

# Rare Inherited Anaemias Priority Setting Partnership

JLA PSP final report (2018)

<https://www.jla.nihr.ac.uk/priority-setting-partnerships/rare-inherited-anaemias/downloads/Rare-Inherited-Anaemias-PSP-FINAL-REPORT.PDF>

## For which topic were research priorities identified?

rare inherited anaemias

## In which location was the research priority setting conducted?

Europe - United Kingdom

## Why was it conducted at all?

Patients with a rare inherited anaemia may take years to receive a precise diagnosis, if at all. Lack of specialist knowledge, or poor access to the few centres of specialist expertise, means that not all patients will have access to the same standard of care. Few treatments have been developed because of the lack of research in this area. When treatments are available, not all patients can access them, and their doctors may not have full knowledge of how the treatment should be given.

## What was the objective?

to stimulate research on rare inherited anaemias by finding out what people with these conditions, their carers and health professionals believe to be the most important areas for future research

## What was the outcome?

a ranking list of 10 research questions

## How long did the research prioritization take?

early 2016 - December 2017

## 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 discussed limits of PSP and agreed on which rare inherited anaemias should be included. Step 2: collecting research questions: via survey, participants were asked to identify the questions they would like answered by research, 557 questions submitted. Step 3: data processing: out-of-scope removed, duplicates combined, check against published evidence, resulting in 48 unanswered questions. Step 4: interim ranking: survey asking people to rate each question to indicate the degree of importance, shortlist of 25 questions was agreed by steering group. Step 5: final prioritization: workshop: participants were asked to look at the 25 questions before workshop and to think about how they would rank them in order of importance, small group discussions

## Which stakeholders took part?

People with rare inherited anaemias, their carers and health professionals. Survey: 88 participants: 23% with a rare inherited anaemia, 27% carers, relatives or friends, 44% health or social care professionals and 6% came from other organizations. Interim ranking: 120 participants: 24% with rare inherited anaemia, 28% carers, relatives or friends, 41% health or social care professionals and 7% came from other organizations. Workshop: 31 people with different rare inherited anaemias, carers and health professionals.

## How were stakeholders recruited?

The partners and Steering Group members sent the survey out to their networks, via email, newsletters, social media, websites and blogs. The second survey went out to everyone from the first survey who wanted to stay involved, and to all the same networks. As to the workshop, participants were recruited through the Steering Group's networks, via patient and clinician groups and through social media.

## 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 15 members: patient representatives, partner organizations, researchers, and 1 JLA advisor. The members oversaw the process, promoted the survey, agreed on a shortlist and participated in the workshop.