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Cincinnati Children's Helps Lead Study Showing Penicillin Significantly Reduces Rheumatic Heart Disease Progression in Children

The findings are an important step in eradicating a disease that affects more than 40.5 million people globally and causes 306,000 deaths annually.

CINCINNATI – A widely available, affordable antibiotic treatment with penicillin significantly reduced the risk of underlying rheumatic heart disease progression in children and adolescents, according to the findings of a clinical trial led by [Andrea Beaton, MD](#), a pediatric cardiologist at Cincinnati Children's Hospital Medical Center. Beaton was the first author of an article, published Nov. 13, 2021, about the study in the [New England Journal of Medicine](#).

The study was conducted in the northern region of Uganda from July 2018 through October 2020 by [Cincinnati Children's](#), [Children's National Hospital](#), [Murdoch Children's Research Institute](#) and the [Uganda Heart Institute](#). It involved 818 children ages 5-17 years with latent rheumatic heart disease. The participants either received penicillin injections every four weeks for two years, or no treatment. All participants underwent echocardiography screening, where ultrasound waves produced images of the heart, at the start and end of the trial.

Beaton and her co-authors reported that just three participants (0.8%) who received penicillin experienced latent rheumatic heart disease progression after two years, compared to 33 (8.3%) who didn't receive the treatment.

Until this study, dubbed "Gwoko Adunu pa Lutino (GOAL), meaning "protect the heart of a child," it was unknown if antibiotics were effective at preventing the progression of latent rheumatic heart disease, according to Beaton, the principal investigator.

"This is the first contemporary randomized controlled trial in rheumatic heart disease. The results are incredibly important on their own, but also demonstrate that high-quality clinical trials are feasible to address this neglected cardiovascular disease," Beaton said. "The GOAL trial is a stunning example of global collaboration, including investigators from six continents¹, working together to find innovative solutions to reduce the global burden of rheumatic heart disease."

Rheumatic heart disease is a chronic valvular heart disease caused by rheumatic fever, which develops after an untreated case of strep throat. More than 40.5 million people globally are estimated to be living with rheumatic heart disease, and approximately 306,000 deaths are attributed to it annually.

Rheumatic fever is diagnosed infrequently in low-resource settings. About 85% of patients are diagnosed only when it is severe and irreversible, medications are ineffective, and surgical intervention is expensive and/or unavailable. Reasons for that include poor health-seeking behavior, varied presentations, and overlap with other common illnesses such as malaria and viral infections. In these areas, most patients

¹ Contributing to the study were researchers from the University of Cincinnati College of Medicine, Makerere University; Uganda, Children's National Hospital; Washington, The Royal Children's Hospital, Telethon Kids Institute, Virginia Tech Carilion School of Medicine, Université de Paris, Instituto Nacional de Saude; Mozambique, Universidade Federal de Minas Gerais; Brazil, Emory University School of Medicine; Atlanta, Starship Children's Hospital; Auckland, Geisel School of Medicine; New Hampshire, Red Cross Children's Hospital; South Africa and All India Institute of Medical Sciences.

receive a diagnosis of rheumatic heart disease when it is advanced, and complications have developed. Late diagnosis of this disease is associated with high mortality at a young age, in part owing to the missed opportunity to benefit from prophylaxis.

“Our study found a cheap and easily available penicillin can prevent progression of latent rheumatic heart disease into more severe, irreversible valve damage that is commonly seen in our hospitals with little or no access to valve surgery,” said [Emmy Okello, MD](#), chief of cardiology at the Uganda Heart Institute and co-principal investigator of the GOAL trial.

Beaton said these results don’t immediately translate into clinical practice because there is still an enormous amount of infrastructure development needed to use echocardiogram screenings as a public health tool. According to the paper, a number of barriers to achieving high adherence in a real-world setting exist, including a lack of retention in care, a lack of availability of medication, a lack of access to transportation, social stigma, pain associated with intramuscular injection, and a limited understanding of the disease.

Though there is still work to do, these findings are an important step in eradicating a disease that disproportionately affects children in low-resource settings around the world and here in the United States. Plans are already in place for continued research to further these efforts.

“We have a funded follow-up study called ‘GOAL Post’ from the [Trasher Research Fund](#) that will follow these kids for another five years,” Beaton said. “That study will look at the durability of prophylaxis in preventing adverse outcomes as well as determining whether it is safe for children to stop antibiotic prophylaxis once their heart returns to normal. We’re also submitting a study to the [National Heart, Lung, and Blood Institute](#) looking at oral versus intramuscular penicillin, which may be a lot more practical in low-resource settings.”

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