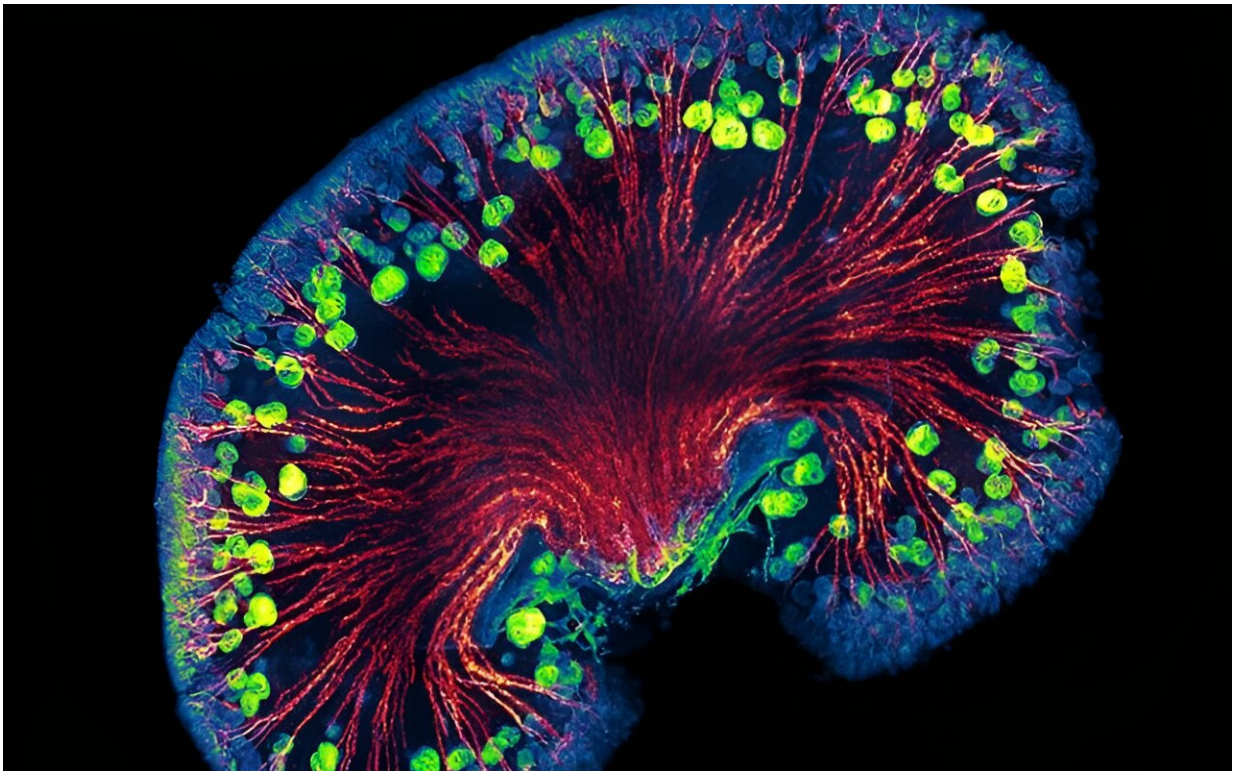


Treatments for rare diseases are needed to beat kidney failure, say researchers

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Credit: David Long, Claire Walsh and Danyal Jafree/UCL

Focusing on rare conditions could significantly reduce the burden of kidney disease on both patients and the NHS, according to a major new study led by UCL and the UK Kidney Association.

The study, published in [The Lancet](#) to mark World Kidney Day, draws on the largest rare kidney disease dataset ever created. It found that patients with rare kidney diseases are 28 times more likely to experience [kidney failure](#) than those with chronic kidney disease (CKD), but are less than half as likely to die before kidney failure treatment is needed.

The results suggest that additional focus on treating rare kidney diseases, many of which have new therapies in development or already available, could disproportionately alleviate the overall demand for expensive and hazardous dialysis and kidney transplantation treatments. The authors hope that the research will reframe perceptions of chronic kidney disease, rare diseases and kidney failure among patients, caregivers, regulators, health care providers, and researchers.

Chronic kidney disease (CKD) ranges from mild loss of kidney function to kidney failure with around 6% of the general population having mild to moderate CKD—most often caused by poorly controlled blood pressure or diabetes.

Rare kidney diseases are a group of conditions that each affect fewer than one in 2,000 people. Though each disease might be rare on its own, collectively their impact is significant. Rare kidney diseases account for 5–10% of people with CKD, but they constitute over a quarter of those receiving dialysis or with a kidney transplant globally.

To find out what happens to people with these difficult-to-study disorders, the UK's National Registry of Rare Kidney Diseases (RaDaR) was set up in 2010. It continues to be developed and run by the UK Kidney Association and now includes over 25,000 patients with rare kidney diseases recruited from 108 UK hospitals.

In this study, researchers from UCL and the UK Kidney Association charted the progression and outcomes in 28 rare kidney diseases by

comparing data from 27,285 RaDaR patients with data from 2.81 million CKD patients in the general population.

They found that over five years, rare kidney disease patients were 28 times more likely to experience kidney failure compared to CKD patients, yet their overall risk of death was less than half.

Professor Danny Gale, senior author of the study from UCL Division of Medicine and director of RaDaR, said, "This study underscores the importance of recognizing the pivotal role rare kidney diseases play in the overall burden of kidney failure.

"Our results show that rare diseases can progress from mild kidney damage to kidney failure so rapidly that, despite being rare in the population, they make a major contribution to the overall burden of kidney failure. This means that therapies effective in these diseases are likely to have a disproportionately beneficial impact on the overall demand for life-sustaining dialysis and kidney transplantation.

"I hope that this will be a call to arms to show how important rare kidney diseases are and the many potential benefits of focusing on these conditions. Treatments for many of these diseases are either available or in development, so I think we now have a golden opportunity to substantially reduce the burden, both for patients and the NHS, of kidney failure."

The authors say that the results are likely to reframe perceptions of [chronic kidney disease](#), [rare diseases](#) and kidney failure among regulators, health care providers and researchers.

Dr. Katie Wong, first author of the study from UCL Division of Medicine and RaDaR clinical research fellow at the UK Renal Registry, said, "In general, rare kidney disease patients are much younger than

those with CKD and are much less likely to die from related conditions such as cardiovascular disease.

"We know that they can live a long life with a successful [kidney transplant](#), but there's also an opportunity to prevent kidney failure in the first place with targeted treatment. I hope that the robust, large-scale data for each of the 28 diseases covered by RaDaR will inform trial design and make developing new treatments a less uncertain endeavor for drug companies."

As well as the difference new treatments would make to patients' lives, reducing the burden of kidney failure could bring significant cost and resource savings. Kidney failure alone accounts for around 3% of the NHS's budget, with dialysis costing £30–40,000 per person each year. Currently, around 70,000 people receive kidney replacement therapy in the UK, with around 45% on dialysis and 55% with functioning kidney transplants.

Ron Cullen, Chief Executive of the UK Kidney Association, said, "Since its inception some 13 years ago, the National Registry of Rare Kidney Diseases (RaDaR) has been a key strategic development led by the UK Kidney Association and the renal community. It is now the largest rare kidney disease registry in the world and its continuous growth and evolution make it an invaluable resource.

"With a strengthening connection between the medical community and patients with rare kidney diseases, RaDaR offers insights that are crucial for shaping future research and clinical discoveries. It is wonderful to see all the hard work of so many individuals come to fruition in a paper that could have significant patient benefit."

Elaine Davies, Director of Research Operations at Kidney Research UK said, "As well as highlighting the importance of finding treatments for

rare kidney diseases, these new results will allow patients and doctors to make more informed decisions about their care and will be invaluable for the planning of future clinical trials. RaDaR is a fantastic resource that has provided and will continue to offer vital insights into rare kidney diseases, and we are proud to support it."

More information: Katie Wong et al, Effects of rare kidney diseases on kidney failure: a longitudinal analysis of the UK National Registry of Rare Kidney Diseases (RaDaR) cohort, *The Lancet* (2024). [DOI: 10.1016/S0140-6736\(23\)02843-X](https://doi.org/10.1016/S0140-6736(23)02843-X)

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