

Using blood tests to identify new drug targets to prevent the development of hypertrophic cardiomyopathy in child gene carriers



Lead researcher	Professor Juan Pablo Kaski
Location	University College London and Great Ormond Street Hospital
Start date	February 2026
Duration	12 months
Cost	£24,919
Project code	C2503
Research priorities	3) Should treatment for cardiomyopathy be tailored to the individual, e.g. based on their specific gene variant, age or gender? 4) What triggers the start of cardiomyopathy (e.g. age, stress, pregnancy, other health conditions)? How do these triggers work and can they be blocked? 5) Are there treatments which can prevent cardiomyopathy developing in people at risk? Are there treatments to stop it getting worse in people with symptoms? 6) What are the biological mechanisms that change heart muscle cells in cardiomyopathy? Could this understanding lead to new treatments?

This research aims to understand how [hypertrophic cardiomyopathy](#) (HCM) starts and progresses in children who have a genetic change linked to the condition.

The researchers have already identified substances in blood and urine (known as biomarkers) that can distinguish children with HCM from those with healthy hearts and identify those at higher risk of dangerous heart rhythms. This study will build on that work by analysing information and stored blood and urine samples from children and teenagers with HCM cared for at Great Ormond Street Hospital over the last 25 years.

The team hope to identify the earliest features of HCM and how these progress over time to uncover the processes that drive the development of the condition. This will help identify new targets for treatments, including the possibility of using existing drugs in new ways (known as repurposing) to prevent or slow the onset of HCM.

Although this research is based on children and teenagers, the findings are likely to be relevant to the wider population of people with HCM.

Our vision is that everyone affected by cardiomyopathy should lead a long and fulfilling life

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