Mitochondrial Disease Priority Setting Partnership: Setting Research Priorities with Patients, Carers and Clinicians

https://www.jla.nihr.ac.uk/priority-setting-partnerships/mitochondrial-disease/downloads/mitochondrial-Disease-PSP-final-report.pdf

For which topic were research priorities identified?
mitochondrial disease

In which location was the research priority setting conducted?
Europe - United Kingdom

Why was it conducted at all?
Despite growing research activity in the UK and across the globe, there are still many unanswered questions about mitochondrial disease. Resources for research are limited and consequently it is important for researchers and funding organisations to understand which are the most important questions for research to address from the point of view of patients, carers and healthcare professionals, so that research funding can be targeted accordingly. As for many rare diseases, mitochondrial disease has received less research attention than common conditions, so the need to consult with those affected is intensified.

What was the objective?
to stimulate research by finding out what people with these conditions, their carers and healthcare professionals believe to be the most important questions about the care, treatment, management and the natural history of mitochondrial disease, for adults and children

What was the outcome?
a ranking list of 10 research questions

How long did the research prioritization take?
March 2019 - January 2020

Which methods were used to identify research priorities?
JLA method

How were the priorities for research identified exactly?
Step 1: setting up PSP: steering group established. Step 2: collecting uncertainties: via survey, participants were asked to identify the questions they would like answered by research, total of 709 questions submitted. Step 3: data processing: out-of-scope removed, similar combined, indicative questions formulated, check against evidence, resulting in longlist of 42 unanswered questions. Step 4: interim ranking: survey asking people to consider each of the 42 questions, choose 10 and then rank them in order of priority, equal weighting was given to responses from patients, carers and healthcare professionals, top 24 questions were taken forward to workshop. Step 5: final prioritization: workshop: participants were asked to look at the 24 shortlisted questions before workshop and to think about how they would rank them in order of importance, series of small group discussions and small group rankings, plenary discussion to achieve consensus

Which stakeholders took part?
Patients, carers, clinicians. Survey: 147 participants: people with a mitochondrial disease (34%), carers or relatives (32%), and healthcare professionals (34%). Interim ranking: 166 participants: 63% patients, 21% carers or relatives, 16% healthcare professionals. Workshop: 32 participants: 8 people with a mitochondrial disease, whose conditions affected vision, control of movement and epilepsy, 9 carers/family members, 2 patient organization representatives, 13 healthcare professionals from 9 different disciplines.

How were stakeholders recruited?
The steering group members and organizations supporting the project sent the survey out to their networks, via email, newsletters, social media and websites. The second survey went out to the same networks as the first survey with additional promotion through four videos in different languages. Workshop: Invitations to the workshop were sent out through the steering group.

Were stakeholders actively involved or did they just participate?
Stakeholders not only participated but were also actively involved in the research prioritization process: They were part of a steering group. The steering group consisted of patient organization representatives and clinicians. The members sent out the survey, invited workshop participants and also took part in the workshop.