomes.

> **For example, in one of NephCure's community programs 175 individuals have been screened and connected with relevant resources.**

For healthcare providers, the campaign would address critical knowledge gaps, particularly among general practitioners, pediatricians, and family physicians who often provide care to RKD patients outside of specialized care settings. Tailored educational resources and training on RKD diagnosis, treatment, and care coordination would promote evidence-based practices and enable more effective management of disease. Ultimately, a targeted awareness campaign would lay the foundation for early intervention, reduced health disparities, and improved outcomes for RKD patients.

ACTION STEPS:
STATE-LEVEL ACTIVATIONS

1. Partner with state health departments and Medicaid agencies to develop and fund RKD awareness campaigns. Advocate for state funding to support these outreach efforts.
2. Design culturally appropriate outreach materials depending on the state and even region within the state in collaboration with community-based organizations, patient advocates, and healthcare providers.
3. Explore opportunities to partner with state physician organizations that can offer RKD educational opportunities.
4. Explore opportunities to partner with state-sponsored medical universities to include RKD education in curriculum.

Recommendation #4:

Ensure RKD Representation in State Policymaking Through Rare Disease Advisory Councils and Kidney Taskforces

RECOMMENDATION:

Ensure the RKD voice is included in state policymaking by increasing the role of RKD stakeholders in existing rare disease advisory councils (RDACs) and kidney taskforces, and work to increase the number of states which have both types of bodies.

SUMMARY:

Where feasible, build state RKD coalitions to ensure all existing and future RDACs and kidney taskforces elevate the needs of the RKD community. Work to nominate members of the RKD community to serve on councils, participate in and amplify surveys with the RKD community to generate critical data, and

RDACs serve as important advisory bodies at the state level, providing a forum for the rare disease community to actively engage and advise state governments and policymakers on rare disease.[6] Since the establishment of the first RDAC in 2015, 33 states have enacted legislation establishing an RDAC, signaling growing recognition of their importance.[7] In addition to RDACs, approximately six states have implemented kidney taskforces dedicated to studying kidney disease, identifying prevention opportunities, and making policy recommendations to state lawmakers to spur positive change for people living with kidney disease.[8]

Working group participants emphasized the vital role RDACs and kidney taskforces play in amplifying the patient voice within state governments. To be most effective, these entities must encompass diverse perspectives, including those of patients, advocates, caregivers, and medical professionals. This diversity ensures that unique needs and experiences of individuals living with all rare diseases, including RKD are adequately represented.

For states with active RDACs and/or kidney taskforces, there is an opportunity for members of the RKD community to engage in public comment periods, participate in surveys, and consider applying to serve on an RDAC or taskforce as vacancies arise. Additionally, working group participants stressed the importance of fostering communication and collaboration in states that have multiple taskforces and groups dedicated to RKD to align goals and efforts.

Last, there remains a pressing need for the creation of RDACs and/or kidney taskforces in states where they do not exist. Establishing these entities often requires legislative action and the support and prioritization from state leadership, including legislators and governors. Expanding these efforts nationwide would ensure a stronger, unified voice for individuals living with RKD in state governments.

ACTION STEPS:
STATE-LEVEL ACTIVATIONS

1. Build State RKD coalitions to advance RDACs and kidney task forces in additional states.
2. Nominate members of the RKD community to serve on existing councils and task forces.
3. Participate in public comment opportunities, surveys, and/or hearings.

CONCLUSION:

States that invest early in RKD detection and awareness can save lives and reduce healthcare costs. The four recommendations in this paper are meant to serve as an action guide for patients, caregivers, families, and state policymakers to create meaningful change. The time is now.

WORKING GROUP PARTICIPANTS:

Patients & Caregivers
American Kidney Fund
American Nephrology Nurses Association
Asian and Pacific Islander American Health Forum
Colorado State House Representative
EveryLife Foundation for Rare Diseases
Gene Dx
IgAN Foundation
Illinois State Senate
ISGD International Society of Glomerular Disease
Mannatt
Natera
National Kidney Foundation
National Minority Quality Forum
National Organization for Rare Disorders
NephCure
Office of the Texas Governor
Otsuka
Rare Access Action Project
Rare Disease Diversity Coalition - BWHI
Rare Disease Innovations Institute
Renal Pathologists Association
South Texas Renal Care
Stanford University Medicine
Travere Therapeutics
University of Michigan Medicine School
VCU Health

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1. https://www.sciencedirect.com/science/article/pii/S014067362302843X#
2. https://www.kidney.org/about/kidney-disease-fact-sheet
3. https://pmc.ncbi.nlm.nih.gov/articles/PMC12014854/
4. https://pmc.ncbi.nlm.nih.gov/articles/PMC6685068/
5. https://www.kidneyfund.org/government-affairs-advocacy/our-policy-agenda/improving-early-detection-kidney-disease-and-addressing- unknown-causes-kidney-disease
6. https://rarediseases.org/policy-issues/rare-disease-advisory-councils/
7. https://rarediseases.org/rare-disease-advisory-councils/
8. https://www.kidneyfund.org/article/whats-happening-states-july-2021

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