Patients with rare heart condition given lifeline through new drug

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People diagnosed with a life-threatening cardiac condition have been given new hope, thanks to a ground-breaking new drug that protects the heart developed by researchers from UCL and the Royal Free Hospital.

The trial results, published in the *New England Journal of Medicine*, show that patients with transthyretin amyloid cardiomyopathy (ATTR-CM) benefitted significantly when they took the drug Acoramidis.

ATTR-CM is the most commonly diagnosed form of amyloidosis. If left untreated, clumps of protein build up on the heart that prevent it from working properly, ultimately leading to heart failure and death within three to six years.

In the trial, 632 patients with ATTR-CM were given Acoramidis or a placebo for 30 months. The drug works by stabilizing the transthyretin protein to stop it falling apart, thereby preventing amyloid formation.

Results indicated that the drug had clear benefits in terms of mortality, morbidity and physical function compared to the placebo.

UCL's Professor Julian Gillmore, primary investigator of the global trial from UCL Division of Medicine and the Royal Free Hospital, said, "The results from the study are exciting for patients and researchers, as we now have a further effective treatment option for ATTR-CM. In every measure, patients receiving Acoramidis did significantly better than those on the placebo, from a reduction in mortality and cardiovascular hospitalizations to improved physical functioning as measured by a walking test. We are hoping that this drug will become widely available to patients with ATTR-CM by the end of 2024."

Professor Gillmore added, "ATTR-CM used to be thought of as a rare cause of heart failure but the number of people being diagnosed as well as those being diagnosed early in the disease course has dramatically increased, largely due to improvements in imaging which themselves were developed at the National Amyloidosis Center."'

"These are beautiful data because all of the treatments for amyloidosis have been shown to work better if patients are treated earlier in the course of the disease and we will finally have an effective treatment to offer UK patients with ATTR-CM. In the vast majority of patients receiving Acoramidis within the trial we saw disease improvement or

stability as a result of treatment with the drug."

Stephen Neil, 71, a former Royal Navy diver from Portsmouth who was diagnosed with ATTR-CM in 2019, was one of the people who took part in the global trial.

Stephen said, "I count myself lucky. Firstly, that I was diagnosed and referred so quickly to the National Amyloidosis Center (NAC) and then that I was deemed a suitable candidate to be put straight on the trial."

Although Stephen doesn't know whether he was given the drug or a placebo during his time on the 30-month trial, as it was a "double-blind" trial, once it had ended all participants were offered the opportunity to take the drug, as the benefits of Acoramidis had become clear.

Stephen added, "In 2023 my wife Pauline and I went to Italy and Venice and Devon and Cornwall. I can now look forward to a future with more cycling, further traveling and time to enjoy with my family. I want to thank the NAC team for their unwavering professionalism and support. Even through COVID they managed to keep my continuity of care going, ensuring I got the medication delivered, overcoming any obstacles. They were organized beyond belief."

More information: Julian D. Gillmore et al, Efficacy and Safety of Acoramidis in Transthyretin Amyloid Cardiomyopathy, *New England Journal of Medicine* (2024). DOI: 10.1056/NEJMoa2305434

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