IMPROVING OUTCOMES FOR INDIVIDUALS WITH RARE KIDNEY DISEASES (RKDs) AND THEIR FAMILIES

THE BASICS

Nearly 300 RKDs. Though individually rare, **together** they are a **significant burden.**

Most RKDs are **genetic** and start in **childhood**.



A **global** problem affecting **<1 in 2000** people.



Lifelong economic, **social** and **emotional** impact.

THE CHALLENGE

LIMITED RESEARCH INVESTMENT

- Most evidence is from **HICs** and focuses on **selected RKDs**.
- Data not always **disaggregated by equity factors**.
- Methodologies should be adapted to consider the specific challenges posed by rare diseases.
- Lack of studies into RKDs other than those conducted by pharmaceutical companies which may not represent all non-market regions and non-drug interventions.

DIAGNOSIS AND MANAGEMENT

Long & challenging to diagnose
- Limited diagnostics
- Unidentified causes

Effective treatments for < 10% RKDs

Global variability in access to diagnostics and treatments

SOCIOECONOMIC IMPACT

Education – Absenteeism, interrupted or delayed education, performance impairment for individuals with RKDs.

Work and employment – Productivity loss and work absenteeism (patients and caregivers), job discontinuation and reduced career advancement (patients)

Out-of-pocket expenses and care affordability
- Costs include: diagnostics, diet-costs, medication, copayments, and transport
- Financial constraints leading to care unaffordability (patients and caregivers)
- Patients with RKDs incur higher costs than those without, driven by direct medical costs.

* Within these, geographic and gender-based disparities exist.

PSYCHOSOCIAL AND EMOTIONAL IMPACT

Anxiety & depression (patients & caregivers)

Inability to engage in social activities (patients & caregivers)

Guilt (caregivers - due to genetic nature of many RKDs)

THE NEED

Rare kidney diseases are lifelong conditions that require a broader understanding of their wider social and economic burden to drive investment and improve support for patients and caregivers across education, employment, and social services.

This fact sheet summarises the key points of the following two papers:

Angell, Blake et al. "Scoping Review of Economic Analyses of Rare Kidney Diseases." *Kidney International Reports* vol. 9,12 3553-3569. 12 Sep. 2024, doi:10.1016/j.ekir.2024.09.004

Palagyi, Anna et al. "Systematic Scoping Review of Socioeconomic Burden and Associated Psychosocial Impact in Patients With Rare Kidney Diseases and Their Caregivers." *Kidney International Reports* vol. 10,3 838-854. 10 Dec. 2024, doi:10.1016/j.ekir.2024.12.005

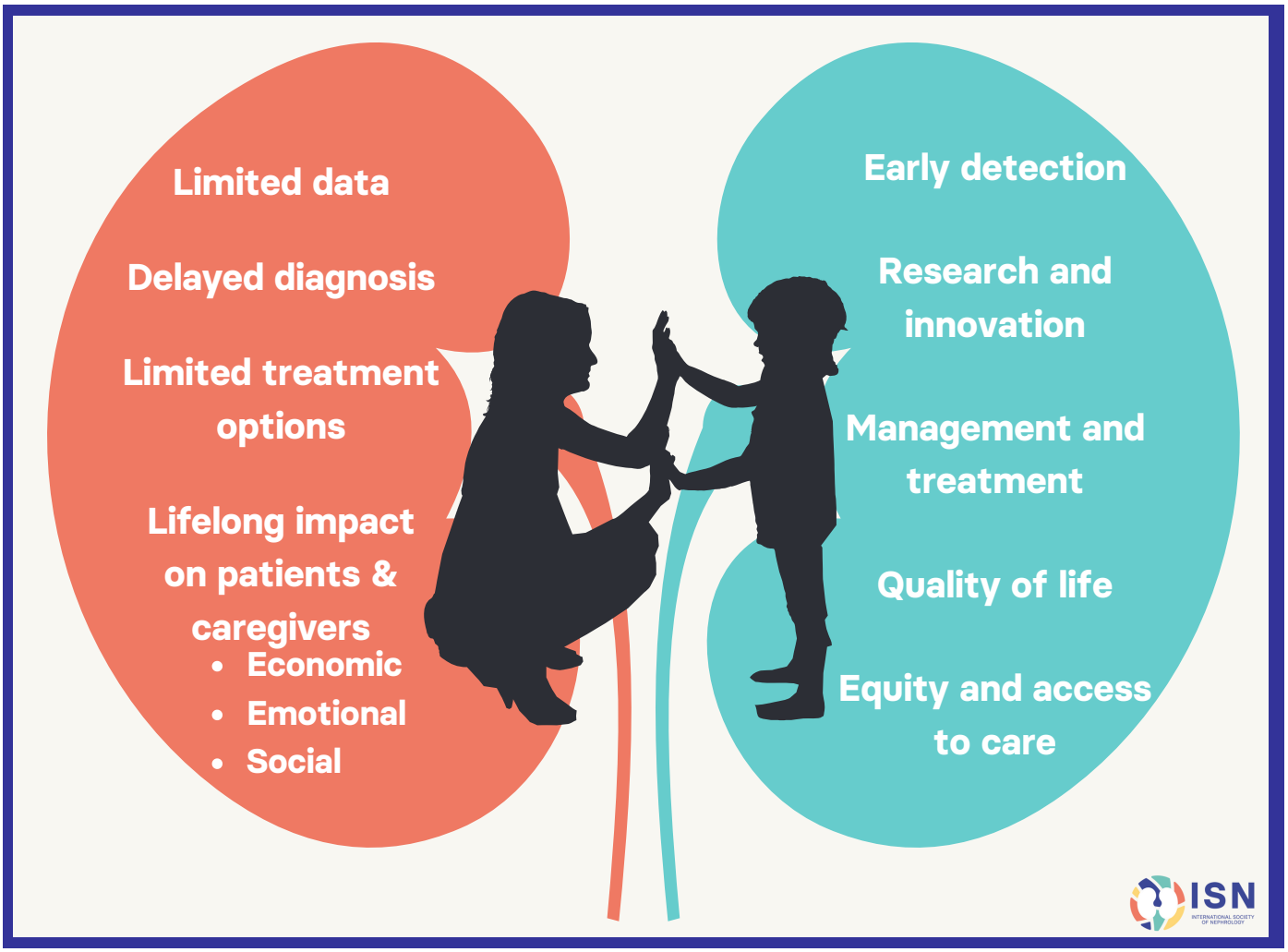
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THE SOLUTION

Policy recommendations to improve outcomes for individuals with RKDs and their families

1. EARLY DETECTION	• Strengthen early detection. • Enhance diagnostic infrastructure.
2. RESEARCH & INNOVATION	• Invest in etiological and risk factor research. • Provide financial and technical support on development of new therapies.
3. MANAGEMENT & TREATMENT	• Universal Health Coverage (UHC). • Comprehensive integrated care models.
4. QUALITY OF LIFE	• Socioeconomic determinants. • Psychosocial support.
5. EQUITY IN ACCESS TO HEALTHCARE	• Reduce disparities. • Data collection. • Enhance multisectoral partnerships.

For additional policy information, see: https://www.theisn.org/RKDPolicy



References
International Society of Nephrology. Manifesto. International Society of Nephrology, n.d., www.theisn.org/about-isn/mission-vision-values/manifesto/. Accessed 9 May 2025.
World Health Organization. Rare Diseases: A Global Health Priority for Equity and Inclusion. EB156/CONF.2, World Health Organization, 2025, https://apps.who.int/gb/ebwha/pdf_files/EB156/B156_CONF2-en.pdf.

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