Introduction to cardiomyopathy research

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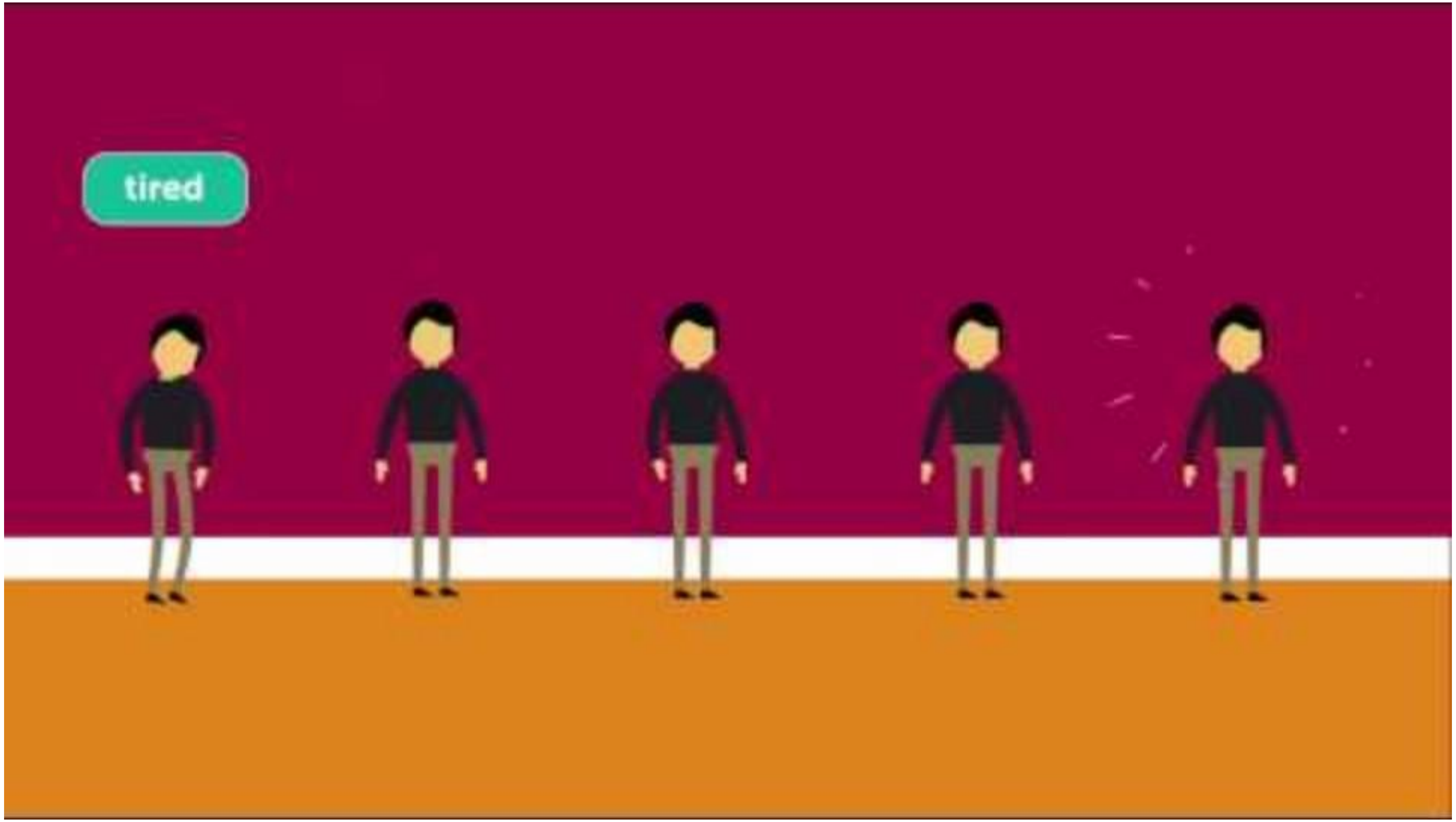
Cardiomyopathy UK
the heart muscle charity

Introduction to cardiomyopathy



About Cardiomyopathy UK

We’re here for everyone affected by cardiomyopathy

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

- Information
- Support
- Campaining
- Research

About me

CureHeart

Understanding clinical trials

Research is paving the way for new treatments for cardi

a potential treatment has been developed and tested i

step is finding out if it is safe and effective through clinic

Cardiomyopathy UK is part of the £30 million British Heart Foundation funded CureHeart project, which aims to develop the world's first cures for inherited cardiomyopathies. The international team of researchers hope that at least one new therapy will be ready for clinical trials towards the end of the project in 2027.

Clinical trials are carried out in a series of steps called phases (which often include a placebo or dummy treatment for comparison). Depending on the outcome, a potential new treatment may or may not progress to the next phase.

Phase 1

The new treatment is usually tested for the first time in a small number of people. The aim is to understand what happens to it in the body, learn about safety, identify side effects and find the best dose.

→ This is where therapies developed by the CureHeart team will start.

Phase 2

tment is tested in a larger

mine whether

Phase 3

The new treat

large number

effectiveness

compare it

→ The

trial

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Cardiomyopathy UK

the heart muscle charity

The latest in cardiomyopathy research

www.cardiomyopathy.org research@cardiomyopathy.org 01494 7912

Dear Team Cardio,

Thank you for your continued interest in cardiomyopathy research. And welcome if you're new to the research network.

As part of the network, we keep you updated on the latest research ne let you know about opportunities to take part in and help shape cardio research.

Research involvement opportunities

We want to make sure the voices of people affected by cardio are at the heart of research into the condition. This could inv commenting on research proposals or being part of a focus

BHF CRC Clinical Research Collaborative

Join the BHF CRC national cardiovascular clinical researc

The British Heart Foundation Clinical Research Collaborative

For cardiomyopathy researchers

Our support for researchers

We support high-quality research that delivers real benefits to people affected cardiomyopathy, with a focus on our research priorities.

How we can help

- Recruit participants to your study
- Patient and Public Involvement (PPI) supp
- Request a letter of support
- Partner with us

Contact us at research@cardiomyopathy.org for more information.

Funding opportunities

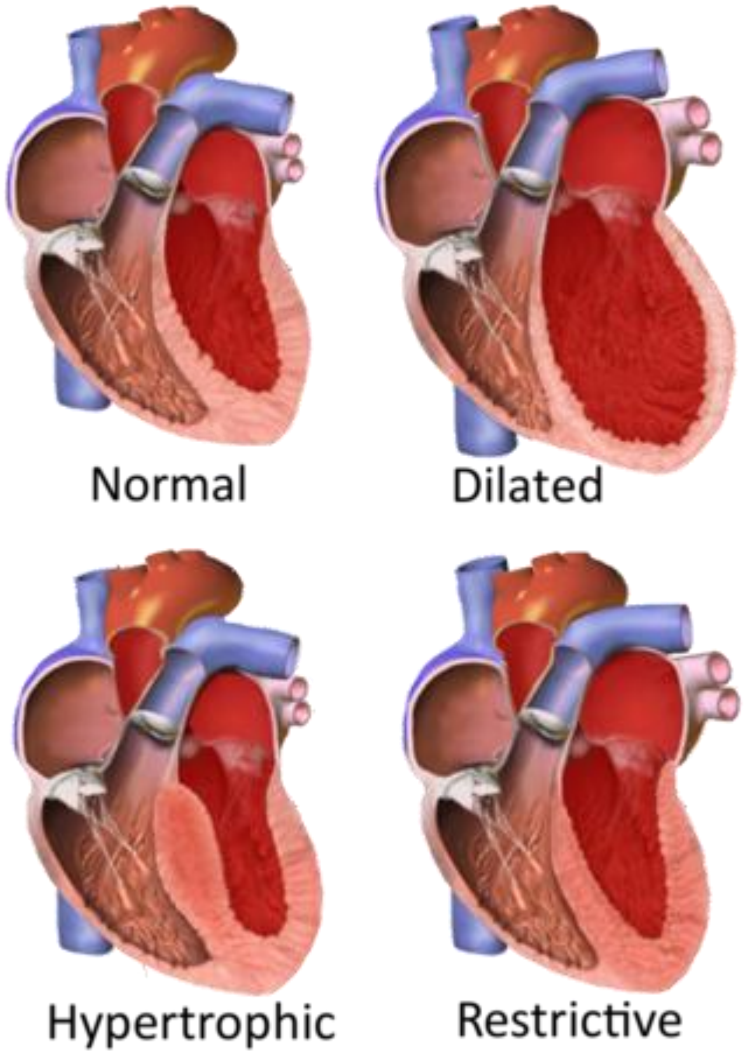
Catalyst grants

We will be launching our catalyst grant scheme in 2025. These awards will directly address our research priorities with the aim of attracting further investment from other sources. Our goal is to fund groundbreaking research that will advance our understanding of cardiomyopathy, improve diagnosis and treatment, and ultimately make life better for people affected by the condition.

NIHR doctoral fellowship

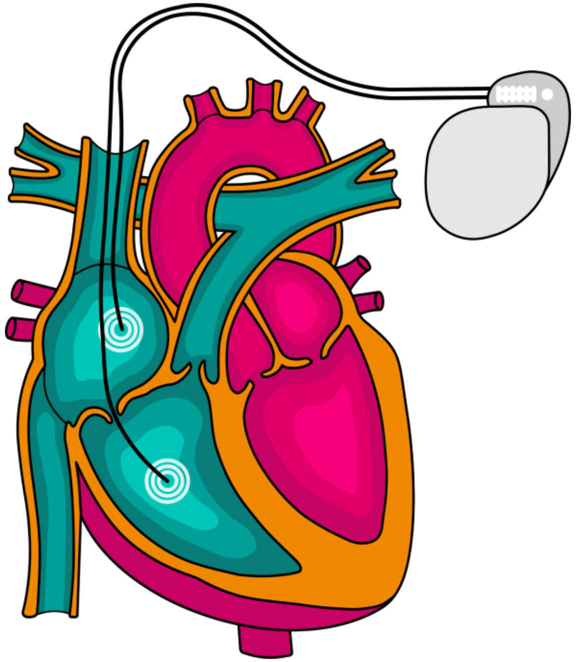
What is cardiomyopathy?

- A group of conditions that affect the structure of the heart and reduce its ability to pump blood around the body
- Around 1 in 250 people in the UK are affected
- Often inherited
- Effective treatments but no cure



Npatchett at English Wikipedia, CC BY-SA 3.0 <https://creativecommons.org/licenses/by-sa/3.0>, via Wikimedia Commons

Current treatments

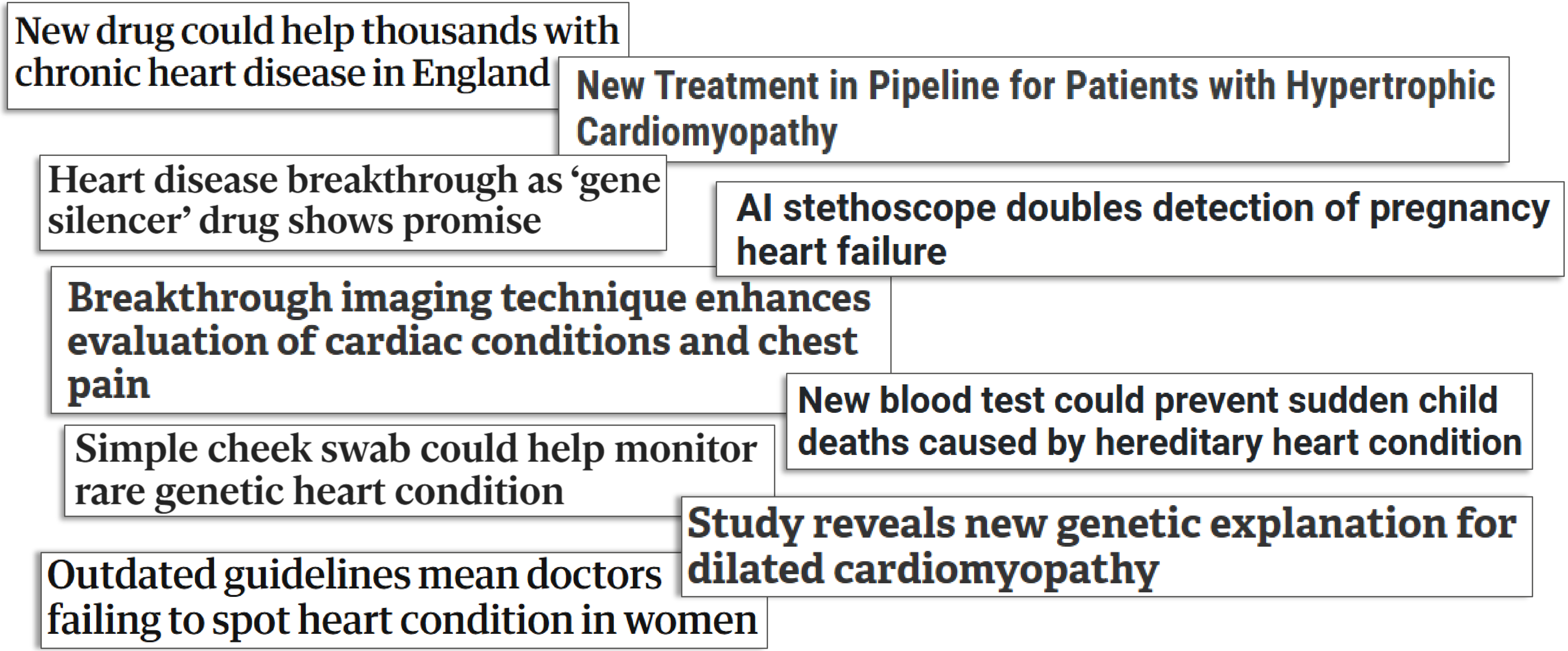


- ACE inhibitors
- Beta blockers
- Anti-arrhythmics
- Blood thinners

- Pacemakers
- Implantable Cardioverter Defibrillators (ICDs)

- Alcohol septal ablation
- Septal myectomy
- Transplant

It’s an exciting time for research



New drug could help thousands with chronic heart disease in England

New Treatment in Pipeline for Patients with Hypertrophic Cardiomyopathy

Heart disease breakthrough as ‘gene silencer’ drug shows promise

AI stethoscope doubles detection of pregnancy heart failure

Breakthrough imaging technique enhances evaluation of cardiac conditions and chest pain

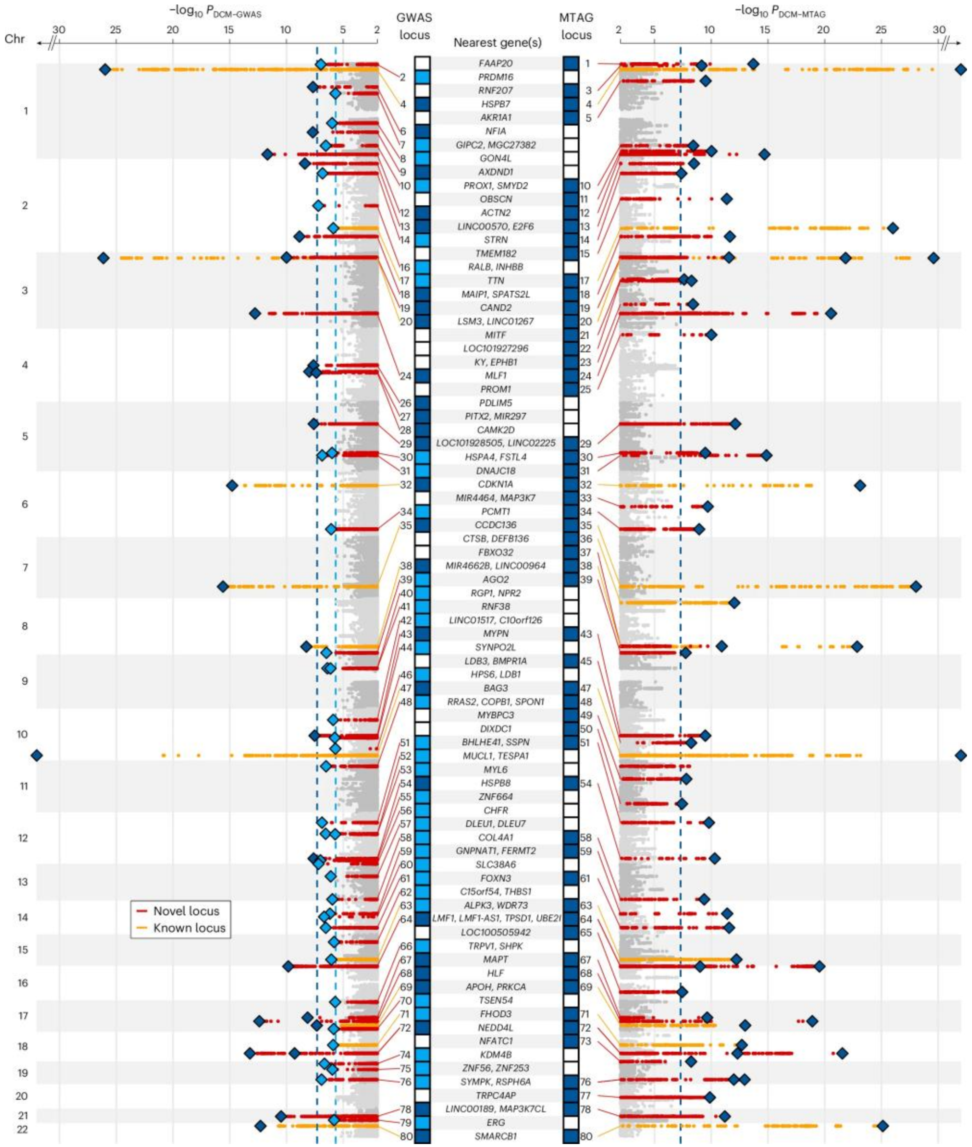
New blood test could prevent sudden child deaths caused by hereditary heart condition

Simple cheek swab could help monitor rare genetic heart condition

Study reveals new genetic explanation for dilated cardiomyopathy

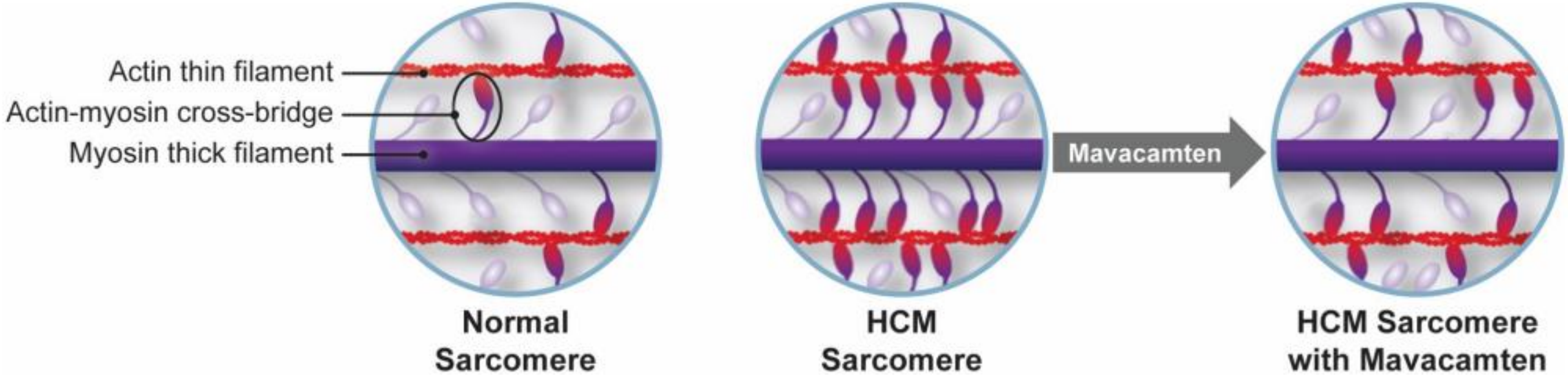
Outdated guidelines mean doctors failing to spot heart condition in women

Understanding genetics



Zheng, S.L., Henry, A., Cannie, D. *et al.* Genome-wide association analysis provides insights into the molecular etiology of dilated cardiomyopathy. *Nat Genet* **56**, 2646–2658 (2024).

The first cardiomyopathy-specific drug

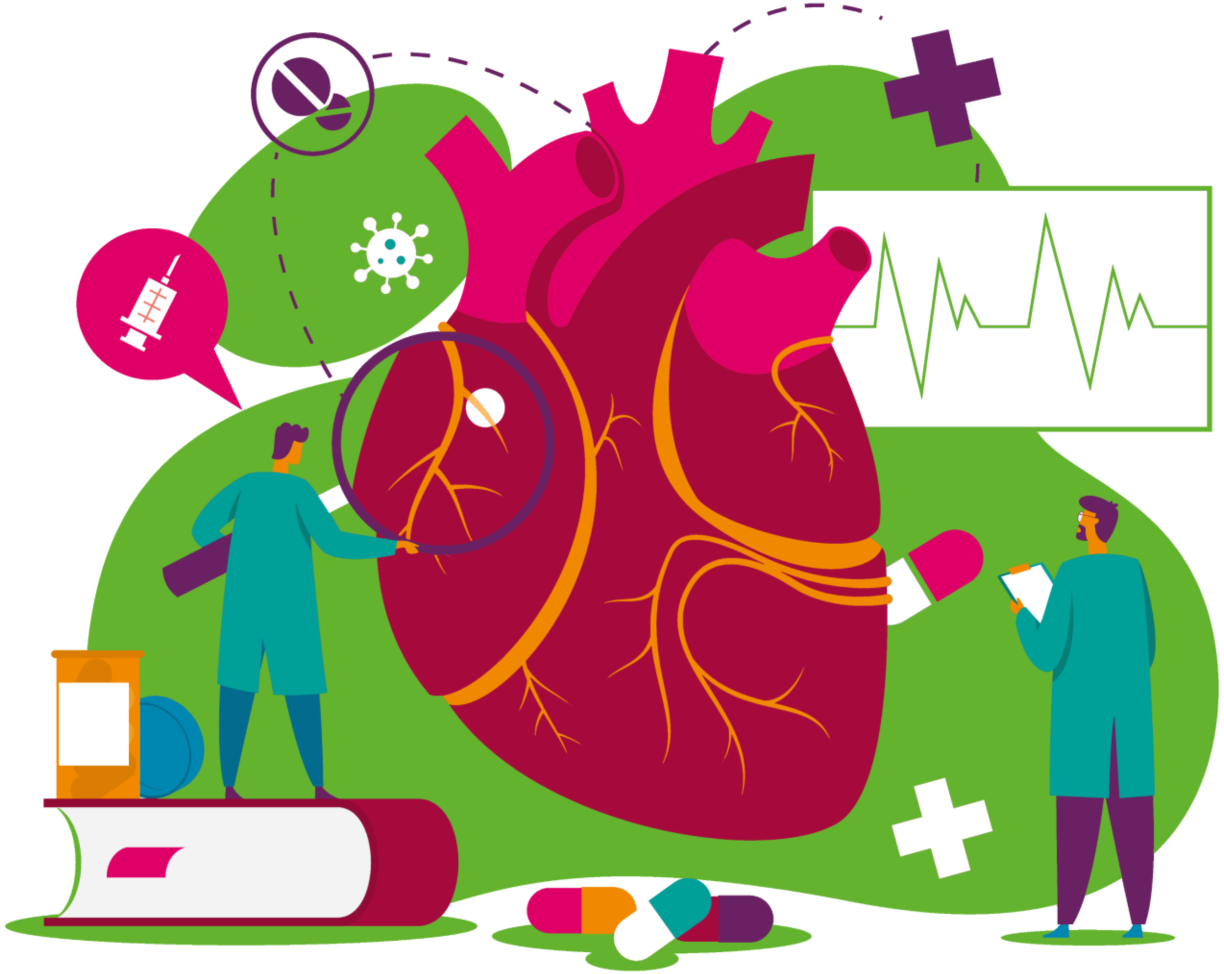


https://www.nice.org.uk/guidance/ta913/documents/1

CureHeart

Our research journey

- Supporting research
- Partnership projects
- Research priorities
- Research network
- Funding research



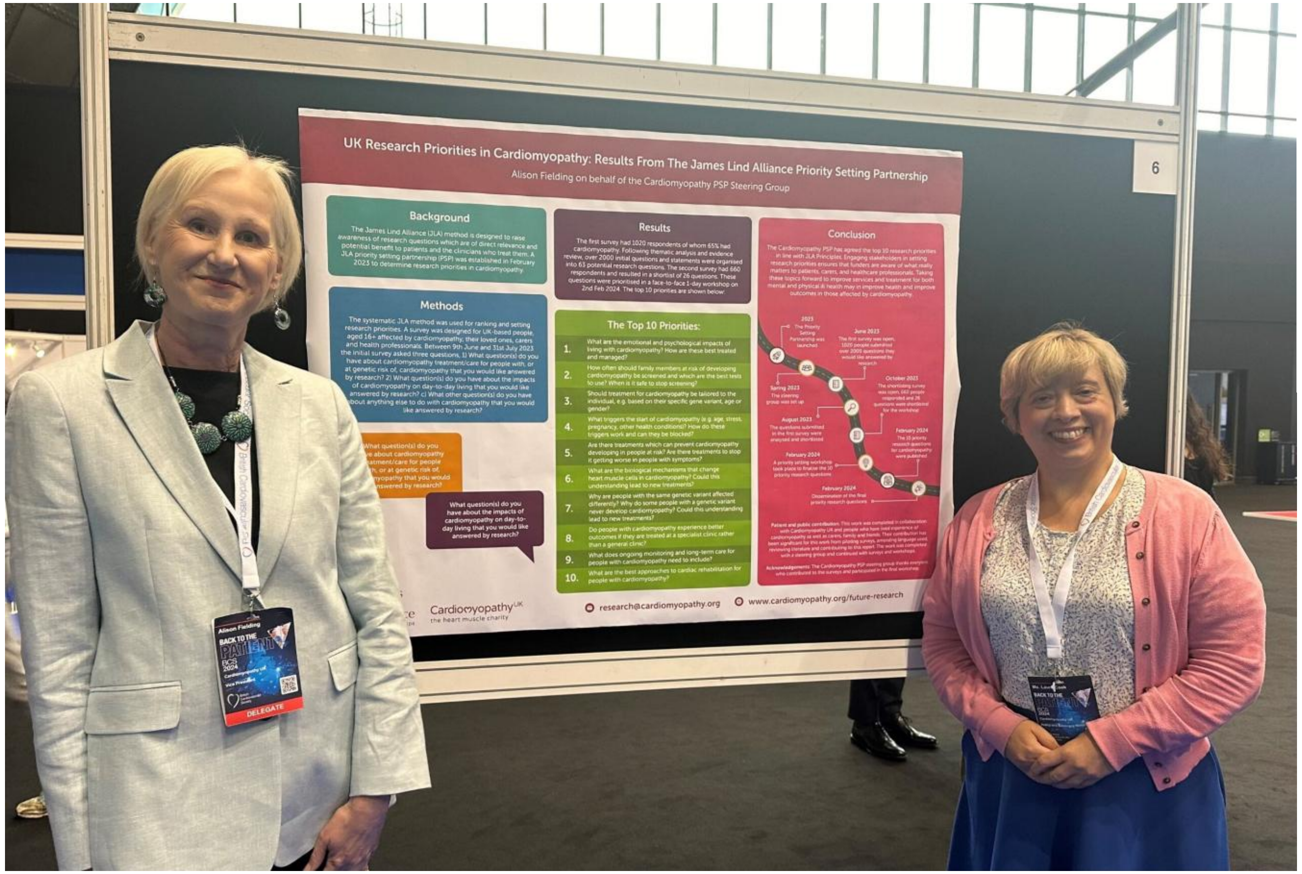
Partnership projects

CureHeart

CRUCIAL

SMASH
HCM

Priority setting partnership



UK Research Priorities in Cardiomyopathy: Results From The James Lind Alliance Priority Setting Partnership

Alison Fielding on behalf of the Cardiomyopathy PSP Steering Group

Background

The James Lind Alliance (JLA) method is designed to raise awareness of research questions which are of direct relevance and potential benefit to patients and the clinicians who treat them. A JLA priority setting partnership (PSP) was established in February 2023 to determine research priorities in cardiomyopathy.

Methods

The systematic JLA method was used for ranking and setting research priorities. A survey was designed for UK-based people, aged 16+ affected by cardiomyopathy, their loved ones, carers and health professionals. Between 9th June and 31st July 2023 the initial survey asked three questions, 1) What question(s) do you have about cardiomyopathy treatment/care for people with, or at genetic risk of, cardiomyopathy that you would like answered by research? 2) What question(s) do you have about the impacts of cardiomyopathy on day-to-day living that you would like answered by research? c) What other question(s) do you have about anything else to do with cardiomyopathy that you would like answered by research?

What question(s) do you
...e about cardiomyopathy
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...nswered by research?

What question(s) do you have about the impacts of cardiomyopathy on day-to-day living that you would like answered by research?

Results

The first survey had 1020 respondents of whom 65% had cardiomyopathy. Following thematic analysis and evidence review, over 2000 initial questions and statements were organised into 63 potential research questions. The second survey had 660 respondents and resulted in a shortlist of 26 questions. These questions were prioritised in a face-to-face 1-day workshop on 2nd Feb 2024. The top 10 priorities are shown below:

The Top 10 Priorities:

1. What are the emotional and psychological impacts of living with cardiomyopathy? How are these best treated and managed?
2. How often should family members at risk of developing cardiomyopathy be screened and which are the best tests to use? When is it safe to stop screening?
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?
7. Why are people with the same genetic variant affected differently? Why do some people with a genetic variant never develop cardiomyopathy? Could this understanding lead to new treatments?
8. Do people with cardiomyopathy experience better outcomes if they are treated at a specialist clinic rather than a general clinic?
9. What does ongoing monitoring and long-term care for people with cardiomyopathy need to include?
10. What are the best approaches to cardiac rehabilitation for people with cardiomyopathy?

Conclusion

The Cardiomyopathy PSP has agreed the top 10 research priorities in line with JLA Principles. Engaging stakeholders in setting research priorities ensures that funders are aware of what really matters to patients, carers, and healthcare professionals. Taking these topics forward to improve services and treatment for both mental and physical ill health may in improve health and improve outcomes in those affected by cardiomyopathy.

2023
The Priority Setting Partnership was launched

Spring 2023
The steering group was set up

June 2023
The first survey was open, 1020 people submitted over 2000 questions they would like answered by research

August 2023
The questions submitted in the first survey were analysed and shortlisted

October 2023
The shortlisting survey was open, 660 people responded and 26 questions were shortlisted for the workshop

February 2024
A priority setting workshop took place to finalise the 10 priority research questions

February 2024
The 10 priority research questions for cardiomyopathy were published

February 2024
Dissemination of the final priority research questions

Patient and public contribution: This work was completed in collaboration with Cardiomyopathy UK and people who have lived experience of cardiomyopathy as well as carers, family and friends. Their contribution has been significant for this work from piloting surveys, amending language used, reviewing literature and contributing to this report. The work was completed with a steering group and continued with surveys and workshops.

Acknowledgements: The Cardiomyopathy PSP steering group thanks everyone who contributed to the surveys and participated in the final workshop.

Cardiomyopathy UK
the heart muscle charity

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www.cardiomyopathy.org/future-research

James
Lind
Alliance
Priority Setting Partnerships

cardiomyopathy.org/future-research

Top 10 priorities

1) What are the **emotional and psychological impacts** of living with cardiomyopathy? How are these best treated and managed?
2) How often should family members at risk of developing cardiomyopathy be screened and which are the best tests to use? When is it safe to stop **screening**?
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?
7) **Why are people with the same genetic variant affected differently**? Why do some people with a genetic variant never develop cardiomyopathy? Could this understanding lead to new treatments?
8) Do people with cardiomyopathy experience better outcomes if they are treated at a **specialist clinic** rather than a general clinic?
9) What does ongoing **monitoring and long-term care** for people with cardiomyopathy need to include?
10) What are the best approaches to **cardiac rehabilitation** for people with cardiomyopathy?

cardiomyopathy.org/future-research

Research network

Help shape research

We want to make sure the voices of people affected by cardiomyopathy are at the heart of research. This could involve commenting on research proposals or being part of a focus group.

Take part in research

Find out about opportunities to participate in cardiomyopathy research, from questionnaires that can be done at home to testing new treatments.

Get the latest news

Get the latest news and updates about cardiomyopathy research delivered directly to your inbox.

> "...for research to be as effective as possible... We need to ensure that people with lived experience are fully involved in the research process from the outset"
>
> **NHS England**

Join our research network

cardiomyopathy.org/form/research-network-subscribe

Other ways to get involved

the**heart**hive.org

NIHR | Be Part of Research

bepartofresearch.nihr.ac.uk

BHF CRC

Clinical Research Collaborative

researchdatabase.bhfcrc.org

Funding research

- Partnering on a doctoral fellowship with the NIHR
- Our first research grant round is coming soon!!

NIHR | National Institute for Health and Care Research

amrc
ASSOCIATION OF MEDICAL RESEARCH CHARITIES

Thank you!
Any questions?

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the heart muscle charity