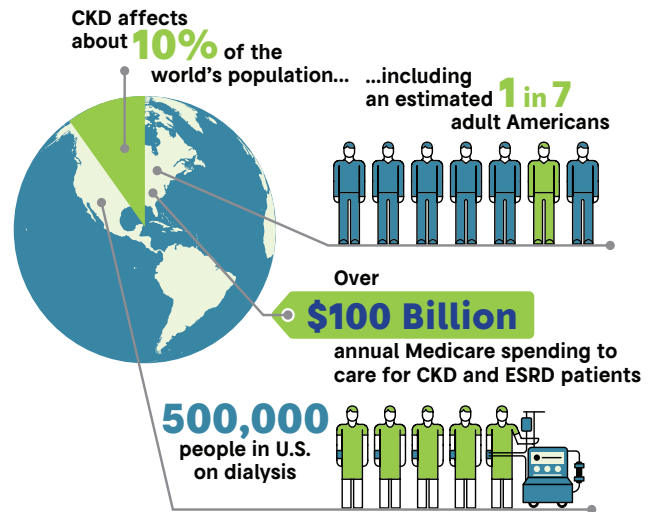


# Technology Roadmap for Innovative Approaches to Renal Replacement Therapy

## PATIENT EDITION

### Time for Change

Current treatment options for chronic kidney disease (CKD) are time-consuming, have a negative impact on patient well-being and quality of life, and can be extremely costly. For patients whose kidney function has worsened to the point of end stage renal disease (ESRD), dialysis and transplant remain the only treatment options. Methods for treating the disease—collectively referred to as renal replacement therapy (RRT)—have not changed significantly since dialysis was introduced about 60 years ago.



### Now is the time for change—because patients deserve better options.

To address the urgent need for RRT innovation and help accelerate the availability and adoption of solutions, the Kidney Health Initiative published the *Technology Roadmap for Innovative Approaches to Renal Replacement Therapy* in October 2018. The roadmap outlines:



**A vision of improved patient quality of life** that innovative RRT solutions should achieve



**Technical and market challenges** that must be overcome and the overarching **action plan** for doing so



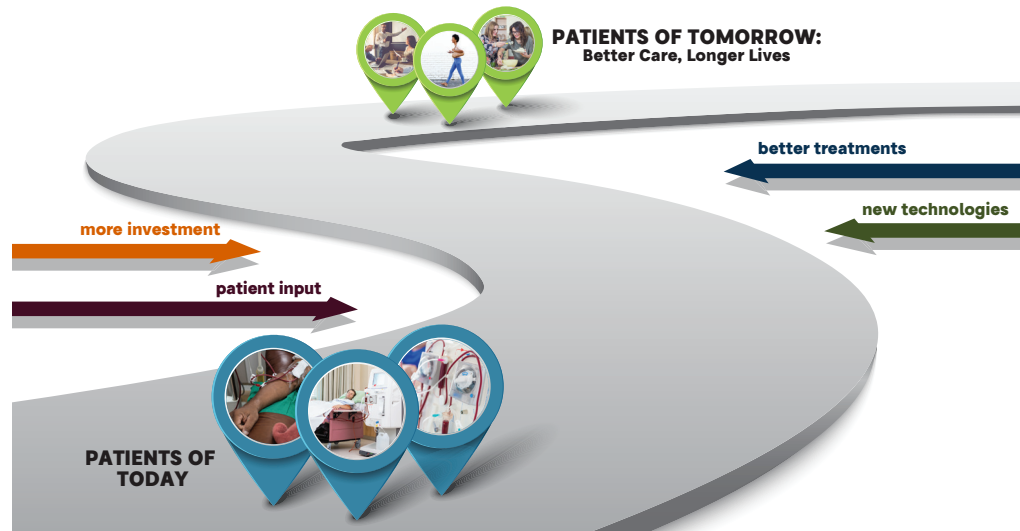
**High-priority research activities** that can bring about the biggest changes

To improve the lives of millions of patients, **the community needs active participation from a diverse group to help identify the best solutions.** These contributors must include the medical community but also technology developers, engineers, entrepreneurs, and, perhaps most importantly, patients with kidney disease.

**KHI and its partners need patient involvement to help turn innovative ideas into reality.**

# A Better Quality of Life for Patients

Living with kidney disease takes a heavy toll on patients and caregivers. Most patients have few choices beyond dialysis, the side effects of which often leave them feeling sick and longing for a better existence. Additionally, patients receiving treatment often feel that they are unable to live a “normal” life, and ordinary activities—exercise, working, traveling, dining out, spending time with friends and family—may become challenging, if not impossible.



The goal of the roadmap is to change the lives of patients with kidney disease and give them a better tomorrow. And this is what better will look like:



**Minimized impact on family and social life**



**Fewer pills to take**



**Ability to be more physically active**



**Fewer side effects or complications like fatigue, nausea, dizziness, infection, cramping, and depression or other mental health issues**



**More freedom to work and travel**



**More treatment choices and options based on lifestyle or life stage**



**Less time spent in the hospital or undergoing procedures**



**A less restrictive diet, with greater freedom to eat and drink what you want**



**Reduced financial impact**

# Key Challenges to Overcome

Treatment methods for kidney disease have not changed significantly in more than 60 years. This is largely due to layers of complexity related to basic science, technology development, clinical trials, product commercialization, and the need for individualized care so that all patients can receive the treatment that is best for them.

**The roadmap identifies the key challenges that must be overcome**—the challenges keeping you and patients like you from enjoying an improved quality of life.

## Technical Challenges



**All the biological functions of the kidney are difficult to mimic:** The complexity of kidney function and its internal connectivity makes it scientifically difficult to create and test an alternative or to model human kidney function to prove solution reliability and safety.



**Isolated solution development can limit fresh ideas:** Developing effective and innovative solutions will require people from diverse background to work together, including scientists, clinicians, technology developers, and patients.



**Patients are unique and require customizable or adjustable treatments:** Because patients are all different—the cause of their loss of kidney function, their treatment response, their ethnicities and socioeconomic situations, their cultural backgrounds, their life stages—new solutions must be flexible.

## Market Challenges



**Innovators don't see enough economic incentive:** Lengthy approval processes and time to market for new products, in addition to the high costs and risks of development, tend to discourage potential funders and investors. Additionally, the current insurance coverage and reimbursement model is focused on dialysis, not disruptive new technologies.



**Developers lack experience dealing with government regulations, and the industry doesn't offer enough relevant standards to assist them:** Industry stakeholders lack experience with regulations and regulatory agencies (e.g., the Food and Drug Administration [FDA]). Next-generation RRT solutions are also likely to be combination products—products that integrate biologics, devices, and/or drugs—which can introduce even more regulatory complexity.



**Patients with kidney disease are underrepresented in clinical trials:** The nephrology field has too few randomized controlled trials—studies that yield the unbiased results needed to bring new products to market because of their random selection of participants that receive an intervention/treatment. Additionally, patients with kidney disease are often excluded from trials for other health conditions (e.g., cardiovascular disease and diabetes), due to associated higher health risks and trial length. As a result, they may be treated for these conditions with interventions that have not been adequately tested in patients with kidney disease.



**Patients have different levels of risk tolerance and confidence in new solutions:** Some patients may want and would adopt partial or incremental yet near-term solutions (e.g., hemodialysis 1x/week versus 3x/week) while others would prefer the industry focus on more disruptive but potentially longer-term solutions.

# Roadmap to a Better Tomorrow

While these challenges have prevented innovation for decades, collaborative and innovative efforts can change the status quo and lead to new solutions with real impact for patients. However, advancing these necessary alternatives will take time—time that many patients may not have.

Therefore, the roadmap outlines a plan of action to more rapidly deliver meaningful advancements and improved patient quality of life.

## Solution Strategy

To achieve a better quality of life for patients while overcoming existing challenges, the roadmap outlines a strategy that will allow the technology and nephrology communities to work within different solution areas at the same time. This plan of action involves working on not only long-term innovations that will eliminate disease impact on patient lives but also shorter-term, more immediate advances that will offer greater lifestyle flexibility and fewer treatment complications.



**Enhanced Dialysis:**  
incremental improvements to existing dialysis

- Increased treatment flexibility
- Reduced disease complications



**Portable/Wearable:**  
external devices that provide alternatives to stationary treatments

- Increased patient independence and freedom of movement (work, travel)
- Continuous or near-continuous treatment
- Reduced treatment impact (e.g., pill burden, dietary restrictions)



**Implantable / Biohybrid:**  
bioengineered products that more closely mimic kidney function

- Continuous treatment
- Reduced treatment impact for patients (e.g., thirst, diet, work, mobility)
- Unlimited, readily available supply of organs when needed



**Regenerated Kidney:**  
recovered and maintained kidney function

- Elimination of disease impact on patients (e.g., unrestricted diet, return to work)

## Patient-Focused Design Guidelines

Because the integrated function of the kidney is extremely complex, repairing or replacing each individual function will require different solutions. To ensure solutions tackle each kidney function and to help potential innovators who may not be as familiar with these kidney functions, the roadmap also establishes a set of design guidelines that any solution being developed needs to aim to achieve. **All these common design guidelines are geared toward achieving maximum patient benefit** and have been grouped by kidney function or key function-enabling components to better identify the areas in which focused research and funding can have the most impact.



Function/Component



Patient Impact

### RRT Access

The vascular, peritoneal, blood circuit, or alternative (e.g., GI tract) access needed for treatment

Safer, more continuous treatment with fewer complications and reduced need for interventions

### Blood Filtration

Filtering of blood to remove waste and excess fluid

Access to a safe, effective, longer-life, easy-to-use filtration system that avoids clotting issues

### Electrolyte Homeostasis

Maintaining appropriate levels of key components in the blood

More comprehensive and effective regulation of electrolytes, resulting in reduced need for dietary restrictions or supplements

### Fluid Regulation

Regulating the amount of body fluid and/or removing excess fluid

Ability to personalize and optimize the amount and rate of excess fluid removal, resulting in stable blood pressure and less thirst and cramping

### Toxin Removal/Secretion

Limiting or preventing toxin accumulation in the bloodstream and throughout the entire body

Reduced presence of harmful toxins in the bloodstream that could lead to uremic toxicity and/or drug toxicity

### Filtrate Transport and Drainage

Removing excess filtrate after processing; connectivity for filtration, processing, and exterior drainage

Drainage processes and equipment that are safe and effective, with acceptable characteristics (e.g., size, weight, comfort, appearance, compatibility with sanitary infrastructure)

A complete list of all the technical guidelines can be found in the full version of the RRT roadmap: <https://khi.asn-online.org/rrtroadmap>.

## R&D Activities (2019-2026+)

Additionally, the roadmap provides a set of focused research and development (R&D) activities to accelerate the advancement and availability of innovative and comprehensive renal replacement therapy (RRT) solutions. **All the activities are designed to encourage the development of the most effective and patient-friendly solutions**, and were identified as having the greatest potential to improve patient quality of life by achieving meaningful results in the near (2019–2022), mid (2023–2025), and/or long term (2026+).

These activities are grouped by three focus areas:



**Kidney Functions:** Activities to pursue unique solutions for replicating or replacing each kidney function

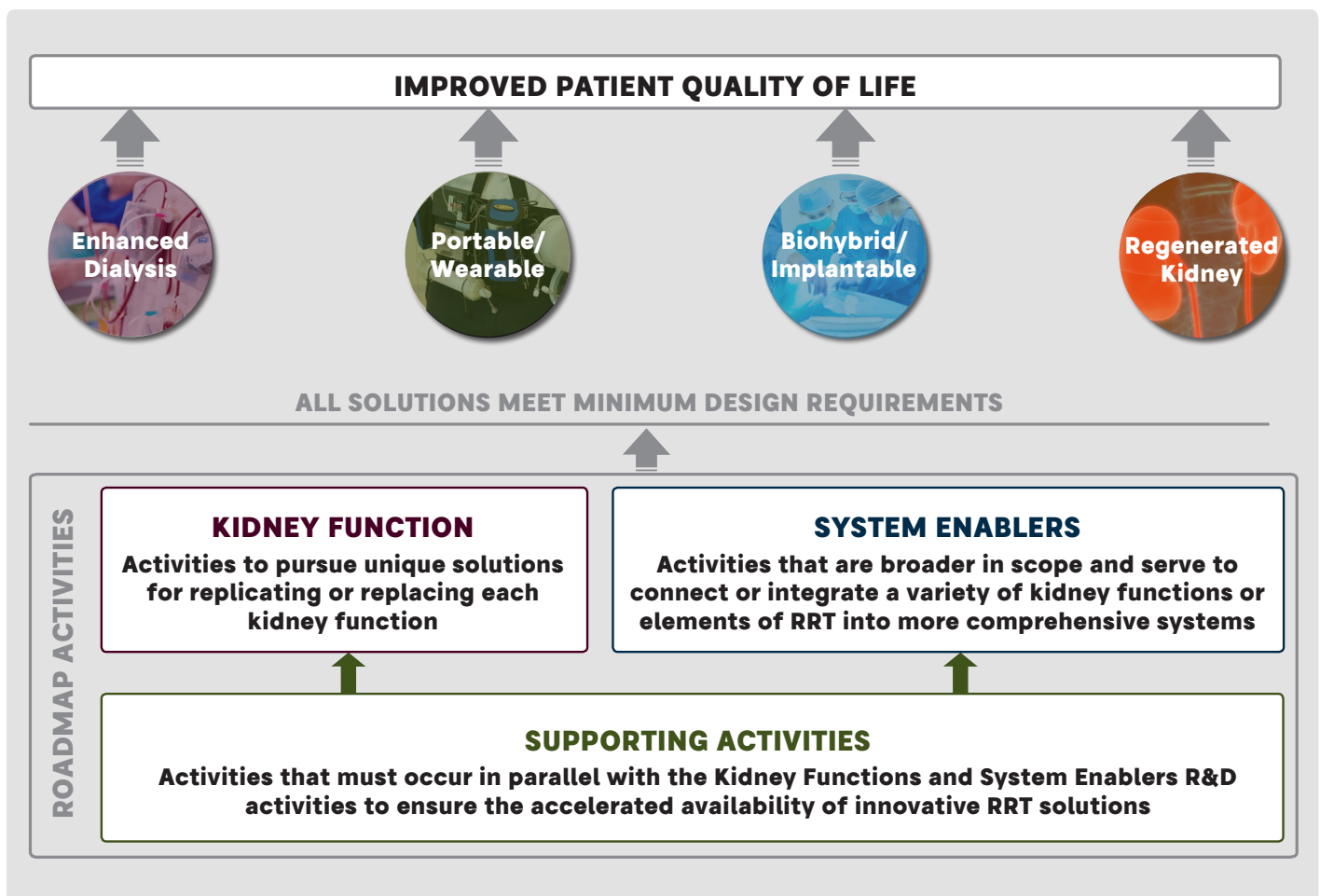


**System Enablers:** Activities that are broader in scope and serve to connect or integrate a variety of kidney functions or elements of RRT into more comprehensive systems



**Supporting Activities:** Activities that must occur in parallel with the *Kidney Functions* and *System Enablers* R&D activities to ensure the accelerated availability of innovative RRT solutions

A complete list of all technical R&D activities and timelines can be found in the full version of the RRT roadmap: <https://khi.asn-online.org/rrtroadmap>



# Patient Contributions to the Roadmap



To maximize the roadmap's impact, KHI incorporated input from a diverse group of contributors, including:

- Patients and care partners
- Researchers and clinicians
- Product developers and entrepreneurs
- Regulatory agencies and payers

**Throughout roadmap development, the Roadmap Patient Advisory Committee represented patient perspectives and ensured that improving patient quality of life remained at the center of the roadmap strategy.**

## Patient Survey

In addition to receiving patient input to the roadmap from the Roadmap Patient Advisory Committee, KHI conducted a detailed survey to explore patient perceptions and preferences about attributes of new therapies. The purpose of this survey was to ensure that both the roadmap and resulting technology advances keep patient needs and improved quality of life as their overarching goal. The survey asked for patient input on the benefits and downsides of new therapies—namely wearable and implantable devices—as well as their interest level in using such therapies.

- Nearly 250 patients completed the online survey
- Responses indicated a preference for implantable over wearable devices
- Keen enthusiasm was expressed for all new therapies or advances that would improve patient quality of life
- The ability to travel, return to work, and carry out family responsibilities were the most preferred attributes of new treatment options

In short, the survey findings support the aspects of improved quality of life articulated in the roadmap and underscore the importance of considering these patient quality of life improvements in the development of treatment innovations.

### **PATIENT INVOLVEMENT IN ACTION: HELPING KHI FOSTER INNOVATION IN FLUID MANAGEMENT**

Following the publication of the RRT roadmap, KHI created a workgroup—composed of patients, nephrologists, researchers, engineers, and technology developers—to develop guidelines for devices that can help patients self-manage fluids in between dialysis treatments or help clinicians in-center manage a patient's fluid during treatment.

These solution requirements will be developed through a patient-centered approach and are intended to inform technology innovators' efforts to develop fluid management solutions that improve patient outcomes related to fluid imbalance (e.g., blood pressure).

Once completed, this guidance will be publicly available on the KHI web site.

# Get Involved

As we move toward the better future envisioned in the roadmap, patient input and engagement will play a critical role in helping change the way kidney disease is treated. There are many ways that patients can get involved.



## Connect with KHI

Through KHI, you can share your story, give feedback to groups creating new products, volunteer, and more.

- ▶ <http://kidneyhealthinitiative.org>
- ▶ Email: [khi@asn-online.org](mailto:khi@asn-online.org)



## Participate in KidneyX Competitions

The Kidney Innovation Accelerator (KidneyX) is a partnership between the American Society of Nephrology (ASN) and the U.S. Department of Health and Human Services (HHS). In addition to its technical prize competition—which offers funding to individuals with exciting ideas to develop new products and treatments for kidney patients—KidneyX is also planning a Patient Innovator Challenge to encourage patients to propose solutions to everyday treatment challenges.

- ▶ <https://www.kidneyx.org/WhatWeDo/patientengagement>



## Be an Advocate

Over 100,000 new Americans begin some kind of dialysis treatment each year. These patients would benefit from awareness of the current initiatives—such as the roadmap and the KidneyX prize competitions—being undertaken to develop new solutions that could improve their lives. Consider sharing this brochure with other patients.

- ▶ Please share this brochure with other patients



## Learn More

Get more involved with your own treatment by learning more about kidney failure, treatment options, how to be your own advocate, and connecting with other patients.

- ▶ American Association of Kidney Patients  
<https://aakp.org/>
- ▶ American Kidney Fund  
<https://www.kidneyfund.org/>
- ▶ Dialysis Patient Citizens  
<https://www.dialysispatients.org/>
- ▶ ESRD National Coordinating Center  
<https://www.esrdncc.org/>
- ▶ Home Dialyzers United  
<https://homedialyzersunited.org/>
- ▶ Medical Education Institute  
<https://meiresearch.org/>
- ▶ National Institutes of Health  
<https://www.niddk.nih.gov/health-information/kidney-disease>
- ▶ National Kidney Foundation  
<https://www.kidney.org/>
- ▶ Veterans Health Administration  
<https://www.va.gov/health/services/renal/learn.asp>