Cardiomyopathy UK
the heart muscle charity

James Lind Alliance
Priority Setting Partnerships

Cardiomyopathy priority setting partnership

Future research priorities for cardiomyopathy

Technical report

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1. Introduction

1.1 What is a priority setting partnership (PSP)?

A PSP is a process that brings together people with lived experience of a health condition and healthcare professionals, working in that field, to decide on research priorities into that condition. The views of both groups are given equal value.

The process of reaching the final priorities is consultative. It often involves surveys, and it always ends with a priority setting workshop, in which a consensus is reached through discussions.

1.2 Why a cardiomyopathy priority setting partnership (PSP)?

Cardiomyopathy UK decided to partner with the James Lind Alliance on a cardiomyopathy PSP as a robust and reputable process of determining research priorities. The James Lind Alliance is a non-profit making initiative bringing patients, carers and clinicians together in JLA priority setting partnerships. There has been an increase in cardiomyopathy research over recent years, however, this research did not match the questions being asked by people living with cardiomyopathy. This led to a power imbalance and frustrations from people affected by the disease not getting answers to the questions that they had.

2. Methodology

Step 1: Project Set Up

The project was set up in early 2023, including the Steering Group and other project roles. These roles are (see the last section of the report for the people assigned to each role):

- PSP Lead
- James Lind Alliance Adviser
- A Steering Group
- PSP Coordinator
- Information Specialist
- Facilitators
- Partners

PSP Lead:

The PSP lead has overall responsibility for delivering the PSP. They present solutions or options to the Steering Group to decide on the best method to take the project forward.

James Lind Alliance (JLA) Adviser:

James Lind Alliance (JLA) Advisers support and guide the PSP as neutral facilitators to ensure the process is followed in a fair and transparent way. They ensure equal input from the perspectives of patients, carers and clinicians.

Steering Group:

The steering group is made up of people affected by cardiomyopathy and healthcare professionals. Members of the steering group bring with them knowledge of cardiomyopathy from either lived or professional experience. The steering group oversees the PSP and is responsible for making key decisions around the PSP. The responsibilities include:

- Setting the project terms of reference.
- Deciding on in and out of scope issues to include in the project.
- Promoting the PSP.
- Supporting with survey design.
- Publicising surveys to their networks.
- Overseeing the analysis and interpretation of survey data.
- Publicising the priority questions to the research community.

PSP Coordinator:

The PSP Coordinator is responsible for the administration of the PSP. They set up the steering group meetings, prepare the agenda and circulate papers, draft minutes and follow up on actions. They also plan the priority setting workshop, such as booking the venue and collating the needs of participants.

Information specialist:

The Information Specialist supports the Steering Group to analyse and summarise the submitted questions from the first survey. They also conduct an evidence check of existing research against the summary questions to ensure that only topics that are under researched are taken to the next stage of the process.

Facilitators:

Other JLA Advisers attend the final priority setting workshop to act as facilitators in the discussion groups.

Partners:

Partner organisations support the promotion of surveys and dissemination of the final research priorities.

Step 2: Gathering Uncertainties

The initial survey invited the cardiomyopathy community to submit questions that they would like answered by research. The design of the first survey was tested with people with cardiomyopathy, not involved in the project. As a result of feedback, the instructions were simplified, some terminology clarified and examples of potential question topics were included.

The survey was open for 6 weeks (from the end of June 2023 to early August 2023) and was available online through SurveyMonkey. Paper copes of the survey were also sent to Cardiomyopathy UK's peer support groups which were scheduled to meet face-to-face during the survey period; peer support group leaders were asked to encourage members to complete the form during the meeting.

Concurrently, the PSP Lead contacted and built relationships with other organisations working on connected heart conditions (see the end of the report for a list of partner organisations). While this was a time-consuming task, the purpose of partnership building was twofold: it raised the profile of the cardiomyopathy PSP and the partners could promote the survey to their own networks and communities. Seven organisations publicly declared their partnership with the cardiomyopathy PSP.

1020 people responded to the survey, submitting just over 2000 questions.

Step 3: Evidence Checking

Members of the Steering Group worked with an information specialist to go through the submitted questions, decide which were in and out of scope, then summarise the questions (where similar questions were submitted using different wording), and placed under topic headings. The whole Steering Group agreed the final list of questions to go into the shortlisting survey, agreed they were an accurate summary and under the correct topic headings.

Step 4: Interim Survey

The subsequent list of questions, of which there were just over 60, went into a shortlisting survey. This survey was split into two parts: the first part was the full list and respondents could choose as many as they liked from the list; the second part carried forward the respondent's selection to a new list and they were asked to choose the top 10 questions which were most important to them. This was also conducted on SurveyMonkey, and issues with using this software is explained later in this report. There were 660 responses.

The questions were ranked into two lists - by people with lived experience and healthcare professionals. As fewer healthcare professionals than people with lived experience had responded, the answers were weighted accordingly.

The two lists were merged, and the Steering Group decided where the cut-off point should be to ensure a fair balance of priority questions from each group of people, but so there were not too many to take to the workshop.

It was decided to take a list of 26 questions to the priority setting workshop.

Step 5: The Priority Setting Workshop

There were 31 participants in the workshop, which was very well attended. The James Lind Alliance had never facilitated a workshop with so many participants. This obviously demonstrates the dedication of the cardiomyopathy community.

The workshop followed the process of the James Lind Alliance in terms of there being three facilitated discussion groups. Each participant had a chance to choose their priorities beforehand and present these to their discussion group. The priorities from each group were ranked and ranking combined throughout the course of the day to establish the final list.

In terms of diversity of the workshop: there was a good balance of people with lived experience and healthcare professionals (across disciplines); the gender ratio was well-balanced; ages varied, but there were no under 30s represented; there was a much higher proportion of people from white backgrounds than from black or Asian communities.

Timeline

- The cardiomyopathy future research priorities project was launched in early 2023 and the Steering Group set up in spring 2023.
- June 2023 to August 2023: First survey open. Respondents asked to submit potential questions that could be answered by research.
- August 2023 to October 2023: All questions analysed and summarised and decision made on which questions were in and out of scope.
- October 2023 to December 2023: Shortlisting survey open. Respondents selected the most questions important to them from a long list of questions.
- February 2024: Priority setting workshop to finalise the 10 priority research questions.
- February 2024: The ten priority research questions for cardiomyopathy published.

3. The 10 research 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?

Questions 11-26

11. Are there tests which can predict the risk of getting worse, heart failure and/or sudden death?
12. Can gene therapy be used to prevent cardiomyopathy developing in people at risk or to treat people with symptoms?
13. What is a safe and beneficial level of exercise for adults and children with a genetic risk of cardiomyopathy?
14. Which people with cardiomyopathy benefit most from an ICD (implantable cardioverter defibrillator)?
15. What is a safe and beneficial level of physical activity or exercise for people with cardiomyopathy?
16. What genetic and environmental factors influence the risk of getting worse, heart failure and/ or sudden death?
17. What are the best ways for people to monitor their symptoms at home and to know when to seek medical help?
18. What are the long-term side-effects of treatment? Can drugs with fewer side-effects be developed?
19. What happens to people with cardiomyopathy as they get older? How does the condition change over time?
20. Can drug treatment reverse changes to the heart to a point where people can safely stop their medication?
21. Can stem cells be used to repair or restore damaged heart muscle in people with cardiomyopathy?
22. How is cardiomyopathy linked to atrial fibrillation (an irregular and fast heartbeat)?
23. What are the best ways to treat heart failure in people with cardiomyopathy?
24. What causes fatigue in people with cardiomyopathy and how is it best treated and managed?
25. What lifestyle changes help avoid getting worse, and/or reduce their risk of heart failure and sudden death?
26. What are the best ways to treat and manage breathlessness in people with cardiomyopathy?

Why these top 10?

It is important to recognise that on another day with a different group of participants – or even the same participants with more time – the contributors could have produced a different ranking. It is equally important to remember that questions in the 11-26 list will not be forgotten and are still to be considered priorities for future research. The list of topics is also broad and some topics can be subdivided into more specific details.

Some of the priorities in the top ten or 11-26 may be unexpected. Therefore, it is useful to provide some context to the discussions at the priority setting workshop in which the ten priorities were decided.

Priority number one on psychological and emotional needs almost did not make it to the workshop, as it was ranked 23 in the second survey (i.e. outside of the top 20 at that stage - see above for the methodology). However, the Steering Group had noted that there were no questions on psychological support in the overall top 20 ranking and therefore decided to take the highest ranking question addressing this topic to the workshop. This was on the basis that mental health support is asked about regularly on the helpline and in peer support services. Not every participant at the workshop thought that this should be a research priority. However, many participants made the case for this topic in the workshop and brought others round to their perspective, resulting in it moving up and up the list to the number one position.

An observer stated, "it was moving to listen to the eloquence of patients and their families as they explained the impact of cardiomyopathy on mental health. The threat of a cardiac event including death, the change of health and economic status, the guilt and worry of possible family inheritance, relationship changes and living with constant uncertainty were all cited as having major daily impacts on their lives. Health professionals understood that people needed ways to manage these feelings and views on research priorities changed during the workshop."

There was much debate around the question on safe and beneficial levels of exercise. This was identified as a challenge in the Cardiomyopathy 2022 national survey and was ranked as the number 4 topic in the second survey – it was high for both healthcare professionals and people with cardiomyopathy. The reason it is not in the top ten priorities is that several workshop participants advocated strongly for exercise to be included under the general heading of cardiac rehabilitation. Therefore, a decision was made that cardiac rehabilitation would be an umbrella topic to include exercise.

The participants decided that some of the questions were related to heart failure more broadly rather than cardiomyopathy specifically. As a result, these questions (for example, fatigue and breathlessness) were ranked towards the bottom of the 26 priorities.

As another observer noted, the participants with lived experience were well enough to travel to the workshop as they had not reached a stage where their health would make it too difficult to travel. This is noteworthy as the priorities could be in a different order if there were people present whose health had progressed to a stage where the workshop was inaccessible to them.

4. Project statistics and learning

4.1 Key project statistics

- 7 partner organisations who promoted the surveys.
- 1020 individual responses to the initial survey.
- Over 2000 questions submitted.
- Over 60 questions were listed in the shortlisting survey.
- 660 responses to the shortlisting survey.
- 26 questions taken to the final priority setting workshop.
- 31 people participated in the workshop.

4.2 Limitations of the project

The project had some challenges, which has resulted in some limitations in the process.

We understood that it could be difficult to engage with young people (under the age of 25) and people from ethnic minority backgrounds in this project. For the first survey, we planned two workshops aimed at the different underrepresented groups. The workshops were planned for evenings and online to avoid work or education times, and they were promoted on social media channels.

Despite taking these steps, the workshops were unattended, and the numbers of young people and people from ethnic minority backgrounds who completed the survey remained low.

We had a similar experience with the interim survey.

The second limitation of this project is the survey design for the interim survey. Having decided on the survey design with the steering group, it quickly because clear that Survey Monkey (the charity's survey programme) was not well suited to the chosen survey design. As Survey Monkey's limitations became apparent other survey design software was immediately investigated, but unfortunately was beyond our budget. As SurveyMonkey would not allow us to design the survey in the way we had hoped we had to make do with a long list of questions which could not be divided into topic groups.

While we do not have feedback from people who left the survey early, this was commented on by a couple of potential respondents. One academic with lived experience of cardiomyopathy contacted us to say that the survey did not meet standards of validity and reliability.

4.3 Learning from the process

The project has provided many opportunities for learning, beyond the top 10 priority questions. Through promotion of the surveys, we have learnt which are the most effective communication channels with people with lived experience of cardiomyopathy. There was an obvious by a spike in numbers of survey responses following certain communications. In terms of engagement from people with lived experience, the monthly email roundup yielded the most responses. There was also an increase in survey completion when the partner organisations promoted the surveys in their newsletters. The interim survey was timed to be open over the period of the cardiomyopathy conference, and the survey was promoted in multiple ways at the conference; however, it did not result in a large increase in responses, which was unexpected.

Designing the second survey was also a learning experience. Fundamentally, additional resources such as survey software needs to be built into the initial budget plans. This is difficult to manage, as the second survey design is not known until after the initial survey responses have been analysed. As a result, we found out too late that SurveyMonkey did not meet our survey design requirements, but we also had no budget to use other survey software.

We also learnt about the importance of the different roles in the project, in particular that it is essential to have an organised an effective PSP Coordinator. Due to constraints in capacity in other teams, the PSP Coordinator was moved away from this PSP in the final month of the project, at the point of final arrangements for the priority setting workshop. This was the most administrative intensive part of the project and was time consuming. Given the complex nature of the project, it was a challenge to hand this over to another colleague in administration. The learning from this episode was that, once assigned, the project roles should be respected until the end of the project.

5. Next steps

Cardiomyopathy UK is working with partners on the PSP and members of the Steering Group to disseminate the priorities for future research. This includes:

- Communicating the research priorities in newsletters, email communications and on social media.
- Submitting abstracts to relevant conferences to have posters or presentations.
- Submitting abstracts to relevant journals.
- An opinion piece in a journal.
- Holding events with researchers.
- Identifying research funding opportunities to ensure there is funding available to conduct research.

It is most important that the research takes place and people affected by cardiomyopathy get the answers to the questions they have.

6. Who was involved?

Cardiomyopathy UK led the future research priorities partnership with the James Lind Alliance.

PSP Lead: Laura Cook (with Charlotte Gallagher as the initial lead and Katharine McIntosh in the interim)

JLA Adviser: Louise Dunford

PSP Coordinator: Beckie Gray

Information Specialist: Kristina Staley

We would like to thank the members of the Steering Group:

Alison Fielding, Andy Smith, Brian Halliday, Caroline Coats, Ella Field, Helen Alexander, Jayne Partridge, Jenn Zhang, Jenny Moon, Libby Jarman, LaRisha Porter, Marcia Malcolm, Pauline Aiston, Robbie Jones, Ruth Newbury-Ecob, Sandra Miller, Tootie Bueser

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