

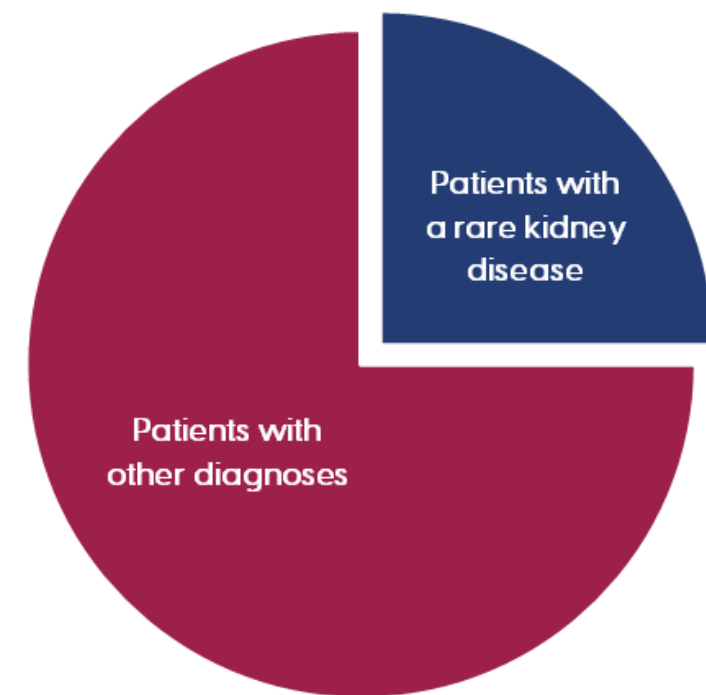
The strengths and challenges of creating a large-scale, real-world data rare disease registry

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Background

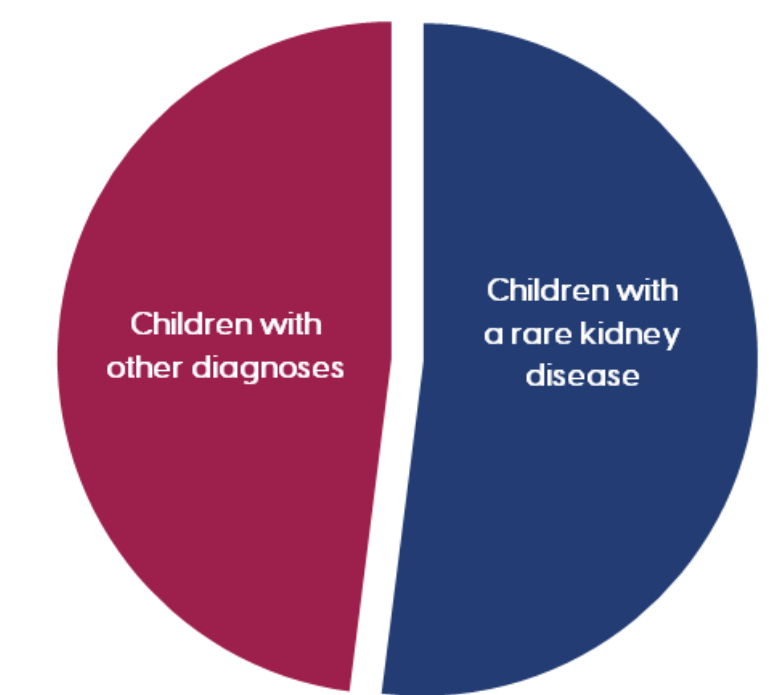
Patients with end stage kidney disease



People with rare kidney diseases are
28 times more likely to face kidney failure

than those in the general population with chronic kidney disease

Children on kidney replacement therapy



Despite representing just under 10% of the chronic kidney disease population, people with rare kidney diseases comprise 25% of patients with end-stage kidney disease, and 10% of adults/>50% of children on kidney replacement therapy.

While people with rare diseases are more likely to face kidney failure, they are less likely to die before needing dialysis or transplant.

Understanding of rare diseases is hampered by small patient numbers. Nephrologists may only see rare diseases a handful of times in their career.

Established in 2010, RaDaR collects data on patients with rare kidney diseases across the UK, creating an essential resource to further knowledge and understanding of rare kidney diseases.

With **>40 million** individual results & observational records
and **>36,000** patients

RaDaR is currently the **largest rare kidney disease registry** in the world

Methods and Outputs

Methods:

- Patients are individually consented. Their records are pseudonymised.
- Data is collected retrospectively and prospectively and stored in a secure database with a web front-end.
- Data sources include: hospital records, patients, automatic transfers from renal IT systems, batch uploads, and other organisations and registries such as the UK Renal Registry and Genomics England.
- Metadata is publicly available however data analyses are performed in-house. Only aggregate data is released. Small numbers are suppressed.

(Some of) RaDaR's outputs:

- 34 academic papers reference RaDaR directly.
- The Rare Disease Groups associated with RaDaR have created new hubs and networks, improved patient care and introduced novel therapies.
- RaDaR has identified eligible patients for several clinical trials.
- RaDaR ran its first patient surveys in 2024, achieving response rates of just under 25%.

Strengths and Challenges

	Strengths	Challenges	Mitigations
Rare disease data	Facilitates research. Supports drug development/regulatory decisions by providing enough patients for studies.	Low prevalence, wide geographical spread, limited funding. Low numbers introduce a re-identification risk.	Central registry financially sustained by the UKKA (over £1M invested to date), and other collaborations. In-house analyses.
Long-term, real-world data	More generalisable analyses. Disease progression/treatment impacts tracked over time.	Incomplete, inaccurate and complex data makes interpretation/analysis difficult.	A team of expert staff enrich, validate, clean and analyse data. Automatic data feed for 75% of patients.
Consented NHS cohort	UK-wide coverage. Patients can be contacted with research opportunities.	NHS staff capacity. Maintaining consent and approvals. Transition child -> adult.	Registered on the NIHR Portfolio. Core operational team + IG expert.

Where next for RaDaR?

Looking ahead to the next 15 years, RaDaR is:

- collaborating with Genomics England and aims to carry out Whole Genome Sequencing of the entire registry;
- a key data partner in the newly formed LifeArc Translational Centre for Rare Kidney Disease; and
- developing systems and processes to make its data clinical trial ready.

RaDaR was set up with initial significant grants from Kidney Research UK and Kidney Care UK and is now developed, maintained and supported by the UK Kidney Association. An annual charity grant ensures RaDaR remains on the NIHR portfolio.