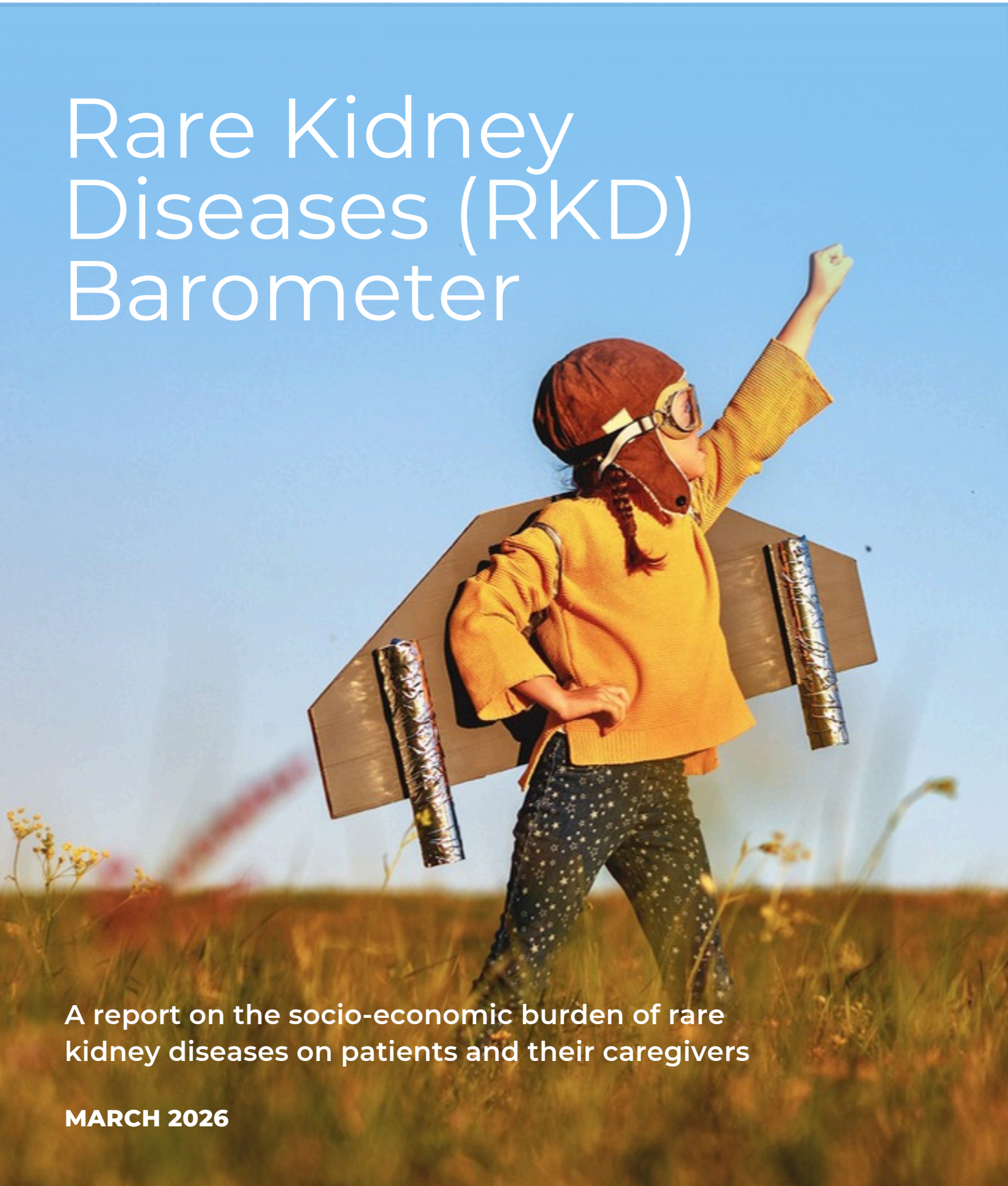
Rare Kidney Diseases (RKD) Barometer



A report on the socio-economic burden of rare kidney diseases on patients and their caregivers

MARCH 2026

About the Barometer

Expert Advisory Group

TheExpert Advisory Group comprising clinicians, researchers, and patient organisation representatives was convened by SOBI to guide the design, interpretation, and validation of all methodological components. The group operated independently, with full autonomy over scientific and strategic decisions regarding the selection of evidence, interpretation of findings, or formulation of recommendations.

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Foreword



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It is my great pleasure to introduce this rare kidney disease barometer report, which combines the efforts of patients, caregivers, patient organisations, expert adult and paediatric nephrologists as well as industry to ensure that rare kidney diseases are recognised in public health agendas.

Kidney health has too often been neglected despite its profound human and societal impact. The evidence assembled in the **Rare Kidney Diseases (RKD) Barometer** makes that silence impossible to maintain. Although each RKD affects a small number of patients, but together they contribute to a significant fraction of chronic kidney disease (CKD). CKD was responsible for the death of 1.48 million adults (aged 20 and older) in 2023 and is projected to become the fifth leading cause of quality life years lost within the next decades. When RKDs go unrecognized, the result is delayed diagnosis, fragmented care, and lost opportunities to slow or even prevent progression to kidney failure- ultimately impacting patients quality of life and life expectancy

The Barometer focuses on eight rare glomerular diseases that can lead to kidney failure. Although these conditions affect relatively small populations of both children and adults, their consequences are disproportionately large on healthcare systems. People living with a rare kidney disease encounter diagnostic delays measured in years, arrive late to specialist care, and face life altering therapies such as dialysis and transplantation - events that reverberate through families, schools, and workplaces. Caregivers shoulder substantial emotional and financial burdens, often with limited formal support.

What sets this Barometer apart is its integrated approach, bringing together relevant literature, patient and caregiver surveys across several European countries, and expert review. The picture that emerges is compelling: rare kidney diseases often progress silently; pathways from primary care to nephrology are uneven; and access to innovation is hindered by evidence expectations that do not always reflect disease evolution or the outcomes that matter most to patients.

Implementing the calls to action, which I urge you to read in the report, is not merely a technical exercise, but **a moral imperative**. Earlier detection and coordinated, multidisciplinary care can delay or avoid kidney replacement therapy and preserve life opportunities, especially for children and working age adults. Aligning health technology assessment frameworks and clinical guidelines with current science and lived experience will enable timely and equitable access to targeted treatments. Strengthening registries and data standards will accelerate learning and progressively improve decisions.

Europe now has a window to act. As cardiovascular prevention frameworks evolve, kidney health must be woven into their fabric, not as an afterthought, but as a central pillar of cardio renal metabolic well being. By providing a common evidence base, the RKD Barometer equips policymakers, clinicians, patient groups, and payers with the insight needed to finally bring RKD out of the shadows and drive coordinated action. Let us use it to move decisively from late rescue to **early intervention**, from fragmented pathways to **integrated care**, and from invisible burden to **visible, measurable progress**. The life and future of people living with rare kidney diseases and their caregivers demand nothing less.

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Executive summary

InEurope, rare diseasesaredefinedas affecting fewer than 5 in 10,000 people [1]. Rare kidney diseases (RKDs) are a heterogeneous group of genetic, immune-mediated or complement-driven conditions that damage the kidneys. The number of people living with a rare kidney disease (RKD) account for <5% of the total number of people living with chronic kidney disease [2]. RKDs often progress silently, with symptoms that tend to be overlooked in primary care settings. As a result, many people living with an RKD are diagnosed later in life, once their kidneys have already undergone irreversible damage, demonstrating the persistent gaps in early detection and timely referral.

The RKD barometer addresses a subset of eight glomerular diseases that can cause kidney failure, including C3G, pICMPGN, FSGS, IgAN, LN, MN, AAV and anti-GBM disease1. Due to their low prevalence and wide range of possible underlying causes, these conditions are particularly complex to diagnose, which makes doing so accurately incredibly challenging.

The RKD Barometer contributes to a growing body of evidence analysing the clinical, psychosocial, and socio-economic burden of RKDs across Europe. The results from the Barometer reveal a repeated pattern across European healthcare systems: diagnostic delays of up to 5 years from disease onset, fragmented care pathways, lack of/limited access to innovative treatments, and insufficient social protection contribute to the poor outcomes that people living with an RKD, and their caregivers, face. Out of the 73 patients surveyed, nearly half (45%, n=33) waited more than one year for a diagnosis, and almost a third (31%, n=23) waited more than three years. One in three had already undergone dialysis and one in four had undergone transplantations, both of which are associated with profound disruption to patients' quality of life.

> *"The post-transplant journey has been very difficult. Maintaining concentration at work is tiring. Unfortunately, I still can't apply for early retirement because I am not yet 56 so I still have three more to go. I'm subjected to constant checks by the medical board to confirm my disability and I always have to explain to work why I'm absent if I have to go to a doctor or get my medicines."*
>
> — C3G PATIENT, FEMALE, 41-65, ITALY

Dialysis patients experience the highest rates of absenteeism, with 83% reporting more than a month of missed work or school in the past six months. 35 caregivers who responded to the survey reported parallel burdens, including income loss (32%, n=12), reduced attendance at work/school (65%, n=23), and emotional strain (80%, n=28) because of the caregiving. It's important to note that paediatric patients often progress to more severe forms of RKD, and face additional challenges around self-management, mental burden, and access issues when they eventually transition to adult care.

Chronic kidney diseases (CKD) which includes rare kidney diseases are projected to become the fifth leading cause of disability-adjusted life years lost – a metric representing the years a patient could have lived in perfect health if not for their disease – globally,

1 C3G = C3 glomerulopathy; pIC-MPGN = primary immune-complex membranoproliferative glomerulonephritis; FSGS = focal segmental glomerulosclerosis; IgAN = IgA nephropathy; LN = lupus nephritis; MN = membranous nephropathy; AAV = ANCA-associated vasculitis; anti-GBM disease = anti-glomerular basement membrane disease.

by 2040 [3] [4] [5]. Despite this, kidney health remains largely invisible in both European and national policy frameworks and is not systematically integrated into noncommunicable disease (NCD) strategies, cardiovascular plans, or rare disease policies. This lack of visibility deepens the awareness gap and contributes to under-investment in disease prevention, early detection, multidisciplinary care and research.

At the same time, there is a strong clinical push to slow the progression of RKDs, with new therapies emerging. However, securing access to these therapies remains slow and challenging. Current health technology assessment (HTA) frameworks often overlook disease specific evidence needs; rely more on long term endpoints like end-stage renal disease or overall mortality than on shorter-term metrics; and systematically reject (or challenge) clinical evidence based on surrogate markers like proteinuria, albuminuria, haematuria and estimated glomerular filtration rate. As a result, targeted therapeutic options that could delay dialysis, improve quality of life, or reduce long term costs are undervalued and underutilised.

Calls to Action

The Rare Kidney Disease Barometer highlights the urgent need for cohesive policy action across four broad areas: public health prioritisation, early detection, socio economic support, and HTA reform to improve outcomes for patients and caregivers across Europe. These calls to action are informed, and supported by, both literature and market research with patients and caregivers across several European countries.

1. Recognise chronic and rare kidney diseases as public health priorities.

RKDs must be formally integrated into EU and national health strategies to unlock coordinated investment, improve visibility, and drive earlier intervention.

2. Streamline investment of financial and healthcare resources towards early detection and faster referral to prevent and slow disease progression.

Systematic kidney health checks and clear referral pathways are essential to identify disease earlier, prevent irreversible damage, and reduce the need for dialysis and transplantation.

3. Reduce the socio-economic burden on patients and their caregivers by strengthening care pathways and offering cohesive legal and financial support mechanisms.

Patients and caregivers need consistent psychosocial, financial, and workplace support to mitigate the profound daily, emotional, and economic impact of RKDs.

4. Ensure that health technology assessments (HTA) and reimbursement decisions for rare kidney diseases (RKDs) reflect the full clinical and patient experience.

HTA frameworks must recognise the disease specific evidence that is currently disregarded, including surrogate endpoints and quality of life aspects, to enable timely and equitable access to targeted therapeutic options.

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Introduction

Chronickidneydisease (CKD)representsa rapidly escalating global health challenge. In 2023, 788 million adults were living with chronic kidney diseases (CKDs) globally, more than double the number in 1990. CKD was responsible for the death of 1.48 million adults (aged 20 and older) in 2023 and is projected to become the fifth leading cause of quality life years lost by 2040. Kidney dysfunction is also a leading cardiovascular risk factor, underscoring its central role in cardio-renal-metabolic health. Overall, these findings demonstrate that CKD is a major driver of cardiovascular and metabolic complications, making it a critically under-recognised public health priority.

Rare kidney diseases are a heterogeneous group of disorders that may affect different parts of the nephrons. They may involve the glomerulus, leading to glomerular diseases; the renal tubules, causing tubular disorders; the interstitial, resulting in tubulointerstitial diseases; or the renal blood vessels, leading to vascular kidney diseases. RKDs tend to progress more rapidly than non-rare CKD, with many patients experiencing early and severe loss of kidney function, particularly in children and young adults, that leads to kidney failure.

Despite the speed at which they progress, the diagnosis of RKDs is often delayed, meaning the disease is already severe by the time it's found. Limited awareness in primary care settings, non-specific symptoms, and fragmented referral pathways all contribute to this delay, as kidney biopsies are essential for disease-specific diagnosis, but access to them is varied across Europe, driving underdiagnosis even further.

Current care models remain heavily focused on treating kidney failure, rather than preventing it from happening in the first place. Historically, drug development for rare kidney diseases has been slow. This due in part to the requirement for hard clinical endpoints (e.g., avoidance of dialysis or transplantation). But, due to regulatory changes which allow the use of softer, surrogate endpoints, the number of publications reporting clinical research into rare kidney diseases increased by 123% in the last decade [6]. Despite a strong clinical push to slow disease progression, patients' unmet needs remain high, as do the rates of kidney failure, because RKDs significantly increase this vulnerability.

The creation of large kidney disease registries like ERKReg [7], RaDaR [8] and GLOSEN [9] is improving clinical trial feasibility and accelerating knowledge generation. However, CKDs and RKDs remain largely invisible in EU and national policy frameworks.
The EU continues to neglect kidney health, with insufficient funding for targeted action. Many national governments do not recognise CKD or RKD as public health priorities. The EU cardiovascular plan "Safe Hearts", published in December 2025, offers a critical opportunity to integrate kidney health and strengthen screening measures.

Why this Barometer

The Barometer was developed to contribute to the body of evidence about the disease burden on patients and caregivers, and to present a comprehensive perspective to policymakers on the need for immediate action to preserve kidney function and delay progression of kidney disease. The RKD Barometer focuses on the conditions C3G, pIC-MPGN, FSGS, IgAN, LN, MN, AAV, and anti-GBM disease, as these patients face severe reductions in life expectancy compared to the general population.

Methodology

The RKD Barometer was developed through a structured, multi step process combining targeted literature review, a patient and caregiver survey, and expert validation (see Figure 1). This approach ensured that the findings reflect both the scientific literature and the lived realities of people affected by RKDs. The main methodological components of the Barometer development included:

1. **Targeted Literature Review.** A targeted literature review of 864 articles and peer reviewed studies, complemented by clinical guidelines (including KDIGO and ERKNet), regulatory documents, and other reports. The review focused on Europe and covered publications from the past ten years.
2. **Patient and Caregiver Surveys.** Two surveys, for patients and caregivers respectively, were conducted in four countries (France, Germany, Italy, and Spain) to capture quantitative and qualitative data on diagnosis, treatment experience, quality of life, mental health, socio economic impact, and caregiver burden using KDQOL, WPAI General Health, and Chronic Pain questionnaires.
3. **Data Synthesis.** Insights from the literature review and surveys were consolidated to identify converging trends, unmet needs, and systemic gaps across clinical, psychosocial, and economic domains. This strengthened the robustness of the evidence base and highlighted areas where published data remains limited.
4. **Expert Advisory Group Review.** A multidisciplinary Expert Advisory Group comprising of paediatric and adult nephrologists, patients and caregivers, health economists, and policy specialists advised on each stage of the process. The group validated the research protocols, interpreted findings, and ensured that the analysis remained clinically accurate, contextually relevant for all relevant parties including patients, health professionals, and policymakers, and aligned with current kidney health policy discussions.

Figure 1. A visual representation of the RKD Barometer development process.



More details on methodology and limitations are available in **Appendix 2.**

Results

ThisBarometerbringstogether survey findings, published evidence, and expert perspectives to provide the most comprehensive picture to date of the clinical, psychosocial, and socio economic burden of RKDs across Europe. These insights reveal a striking picture: rare kidney diseases reduce life expectancy, are under recognised, and are insufficiently supported by the current care pathways, reimbursement frameworks, and social protection systems. While the survey responses are relatively small in numbers, reflecting the rare nature of glomerular diseases, they are consistent across countries and with the current literature on the topic.

1. Silent progression and diagnostic delays. The symptoms of RKD are often not recognised; diagnostic delays are common across the entire subset of diseases included diagnostic delays of over 1 year were reported by close to half of all patients (n=32 out of 73). Unfortunately, delayed diagnosis reduces patients' chance to preserve their kidney function.

2. Complexity of diagnosis and fragmented referral pathways. Survey data shows that 64% (n=47) of patients attributed diagnostic delays to lack of clinician awareness, especially in primary care settings.

3. Disease recurrence after transplantation. Recurrence after transplantation is a significant clinical issue: 28% of the small number of transplanted patients (n=5) reported recurrence, and 11% (n=2) required multiple kidney transplants.

> *"If I had caught it early, it wouldn't have had such an impact on my lifestyle."*
>
> — C3G PATIENT, FEMALE, 18-40, ITALY

> *"The transplant has given back quality of life. Of course, many doctor appointments, blood tests, restrictions due to medication treatments, and the fear that kidney function will decline again remain."*
>
> — FSGS PATIENT, MALE, <18, GERMANY

4. Humanistic, psychosocial, and socio economic burden. Humanistic and societal burden is both substantial and misunderstood. Barometer data shows loss on quality of life (QoL), absenteeism from work and school, emotional upset, and caregiver income loss. The mental health and psychosocial needs are significant, and unmet: combined evidence from the survey and literature shows that anxiety and depression are the most reported psychosocial impacts among patients with a rare kidney disease and their caregivers. 83% of patients on dialysis (n=16) reported major limitations in their social life, personal appearance, sex life, and ability to travel, whilst 67% (n=13) undergoing dialysis reported social exclusion followed by isolation (P value 0.001)2 Among patients not on kidney replacement, 59% (n=43) were burdened by significant changes in diet (e.g., low NaCl allowed intake). All dialysis patients (n=19) and 62% of transplanted patients (n=11) reported significant disruptions to work or school, whilst 83% (n=16) of dialysis patients reported having missed more than one month of school or work in the previous 6 months (P value 0.006)3. Many caregivers [72% (n=25)] indicate that their work or education performance is adversely affected, often experiencing higher rates of absenteeism, whilst one in three caregivers experience a loss of income amounting to more than 500 EUR per month because of their caregiving duties.

2 Based on univariate regression models, accounting for patients not on dialysis or transplantation (reference group) compared to patients on dialysis (past and current) who have not been transplanted. Patients with current or past dialysis exhibited significantly higher odds of experiencing a high impact on daily lives

3 Idem

A deep dive in the calls to action

01

Recognise chronic and rare kidney diseases as public health priorities.

RKDs must be formally integrated into EU and national health strategies to unlock coordinated investment, improve visibility, and drive earlier intervention.

1.1 Include CKD and RKDs in the broader EU and national cardiovascular policies.

1.2 Mandate integrated multidisciplinary care models within national health strategies ensuring collaboration between primary care, cardio-renal-metabolic, and other relevant specialities to optimise diagnosis and outcomes.

1.3 Implement comprehensive, standardised collection of clinical data into national and European-wide registries – ensuring disease progression from early stages and disease outcomes for all kidney diseases (including rare kidney diseases) are covered – to strengthen evidence generation and inform policy decisions.

02

Streamline investment of financial and healthcare resources towards early detection and faster referral to prevent and slow disease progression.

Systematic kidney health checks and clear referral pathways are essential to identify disease earlier, prevent irreversible damage, and reduce the need for dialysis and transplantation.

2.1 Regular kidney health checks based on KDIGO-recommended creatinine and uACR screening to be included in the EU protocol for cardiovascular health checks.

2.2 Include screening for at-risk populations (family history of kidney disease, CKD of unknown aetiology, early onset CKD) before the age of 35 in national health insurance coverage. Strengthen the reference centre model in each Member State, and implement an early referral system to nephrologists upon identification of abnormal kidney markers.

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A deep dive in the calls to action

03

Reduce the socio-economic burden on patients and their caregivers by strengthening care pathways and offering cohesive legal and financial support mechanisms.

Patients and caregivers need consistent psychosocial, financial, and workplace support to mitigate the profound daily, emotional, and economic impact of RKDs.

3.1 Integrate psychosocial support in multidisciplinary care, including patient navigation support in reference centres. Facilitate family counselling and peer support groups.

3.2 Ensure early recognition of disability status and facilitate workplace and school accommodations for patients undergoing dialysis and transplantation. Recognise caregiver status and improve access to financial support schemes for caregivers of paediatric patients.

3.3 Standardise transition protocols for paediatric-to-adult care including age-flexible transfer (18-21 years old), readiness assessments, transition-specific psychological support, and post-transfer follow up.

04

Ensure that health technology assessments (HTA) and reimbursement decisions for rare kidney diseases (RKDs) reflect the full clinical and patient experience.

HTA frameworks must recognise the disease specific evidence that is currently disregarded, including surrogate endpoints and quality of life aspects, to enable timely and equitable access to targeted therapeutic options.

4.1 Ensure recognition by HTA bodies and payers of registries data and disease-specific predictive markers, such as albuminuria, proteinuria and eGFR, that are used by EMA as indicators of clinical benefit and predictors for kidney survival.

4.2 Accelerate clinical guideline updates to reflect emerging evidence and patient quality of life aspects and thus enable earlier access to innovative therapies.

4.3 Mandate systematic collection of data on household financial burden and productivity loss (income loss, absenteeism, catastrophic health spending) associated with kidney disease and its treatments, including innovative medicines. Use these insights to inform policy adjustments at national level.

CALL TOACTION 01

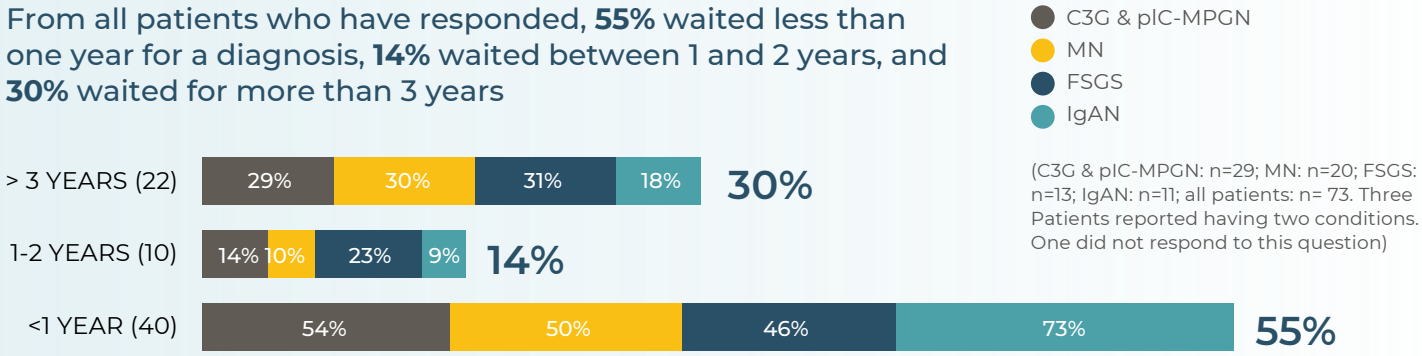
Recognisechronic and rare kidney diseases as public health priorities

The Challenge

Chronickidneydiseases(CKD) represent a major and growing public health burden. Within CKDs, RKDs are heterogeneous, progressive, and often go undiagnosed, as shown by the barometer evidence (see Figure 2) Many RKDs begin in childhood or early adulthood, meaning the burden of these conditions falls disproportionately on school age and working-age populations and families. Due to their rare nature, RKDs tend to be diagnosed later in life, and after the patient has already progressed to kidney failure. As shown by the barometer evidence and supporting literature, many of these glomerular diseases display rapid progression that leads to overdependence on dialysis [10] [11] [12] [13]. Although affecting fewer than 5% of people with CKD, rare kidney diseases cause over a quarter of adult, and more than half of paediatric, kidney failure, meaning that effective therapies could yield disproportionate clinical and economic benefits [2].

Fig. 2 - Number of years it took to receive the final correct diagnosis for all patients vs. specific diseases

From all patients who have responded, **55%** waited less than one year for a diagnosis, **14%** waited between 1 and 2 years, and **30%** waited for more than 3 years



(C3G & pIC-MPGN: n=29; MN: n=20; FSGS: n=13; IgAN: n=11; all patients: n= 73. Three Patients reported having two conditions. One did not respond to this question)

The Policy Gap

Althoughlarge-sizeregistries provide valuable disease knowledge, RKDs remain largely invisible in policy frameworks. Kidney health is not systematically integrated into cardiovascular or metabolic strategies, despite strong evidence linking kidney dysfunction to cardiovascular morbidity and mortality. As a result, people living with RKDs and their caregivers do not benefit from national strategic prioritisation, investment, or coordination between healthcare, employment, and social protection policies. As a result, patients and their caregivers often feel that rare kidney diseases are forgotten or ignored.

The sum of these gaps is fragmented care pathways, limited investment in research and innovation, delayed access to new treatments, and missed opportunities for prevention and early intervention. Without explicit recognition of kidney health as a public health priority, the burden of RKDs will continue to grow for patients, caregivers, and health systems alike.

What needs to change

1.1. Include CKD and RKDs in the broader EU and national cardiovascular policies.

Despite being a major driver of cardiovascular and metabolic complications, kidney disease remains absent from NCD strategies. Formal recognition is essential to unlock funding, improve coordination, and elevate kidney health as a public health priority. As of 2026, numerous stakeholders including patient and scientific organisations are engaged in activities to raise awareness of chronic and rare kidney diseases in ongoing policy discussions on the EU Safe Hearts Plan and to promote a European Parliament Resolution on Kidney Health.

Policy Levers:

- Integrate CKD and RKD into EU and national cardiovascular and metabolic health plans
- Promote kidney health screening as a core prevention measure
- Use the Barometer data to highlight lived experience and systemic burden in policy dialogues

1.2. Mandate integrated multidisciplinary care models within national health strategies ensuring collaboration between primary care, cardio-renal-metabolic, and other relevant specialities to optimise diagnosis and outcomes.

CKD and RKDs rarely occur in isolation. Patients often present with blood pressure, diabetes, cardiovascular disease, or autoimmune conditions. Multidisciplinary care improves outcomes but is inconsistently implemented.

Policy Levers:

- Formalise care pathways linking primary care, nephrology, cardiology, diabetology, rheumatology, psychology, nutrition, and social services
- Introduce bundled payment models to incentivise integrated care
- Monitor quality indicators such as CKD progression, cardiovascular events, and patient satisfaction

1.3. Implement comprehensive, standardised collection of clinical data into national and European-wide registries – ensuring disease progression from early stages and disease outcomes for all kidney diseases (including rare kidney diseases) are covered – to strengthen evidence generation and inform policy decisions.

RKDs affect small populations and are clinically diverse, making evidence generation difficult. Systematic data collection, as per FAIR data principles, strengthens regulatory and policy decisions, supports trial recruitment, and accelerates access to innovation.

Policy Levers:

- Establish a core dataset that combines patient reported outcomes (PROs) with clinical outcomes
- Ensure transparent data sharing with researchers, regulators, patient organisations, and industry
- Recognise registry data as valid evidence for HTA and regulatory submissions.



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CALL TOACTION 02

Streamlineinvestment of financial and healthcare resources towards early detection andfaster referral to prevent and slow disease progression

The Challenge

ManypeoplelivingwithRKDs have increased levels of albuminuria, proteinuria, haematuria, blood pressure, and even develop oedema. Despite this, early referrals to nephrologists are often delayed due to lack of awareness of the symptoms amongst primary care providers. Diagnosis is complex due to the heterogeneous nature of rare glomerular diseases and the need for biopsy to confirm diagnosis, both of which are challenging to secure due to systemic factors and/or patient preference [11] [13] [14]. These challenges are compounded by the limited availability of nephrologists trained in rare kidney diseases and the asymmetric concentration of specialist centres in major cities, creating barriers for rural and semi rural patients [11] [13] [15].

Patients face a high risk of progression to kidney failure/requirement of kidney replacement therapy (KRT) – dialysis or transplantation – which brings a high emotional toll, amplified by the fact that many patients and their caregivers must make many work and life compromises to accommodate the condition. In particular, literature finds glomerular diseases susceptible to rapid progression and overdependence on dialysis [11] [12] [13].

Dialysis imposes substantial constraints: strict dietary and fluid restrictions; high risk of complications like central venous stenosis, infection, and cardiovascular events; frequent hospital events; and reduced survival rates: dialysis can drastically reduce patients' 5-year survival rates [16]. These burdens affect both children and adults, affecting patients across ages, and cause significant disruptions to the daily lives of caregivers. Barometer data shows that 83% of dialysis patients missed more than one month of work or school in six months, and up to 67% experienced major limitations in their social lives.

> *"Knowing that at any moment it could worsen, leading to dialysis and a transplantation, makes me feel like I'm living within a Russian roulette."*
>
> — FSGS PATIENT, FEMALE, 18-40, ITALY

Although transplantation improves quality of life and chances of survival, challenges remain.

> *"They declare us healed, but we are not; we have to take 17/18 pills a day, and each one has its own side effects."*
>
> — pIC-MPGN PATIENT, FEMALE, 41-65, ITALY

Disease recurrence post-transplant, or de novo kidney disease, affects a significant proportion of patients, leading to allograft dysfunction and, in some cases, graft

loss. Published evidence shows variable recurrence risks across rare glomerular diseases, with important implications for transplant decisions and long term outcomes [11] [13] [17] [18] [19]. The emotional toll of graft failure makes decisions about re transplantation particularly difficult.

The Policy Gap

Both the evidence from the literature and the survey underscores the need for systematic kidney health checks; early recognition of albuminuria, proteinuria and haematuria; and timely referral to nephrology to slow progression and reduce the need for kidney replacement therapy (KRT). Despite the risk of kidney failure, increased comorbidities, and the heavy burden of KRT on patients and health systems, early detection is not systematically implemented across Europe. Across the Member States, there is no routine kidney health screening in primary care, the fact of which is made worse by low physician awareness of early CKD signs.

As a result, CKD and RKD patients often reach nephrology care too late, when the kidney damage is already irreversible and treatment options are limited. This leads to progression to dialysis or kidney transplantation that could have potentially been avoided, higher healthcare costs, and greater emotional and economic burden for patients and families. This delay also highlights the need for stronger primary care training, clearer referral pathways, and equitable access to specialist nephrology services.

What needs to change

2.1 Regular kidney health checks based on KDIGO-recommended creatinine and uACR screening to be included in the EU protocol for cardiovascular health checks.

People living with RKDs have the highest risk of progressing to kidney failure compared to those with other forms of the kidney disease. Despite this threat, barometer data shows that 64% of patients attributed diagnostic delays for RKDs to lack of physician awareness. Early detection through simple tests in primary care settings can identify kidney impairment long before symptoms appear, preventing irreversible damage and improving long-term outcomes.



Policy Levers:

- Mandate kidney checks in EU and national public health programmes (e.g., the EU Cardiovascular Health Plan)
- Promote and enable routine urine screening (albuminuria, urinary albumin-creatinine ratio – uACR, proteinuria,haematuria) in primary care settings and for high risk groups
- Provide training modules on CKD risk assessment and early signs of RKDs
- Introduce electronic health record prompts for annual kidney checks

2.2 Include screening for at-risk populations (family history of kidney disease, CKD of unknown aetiology, early onset CKD) before the age of 35 in national health insurance coverage. Strengthen the reference centre model in each Member State, and implement an early referral system to nephrologists upon identification of abnormal kidney markers.

RKDs often progress silently, until an advanced stage of chronic kidney disease is reached. Early risk stratification including family history, early onset CKD, or unexplained albuminuria, proteinuria or haematuria can reduce diagnostic delays and avoid repeated invasive procedures.

Policy Levers:

- Integrate eGFR, uACR, and risk checklists into primary care protocols
- Establish fast track referral pathways for abnormal kidney markers
- Expand access to genetic testing for at risk groups
- Align referral protocols with KDIGO guidelines

CALL TOACTION 03

Reducethesocio-economic burden on patientsand their caregivers by strengtheningcarepathways and offering cohesive legal and financial support mechanisms

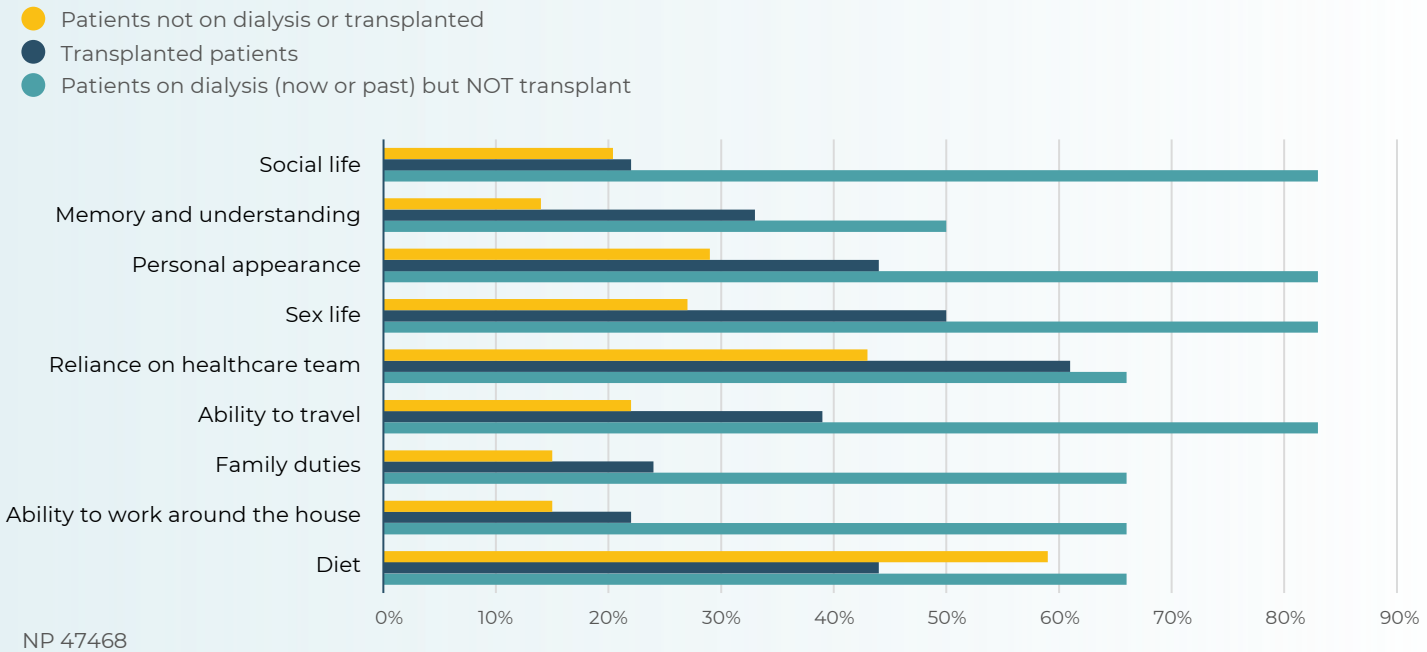
The Challenge

RKDsplace significant socio economic and emotional challenges on patients and caregivers , as indicated by the barometer evidence (see Figure 3 and Figure 4). Hospitalisations, lengthy dialysis, and transplantations frequently disrupt daily routines, work, and school, leading to decreased productivity, absenteeism, emotional stress, and financial strain. Over three quarters of caregivers report an impact on their work, and one in three experience income loss or reduced hours due to caregiving duties.

Published evidence confirms these findings, highlighting substantial quality of life loss, mental

> *"I was 39, with a 1.5-year-old child and an 8-year-old, and I was self-employed. I couldn't work and I had difficulty taking care of the children, even just taking them to school. During those years, my children had to give up sports and all the activities that children usually do because I couldn't leave the house. I worked when I could, but at a certain point I quit. Those were difficult years."*
>
> — FSGS PATIENT, FEMALE, 41-65, ITALY

Fig. 3 - Mental health impact on patients , as reported using KDQOL™-36; KDQOL-SF-1.3 as sources



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health impacts, and caregiver burden that remain under recognised in routine care and frameworks for value assessment [11] [20] [21] [15] [22] [23].

Long- term dialysis is associated with significant lifestyle limitations, increased social isolation, reduced autonomy, and higher mortality (about 25% in the first year of dialysis in high income countries) [24]. Mental health concerns are widespread, underscoring a major gap in integrated psychosocial support.

Education and work performance may decline due to fatigue, treatment schedules, and medical appointments, while personal and societal healthcare costs escalate with disease severity, comorbidities, and hospitalisation rates.

Although paediatric patients represented only a small proportion of the survey sample (approx. 8%), they often experience severe disease trajectories, often culminating in early transplantation. This challenge is compounded by a transition to adult care that is often fragmented, increasing their vulnerability during a critical developmental period. Moreover, transition failures can lead to non-adherence, delays in medical follow-ups, and potential graft loss. Literature recommends structured transition pathways and age-appropriate support to ensure continuity of care [25] [26].

Caregivers face distinct challenges: complex care demands disrupt daily routines, elevate stress levels, and contribute to burnout.

The Policy Gap

Support systems for people living with RKD, and their caregivers, across Europe are inconsistent and often inadequate. Access to mental health services, recognition of disability status, workplace accommodations, and structured paediatric to adult transition pathways vary widely, leaving many without essential legal, financial, or psychosocial support.

Mental health care is seldom integrated within nephrology services (mostly due to lack of reimbursement), despite high rates of anxiety and depression among both patients and caregivers. Social protection mechanisms including sick leave, disability benefits, and caregiver allowances differ significantly between countries, and are often difficult to obtain. Insufficient workplace and educational accommodations further contribute to education setbacks, workforce exclusion, and long term economic vulnerability.

> *"I feel I'm failing my child because I can't help him towards his mental health as a teenager with a chronic condition."*
>
> — CAREGIVER OF MINOR CHILD, GERMANY

> *"It is heavy for me in terms of time dedicated and trying to be positive to my son and convey trust to him when I'm very worried and sad."*
>
> — CAREGIVER OF ADULT CHILD, ITALY

What Needs to Change

3.1 Integrate psychosocial support in multidisciplinary care, including patient navigation support in reference centres. Facilitate family counselling and peer support groups.

Psychosocial needs are central to RKD management but often overlooked. Integrated support improves adherence, resilience, and wellbeing.

Policy Levers:

- Mandate psychosocial services (psychologists, social workers, patient navigators) in RKD care
- Ensure reimbursement for psychosocial services under national health insurance
- Embed mental health referral pathways in clinical guidelines
- Train psychosocial professionals in rare kidney disease care
- Include mental health and caregiver burden indicators in registries

The majority of caregivers indicate that their work or academic performance is adversely affected, often experiencing higher rates of absenteeism and a decrease in their working hours due to their caregiving duties.

Fig. 4 - How caregiving affected work or school performance (n=35; carers of children = 17; carers of adults = 18)

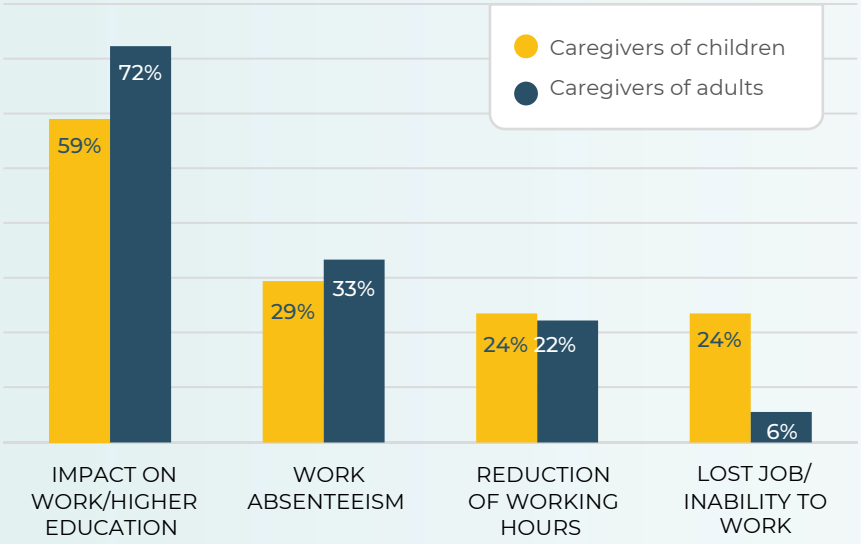
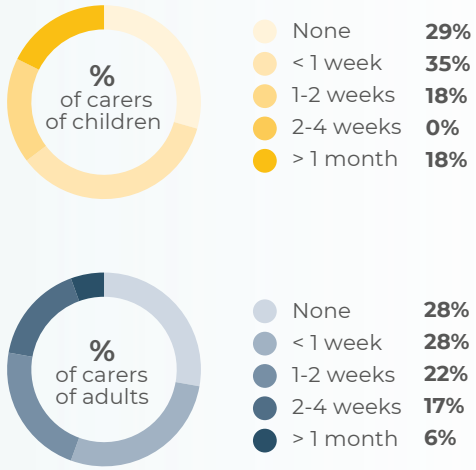


Fig. 5 - Time missed from work or school due to caregiving responsibilities over the previous 6 months (n=35; carers of children = 17; carers of adults = 18)



Sources: KDQOL™-36; KDQOL-SF-1.3

3.2 Ensure dialysis and transplantation facilities are accessible to patients with disability and provide reasonable workplace and school accommodations for patients undergoing dialysis and transplantation. Recognise caregiver status and improve access to financial support schemes for caregivers of paediatric patients.

Dialysis schedules and caregiving responsibilities often conflict with standard work and school structures, increasing the risk of job loss, financial instability, and educational backlog.

Policy Levers:

- Mandate paid medical leave for dialysis
- Introduce caregiver leave for parents of paediatric RKD patients
- Enforce protections against discrimination based on health or caregiving status

3.3 Standardise transition protocols for paediatric-to-adult care including age-flexible transfer (18-21 years old), readiness assessments, transition-specific psychological support, and post-transfer follow up.

Child-to-adult transition is a vulnerable stage for patients, marked by emotional distress and practical burden on patients and their families; fragmented care; and loss of multidisciplinary support they previously had access to via their paediatric care. Some paediatric patients described a loss of continuity of care when moving between services, while others referred to delays or gaps in follow-up after moving to adult services.

Policy Levers:

- Mandate national systems to pilot integrated models for paediatric-to-adult transition clinics
- Mandate ERKNet and European medical societies to co-create, together with patient organisations, standard protocol models for transition of paediatric-to-adult care considering the below components (see figure 6 below).

Fig. 6 - Transition protocol from paediatric to adult care, minum requirements

COMPONENT	KEY ACTIONS
Early Engagement	Begin planning at 11-14 years of age, readiness screening.
Multi-Disciplinary Teams	Ensure coordination with paediatric nephrologist, adult nephrologist, nurse, social worker, and psychologist
Patient & Family Education	Use tools like TRAQ, TRxANSITION; foster self-management skills. HCS should offer age flexibility (18-20 years) when transferring patients to adult care
Transfer of Care Documentation	Electronic medical summaries, patient goals, insurance and medication info (establish a checklist)
Post-Transfer Follow-Up	Scheduled early adult appointments, outreach for missed visits
Outcome Tracking	Monitor transition success, retention, adherence, and clinical stability (paediatric nephrologist as the coordinator)



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CALL TOACTION 04

Ensurethathealthtechnology assessments(HTA)and **reimbursement decisions** for rare kidney diseases (RKDs) **reflect the full clinical and patient experience**

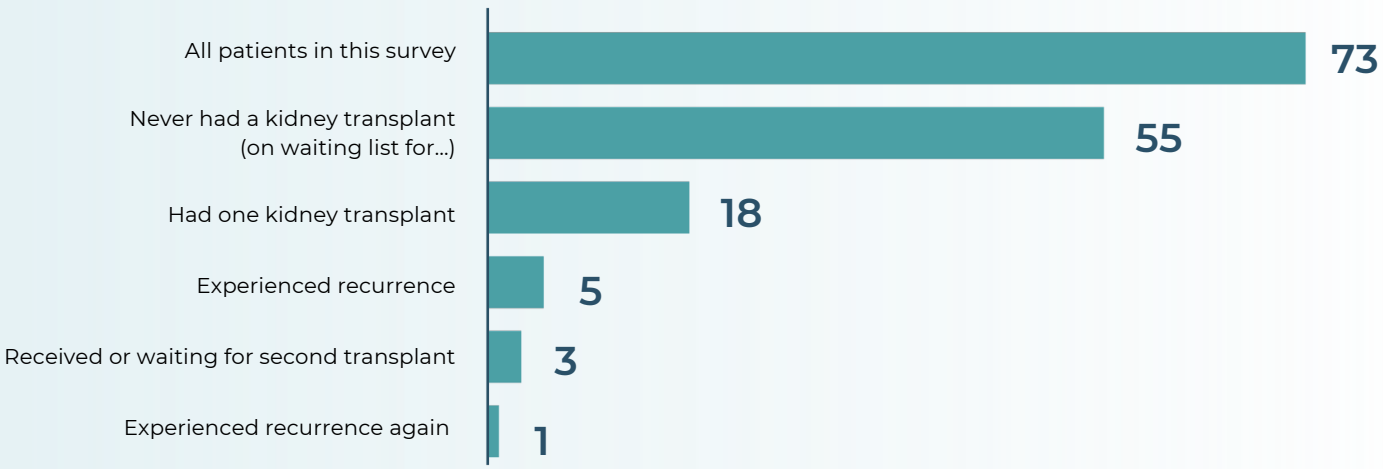
The Challenge

Formany RKDs,delays in diagnosis and limited treatment options leave clinicians with few choices. Current standard of care is mainly supportive: KDIGO guidelines and the literature note reliance on ACEi/ARBs, and in some circumstances SGLT2 inhibition, as first-line treatments and non-targeted immunosuppression for symptom control, despite limitations or side effects including kidney function decline. Disease recurrence post-transplantation further complicates treatment decisions, and contributes to allograft dysfunction and, in many cases, graft loss (see Figure 7). This results in increased dependency on dialysis, which is subsequently associated with a 5-year survival rate as low as 59% in some groups [27].

Despite this high unmet need, HTA bodies often prioritise long term endpoints such as avoided kidney failure or mortality, overlooking outcomes that matter to patients in the process – e.g., quality of life, disease severity, mental health, school attendance, and ability to work and earn wages (see Figure 8). Whilst we have seen regulatory approvals for innovative medicines based on EMA-validated surrogate endpoints, access and reimbursement delays at national level are still encountered, and thus there is a need for medical guideline to be updated, and HTA frameworks to be developed that recognise the value of early disease modification [11] [17] [21] [28] [29].

Fig. 7 - Disease progression

Patients experience disease progression to dialysis and transplantation



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The Policy Gap

Althoughscientificadvances have led to the development of targeted therapies and reliable indicators, policies promoting access to innovative RKD care remain inconsistent across Europe. Surrogate endpoints such as proteinuria and eGFR are recognised by regulators as reasonably likely surrogate endpoints in specific circumstances and occur long before kidney failure, making them essential for clinical trials and HTA evaluations. However, their recognition remains inconsistent across HTA bodies, with gaps in post launch validation and guideline integration. Literature and survey findings highlight the need for HTA frameworks that accept disease specific surrogate markers and incorporate quality of life and disease severity evidence to better reflect real world trajectories [11] [30] [13] [31].

Existing HTA frameworks frequently fail to address the unique evidence requirements for rare kidney diseases, resulting in assessments that may not fully recognise the value of treatments capable of altering disease progression or postponing kidney failure. The absence of surrogate endpoints in HTA evaluations creates discrepancies between EU level regulatory approval and national reimbursement decisions. Moreover, considerations such as the lifetime costs of KRTs, productivity loss, caregiver burden, and the long term economic consequences of early onset disease are seldom incorporated into value assessments. Delays in updating clinical guidelines further widen the gap between scientific progress, regulatory evidence, and reimbursement pathways. As a result, therapies with the potential to delay dialysis, enhance quality of life (often the most important outcome for patients), or reduce long term costs often remain under recognised and underutilised.

What Needs to Change

4.1 Ensure recognition by HTA bodies and payers of registries data and disease-specific predictive markers, such as albuminuria, proteinuria and eGFR, that are used by EMA as indicators of clinical benefit and predictors for kidney survival.

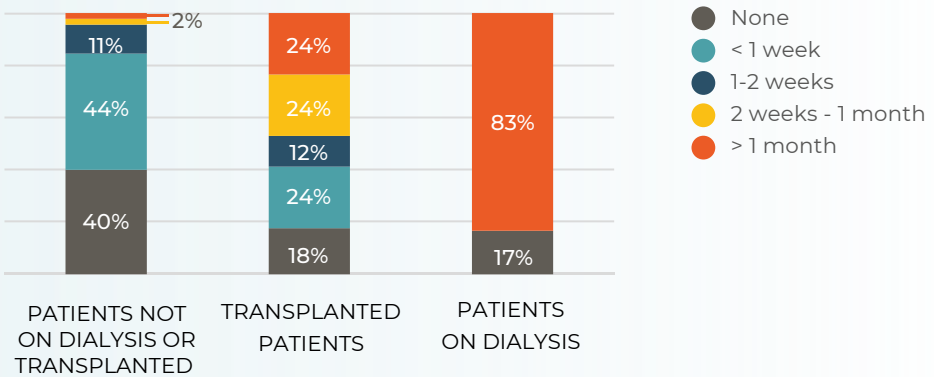
HTA decisions for RKDs often rely on endpoints and cost effectiveness models that do not reflect the realities of progressive, small population diseases. Surrogate endpoints in RKDs such as albuminuria, proteinuria reduction, and eGFR slope have been validated by the EMA as strong predictors of kidney function preservation and should be fully integrated into HTA evaluations. Meaningful patient outcomes, including quality of life and caregiver burden, must also be considered.

Policy Levers:

- Update national and European HTA guidelines to explicitly include surrogate endpoints for RKDs
- Use frameworks such as Orphar SEFH MCDA to support evaluation of rare diseases
- Mandate integration of real world evidence (e.g., registries, observational studies, and patient reported outcome measurements [PROMs])
- Incorporate quality of life and caregiver burden into cost effectiveness and pricing models
- Consider societal and equity impacts in reimbursement decisions
- Formalise multi stakeholder advisory panels, including patients, health professionals, and policymakers, for RKD treatment evaluations

Fig. 8 - Time missed from work or school due to the disease and its management over the previous 6 months (Patients on dialysis (now or past) but NOT transplant: n= 6; transplanted patients: n= 18, patients NOT on kidney replacement therapies; n= 49)

Patients on dialysis experience the highest rates of absenteeism, with 83% reporting more than one month of missed work or school in the past six months



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4.2 Accelerate clinical guideline updates to reflect emerging evidence and patient quality of life aspects and thus enable earlier access to innovative therapies.

Clinical guideline updates for glomerular disease occur infrequently, despite rapid advances in targeted therapies. Current guidance still emphasises ACE inhibitors/ARBs and immunosuppression, which do not address underlying disease mechanisms. Evolving science and disease modifying treatments should be reflected in updated guidelines.

Policy Levers:

- Establish a "living guideline" model with updates every 1–2 years, or as pivotal data emerges
- Create a rapid evidence review task force for high impact trials
- Align coverage policies with updated recommendations through payer–regulator dialogue
- Include patient/caregiver and clinician input to prioritise high need areas (e.g., paediatric RKDs)
- Support global dissemination through WHO and ISN partnerships

4.3 Mandate systematic collection of data on household financial burden and productivity loss (income loss, absenteeism, catastrophic health spending) associated with kidney disease and its treatments, including innovative medicines. Use these insights to inform policy adjustments at national level.

RKDs require long term treatment and frequent hospitalisations, leading to missed school days, missed work days, reduced earning capacity, and high caregiver burden. Patients report direct costs and loss of income that significantly affects household income and overall productivity, yet there is no systematic collection of such data at European or national level, meaning that this important aspect of assessing the impact of RKDs is overlooked in policy or regulatory decisions.

Policy Levers:

- Integrate financial and productivity metrics into HTA frameworks
- Collect data through national registries, insurance claims, and household surveys
- Include indicators such as out of pocket expenditure, income loss, employment changes, and catastrophic health spending thresholds

Conclusion

Rare kidney diseases should not remain a silent threat—progressing unnoticed until lives are disrupted by dialysis, transplantation, and avoidable loss of health and opportunity.

The RKD Barometer shows a consistent pattern across different European healthcare systems: long diagnostic journeys, fragmented care pathways, challenging access to targeted therapeutic options, and inadequate social protection collectively shift the burden onto patients and families.

With standardised, earlier detection in primary care settings, coherent referral pathways, stronger psychosocial and socio economic support, and HTA approaches that reflect the realities of rare kidney diseases, Europe can move from late-stage rescue to earlier intervention—and better lives. The Barometer is a call to act now, together, so that people living with rare kidney diseases are seen, diagnosed earlier, treated fairly, and supported to live fully.

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Appendix 1. Glossary

ACE inhibitors (ACEi): Medicines that lower blood pressure and reduce protein loss in the urine; widely used to slow kidney damage.

Albuminuria / Proteinuria: Excess protein in the urine. A key early warning sign of kidney disease and an important measure of treatment response.

Allograft: A transplanted kidney from another person. Can be affected by rejection or recurrence of the original disease.

AAV (ANCA associated vasculitis): A rare autoimmune disease that inflames small blood vessels, often causing rapid kidney damage.

Anti GBM disease: A rare autoimmune condition where the immune system attacks the kidney filters, leading to sudden and severe kidney failure.

Biopsy (Kidney biopsy): A procedure that removes a tiny piece of kidney tissue for diagnosis. Essential for identifying most rare kidney diseases.

C3G (C3 glomerulopathy): A rare disease caused by overactivation of the complement system, leading to inflammation and scarring in the kidneys.

CKD (Chronic Kidney Disease): A long term decline in kidney function. Can progress silently for years before symptoms appear.

Dialysis: A treatment that cleans the blood when the kidneys can no longer do so. Requires frequent sessions and significantly affects daily life.

eGFR (Estimated Glomerular Filtration Rate): A standard measure of kidney function based on a blood test. Used to track disease progression.

ERKNet: The European Reference Network for Rare Kidney Diseases, providing expert guidance and harmonised care across Europe.

FSGS (Focal Segmental Glomerulosclerosis): A rare disease that causes scarring in parts of the kidney's filtering units, often leading to high levels of proteinuria.

Haematuria: Blood in the urine. Can be visible or detected only through testing; often an early sign of kidney damage.

HTA (Health Technology Assessment): A process used by governments to evaluate the benefits, risks, and costs of new treatments before deciding on reimbursement.

IgAN (IgA nephropathy): A common glomerular disease caused by deposits of IgA antibodies in the kidneys.

Immunosuppression: Medicines that reduce immune system activity. Used to treat many kidney diseases but can cause significant side effects.

KRT (Kidney Replacement Therapy): Treatments used when kidneys fail, including dialysis and kidney transplantation.

KDIGO (Kidney Disease / Improving Global Outcomes): An international organisation that develops global guidelines to standardise how kidney diseases are defined, diagnosed, and managed.

LN (Lupus nephritis): Kidney inflammation caused by lupus, an autoimmune disease.

MN (Membranous nephropathy): An autoimmune kidney disease where antibodies attack the kidney filters, causing swelling and protein loss.

MPGN / pIC MPGN: A rare immune mediated kidney disease involving abnormal immune complex deposits and complement activation.

PROs (Patient reported outcomes): Measures that capture how patients feel and function in daily life, including symptoms, fatigue, and quality of life.

Rare kidney diseases (RKDs): A group of uncommon kidney conditions that often progress quickly and require specialist care.

Registry data: Information collected over time about people living with a disease. Helps track outcomes and support research and policy decisions.

Surrogate endpoints: Early indicators (such as proteinuria reduction or eGFR slope) that predict long term kidney outcomes and are used in clinical trials.

Transplant recurrence: When the original kidney disease returns in the transplanted kidney, potentially affecting its function.

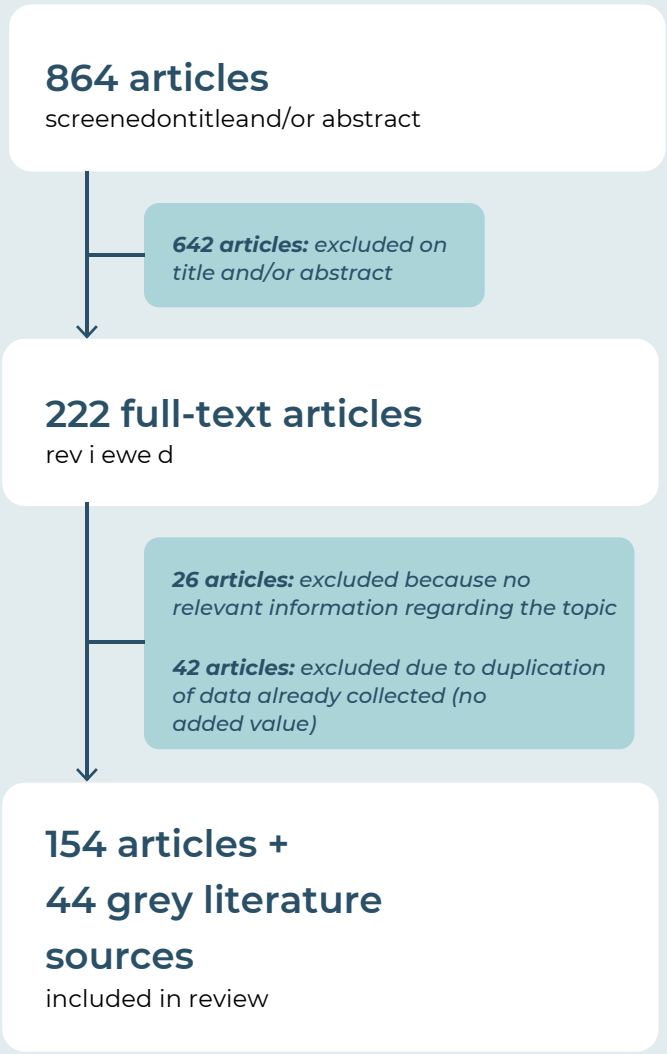
uACR (Urine albumin to creatinine ratio): A simple urine test used to detect early kidney damage and monitor disease progression.

Appendix 2.

Methodology

Literature Review (May-June 2025)

A literature review was conducted to map existing evidence on the clinical, psychosocial, and socio-economic burden of RKDs, with a specific focus on patients whose disease status required kidney replacement therapy (KRT). The review informed the design of the surveys and provided the contextual evidence base for the Barometer.



Objec tives

- Identify published data on the burden of dialysis and transplantation in RKDs
- Characterise disease progression, comorbidities, and risk of kidney failure
- Assess impacts on quality of life, mental health, work, education, and caregiver burden
- Identify evidence gaps requiring primary data collection

Search Strategy and Selection

The review was conducted using PubMed, covering publications from January 2015 to April 2025. The geographical scope included Europe, with a focus on France, Germany, Italy, Spain, and the UK. Eligible publications were in English, French, German, Italian, or Spanish. Included publication types comprised clinical studies, observational studies, reviews, guidelines, meta-analyses, multicentre studies, consensus statements, and congress abstracts. Excluded materials included case reports, biographies, books, interviews, news articles, and audio-visual media.

The search strategy combined disease-specific terms (e.g., C3G, MPGN, FSGS, IgAN, lupus nephritis, membranous nephropathy, anti GBM) with terms related to KRT and outcomes (e.g., dialysis, transplant, progression, quality of life, caregiver burden, employment, financial impact).

Complementary Sources

Grey literature was included from national and international guidelines (KDIGO, ERA, ASN), patient organisations (e.g., CureGN, SONG Initiative), and recent congress abstracts (range: two years).

Return to Methodology

Patient and Caregiver Surveys (August-September 2025)

Two onlinesurveyswereconductedto capture the lived experience and socio-economic burden of RGKDs among patients and informal caregivers.

Study Population and Recruitment

Eligible participants were adults (≥18 years) living in France, Germany, Italy, Spain, or the UK. Patients had a confirmed diagnosis of a RKD; caregivers provided unpaid support to a patient. Recruitment occurred through national and European patient organisations, clinical networks, expert centres, and social media outreach. Participation was voluntary, anonymous, and GDPR compliant. No personal identifiers were collected.

Patient survey responses demographics

Table 1. Distribution of respondents by age

<18	**8%**
18-40	**23%**
41-65	**51%**
>65	**18%**

Table 2. Distribution of respondents by country and type of disease

	Italy	France	Germany	Spain
C3G MN	19	1	1	1
FSGS IgAN	19	0	1	0
pIC-MPGN	7	0	1	5
LN AAV	5	5	0	1
Anti-GBM	7	0	0	0
	1	1	1	0
	0	0	0	0
	0	0	0	0

Caregiver responses demographics

Table 3. Distribution of respondents by country and patient's type of disease

	Italy	France	Germany	Spain
C3G	13	1	3	1
FSGS	1		4	2
IgAN	2	2		1
MN	2	1		
pIC-MPGN			1	1
LN				1
AAV				
Anti-GBM				

Table 4. Distribution of respondents by type of relationship with the patient

Child (<18)	**49%**
Child (>18)	**31%**
Spouse	**14%**
Parent	**6%**

Limitations

ThefindingsoftheBarometer should be interpreted in light of several methodological constraints linked to the nature of the evidence and the challenges of researching rare diseases. The survey relied on voluntary participation through patient organisations, clinical networks, and online outreach. While this approach was necessary to reach a dispersed population, it may over represent individuals who are more engaged, more digitally connected, or living with more advanced disease. People with milder symptoms, limited access to patient communities, or lower digital literacy may be under represented. Differences in sample size across countries and across disease groups also limit the ability to draw robust comparisons or explore certain subgroups in depth.

As with all self reported data, the survey is subject to recall and reporting bias. Respondents may not accurately remember diagnostic timelines, hospitalisations, or financial impacts, and some sensitive questions, particularly those related to income or employment, had higher non response rates. This may lead to an underestimation of the socio economic burden. The cross sectional nature of the survey also means that the Barometer captures a single moment in time and cannot reflect changes in disease progression, treatment access, or psychosocial impact.

The literature review, while systematic, also carries inherent limitations. The streamlined approach may have missed relevant studies, particularly those outside PubMed or published in non indexed national journals. Evidence on caregiver burden and socio economic impact remains limited in the published literature, requiring reliance on small studies or grey literature.

Finally, although the Expert Advisory Group provided multidisciplinary oversight throughout the process, it represents a finite set of perspectives shaped by specific clinical and advocacy contexts. Despite these limitations, the Barometer offers a uniquely integrated view of rare kidney diseases by combining published evidence, lived experience, and expert interpretation, providing a strong foundation for policy dialogue and future research.



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