Study confirms pioneering drug highly effective for rare kidney disease

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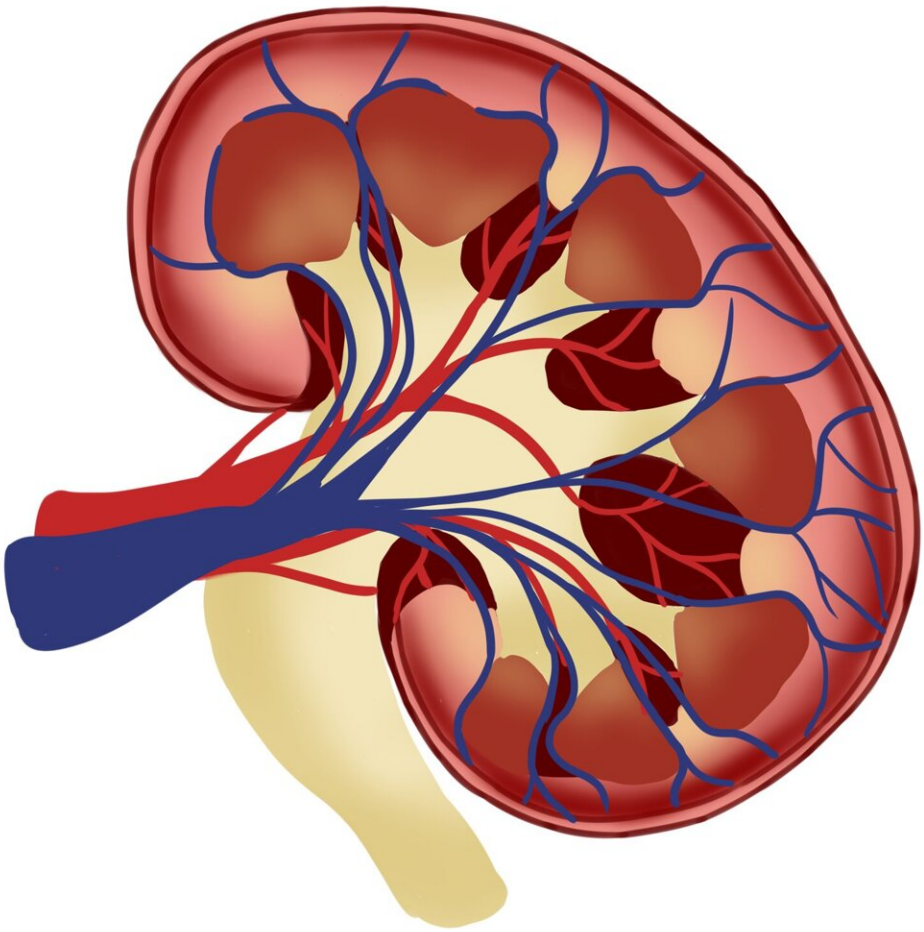

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A pioneering drug for a rare kidney disease prevents organ failure and significantly improves the outcome for patients, new research has confirmed.

Atypical hemolytic uraemic syndrome (aHUS) is a genetic life-threatening condition caused by a defect in the immune system which leads to kidney failure.

Newcastle University, UK, carried out clinical trials into the drug, eculizumab, which eventually led to the NHS approving the treatment for use in patients from 2015.

Now, a study by Newcastle experts, published in *Blood Journal*, revealed that eculizumab prevents 86% of patients going into kidney failure which highlights the importance of the use of the medication.

Life-changing treatment

Professor David Kavanagh, from Newcastle University, who led the study, said, "Our study confirms the effectiveness of eculizumab in preventing kidney failure for those with aHUS."

"It further strengthens the importance of patients receiving early treatment as it's lifesaving and helps significantly improve quality of life without the need for dialysis or a kidney transplant."

"Two decades ago, we began researching aHUS and it's wonderful to see that our efforts have benefitted those with the illness with the NHS approving the drug all those years ago."

In the largest study of its kind, more than 2,000 aHUS patients between 1995 and 2019 were analyzed.

A total of 244 were given eculizumab and responded very well to the treatment. However, a small proportion did not with 14% of patients still requiring long-term dialysis.

For some of those who did not benefit, a new genetic cause for aHUS was identified which allowed the drug to be stopped to avoid the potential side effects of ineffective treatment.

Dr. Vicky Brocklebank, from Newcastle University, said, "This study is a wonderful example of the benefits of close collaboration between patients, clinicians, researchers, and charities."

"Our pioneering research has allowed for genetically targeted treatments to improve patient outcomes and revolutionize the way the condition is managed."

It is estimated that 20–30 new patients are diagnosed with the condition each year.

NHS approval

Eculizumab costs £328,000 per year per adult patient and is given intravenously every two weeks. It was recommended for use on the NHS by the National Institute for Health and Clinical Excellence (NICE) eight years ago.

When the drug was approved, NHS England commissioned the National Renal Complement Therapeutics Centre, a collaboration between Newcastle University and Newcastle upon Tyne Hospitals NHS Foundation Trust, to run the highly specialized aHUS service.

Professor James Palmer, National Medical Director for Specialized Services at NHS England, said, "These important findings provide

renewed evidence that eculizumab is helping people to live longer without kidney failure, and could help enable doctors to identify those who are unlikely to benefit from the drug and could be spared the side effects."

"Collaborative research projects like this are helping to drive real progress for NHS patients with the support of a nationally commissioned service that provides both comprehensive clinical advice and a national patient registry, and it's fantastic to see the results, which will help save and improve patients' lives."

Further research by the Newcastle team will now focus on finding a cure for the small proportion of patients who do not respond to eculizumab.

Dr. Aisling McMahon, executive director of research and policy at Kidney Research UK, said, "This work by David, Vicky and the Newcastle team is a really important example of how, with collaboration between several partners, laboratory research can lead to clinical benefits for patients."

"We are delighted to have been a significant partner in this research and look forward to future projects supporting patients with aHUS."

More information: Vicky Brocklebank et al, Atypical haemolytic uraemic syndrome in the era of terminal complement inhibition- An observational cohort study, *Blood Journal* (2023). DOI: 10.1182/blood.2022018833

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