

Pernicious Anaemia Priority Setting Partnership

PROTOCOL

17th November, 2020

1. Purpose of the PSP and background

The purpose of this protocol is to clearly set out the aims, objectives and commitments of the Pernicious Anaemia Priority Setting Partnership (PSP) in line with James Lind Alliance (JLA) principles. The Protocol is a JLA requirement and will be published on the PSP's page of the JLA website. The Steering Group will review the Protocol regularly and any updated version will be sent to the JLA.

The JLA is a non-profit making initiative, established in 2004. It brings patients, carers and clinicians together in PSPs. These PSPs identify and prioritise the evidence uncertainties, or 'unanswered questions', that they agree are the most important for research in their topic area. Traditionally PSPs have focused on uncertainties about the effects of treatments, but some PSPs have chosen to broaden their scope beyond that. The aim of a PSP is to help ensure that those who fund health research are aware of what really matters to patients, carers and clinicians. The National Institute for Health Research (NIHR – www.nihr.ac.uk) coordinates the infrastructure of the JLA to oversee the processes for PSPs, based at the NIHR Evaluation, Trials and Studies Coordinating Centre (NETSCC), University of Southampton.

The Pernicious Anaemia Society (PAS) was established in 2004 to provide information, help and support to patients with Pernicious Anaemia and their families and friends. Its original remit was to provide newly diagnosed patients with a 'plain English' explanation of their disease and provide peer support via an online forum. The forum attracted patients from across the globe and it soon became obvious that there were serious problems with the way in which Pernicious Anaemia (PA) was diagnosed and treated. A number of active members of the forum decided to establish the PAS on more solid foundations and these members met in 2005 and agreed that the society should become a registered charity and the founding members the Trustees. PAS was entered onto the Charity Commission's register in July 2006. Headed by the Executive Chairman the society then began campaigning to raise awareness of the problems with the diagnosis and treatment of the condition. It has been successful getting health decision makers to appreciate the problems faced by patients but the Covid-19 pandemic brought with it serious problems for patients some of whom had their treatment stopped altogether, or were told to buy B12 Tablets from internet stores while others were simply told that they no longer had PA. Our Healthcare Affiliates were horrified by what they were witnessing and made enquiries about funding streams to finance a thorough review of the issues surrounding diagnosis and treatment of PA; and reported back to the PAS that the James Lind Alliance had been suggested as being an ideal way to get patients and clinicians to identify the issues that need to be addressed. In August 2020 the Executive Chairman made a preliminary telephone call to the JLA who then explained the due processes involved. The society then recruited Health Care Professionals and selected members to form the PSP.

2. Aims, objectives and scope of the PSP

The aim of the Pernicious Anaemia PSP is to identify the unanswered questions about Pernicious Anaemia from patient, carer and clinical perspectives and then prioritise those that patients, carers and clinicians agree are the most important for research to address.

The objectives of the PSP are to:

- work with patients, carers and clinicians to identify uncertainties about the Diagnosis and Management of Pernicious Anaemia and to agree by consensus a prioritised list of those uncertainties, for research
- to publicise the results of the PSP and process
- to take the results to research commissioning bodies to be considered for funding

The scope of the Pernicious Anaemia PSP is defined as:

uncertainties about the Cause, Diagnosis, Treatment and Care of Pernicious Anaemia, including:

- Identifying the uncertainties with the clinical definition of Pernicious Anaemia
- Identifying the uncertainties with the tests to Diagnose Vitamin B12 Deficiency
- Identifying the uncertainties with the tests to Diagnose Pernicious Anaemia
- Identifying the uncertainties with the current management replacement therapy regimen
- Identifying the uncertainties with other replacement therapy delivery methods

The Steering Group is responsible for discussing what implications the scope of the PSP will have for the evidence-checking stage of the process. Resources and expertise will be put in place to do this evidence checking.

3. The Steering Group

The Steering Group includes membership of patients and carers and clinicians¹ as individuals or representatives from a relevant group.

The Pernicious Anaemia PSP will be led and managed by a Steering Group involving the following:

Patient and carer representative/s:

Martyn Hooper Patient
Sophie Barry Patient
Jacqui Gold Patient
Stephen Ganter Patient
Petra Visser Patient
Karyl Carter Patient

Clinical representative/s:

Dr Rakesh Koria General Practitioner, NHS
Dr Nicola Ward Pharmacist, De Montfort University
Dr Heidi Seage Psychologist, Cardiff Metropolitan University
Dr Asim Naqvi General Practitioner, NHS
Dr John Warren Retired Consultant Physician

PSP Lead – Martyn Hooper, Chair of the Pernicious Anaemia Society

Informatics Manager – Petra Visser, Pernicious Anaemia Society

PSP Co-ordinator – Karyl Carter – Pernicious Anaemia Society

James Lind Alliance Senior Adviser and **Chair** of the Steering Group – Katherine Cowan, JLA

The Steering Group will agree the resources, including time and expertise that they will be able to contribute to each stage of the process, with input and advice from the JLA.

1. In some cases, it has been suggested that researchers are represented on the Steering Group, to advise on the shaping of research questions. However, researchers cannot participate in the prioritisation exercise. This is to ensure that the final prioritised research questions are those agreed by patients, carers and clinicians only, in line with the JLA's mission.

4. Partners

Organisations and individuals will be invited to be involved with the PSP as partners. Partners are organisations or groups who will commit to supporting the PSP, promoting the process and encouraging their represented groups or members to participate. Organisations which can reach and advocate for these groups will be invited to become involved in the PSP. Partners represent the following groups:

- people who have had Pernicious Anaemia
- carers of people who have had Pernicious Anaemia
- health and social care professionals – with experience of Pernicious Anaemia.

Exclusion criteria :

Some organisations may be judged by the JLA or the Steering Group to have conflicts of interest. These may be perceived to potentially cause unacceptable bias as a member of the Steering Group. As this is likely to affect the ultimate findings of the PSP, those organisations will not be invited to participate. It is possible, however, that interested parties may participate in a purely observational capacity when the Steering Group considers it may be helpful.

5. The methods the PSP will use

This section describes a schedule of proposed steps through which the PSP aims to meet its objectives. The process is iterative and dependent on the active participation and contribution of different groups. The methods used in any step will be agreed through consultation between the Steering Group members, guided by the PSP's aims and objectives. More details of the method are in the Guidebook section of the JLA website at www.jla.nihr.ac.uk where examples of the work of other JLA PSPs can be seen.

Step 1: Identification and invitation of potential partners:

Potential partner organisations will be identified through a process of peer knowledge and consultation, through the Steering Group members' networks. Potential partners will be contacted and informed of the establishment and aims of the Pernicious Anaemia PSP.

Step 2: Awareness raising:

PSPs will need to raise awareness of their proposed activity among their patient, carer and clinician communities, in order to secure support and participation. Depending on budget, this may be done by a face-to-face meeting, or there may be other ways in which the process can be launched, e.g. via social media. It may be carried out as part of steps 1 and/or 3. The Steering Group should advise on when to do this. Awareness raising has several key objectives:

- to present the proposed plan for the PSP
- to generate support for the process
- to encourage participation in the process
- to initiate discussion, answer questions and address concerns.

Step 3: Identifying evidence uncertainties:

The Pernicious Anaemia PSP will carry out a consultation to gather uncertainties from patients, carers and clinicians. A period of One Month will be given to complete this exercise (which may be revised by the Steering Group if required).

The Pernicious Anaemia PSP recognises that the following groups may require additional consideration:

- People from Black, Asian and minority ethnic groups
- Infants, Juveniles and Teenagers
- Elderly Isolated Patients
- Primary Care Clinicians including GP's and Haematologists

The Steering Group should advise and agree on the best method of consultation for reaching its groups. Previous PSP's have, for example, developed online and paper surveys, or carried out face-to-face work where groups are unlikely to access the internet.

Existing sources of evidence uncertainties may also be searched:

- Legacy survey published and peer reviewed
- Email enquiry analysis
- Focus Group with Support Group Co-ordinators
- Telephone Helpline Call Analysis
- Office Telephone Call Enquiry Analysis
- 'Talking Heads' Video Capture

Step 4: Refining questions and uncertainties:

The consultation process will produce 'raw' questions and comments indicating patients', carers' and clinicians' areas of uncertainty. These raw questions will be categorised and refined by Kristina Staley into summary questions which are clear, addressable by research, and understandable to all. Similar or duplicate questions will be combined where appropriate. Out-of-scope and 'answered' submissions will be compiled separately. The Steering Group will have oversight of this process to ensure that the raw data is being interpreted appropriately and that the summary questions are being worded in a way that is understandable to all audiences. The JLA Adviser will observe to ensure accountability and transparency.

This will result in a long list of in-scope summary questions. These are not research questions and to try and word them as such may make them too technical for a non-research audience. They will be framed as researchable questions that capture the themes and topics that people have suggested.

The summary questions will then be checked against evidence to determine whether they have already been answered by research. This will be done by . The PSP will complete the JLA Question Verification Form, which clearly describes the process used to verify the uncertainty of the questions, before starting prioritisation. The Question Verification Form includes details of the types and sources of evidence used to check uncertainty. The Question Verification Form should be published on the JLA website as soon as it has been agreed to enable researchers and other stakeholders to understand how the PSP has decided that its questions are unanswered, and any limitations of this.

Questions that are not adequately addressed by previous research will be collated and recorded on a standard JLA template by Kristina Staley. This will show the checking undertaken to make sure that the uncertainties have not already been answered. The data should be submitted to the JLA for publication on its website on completion of the priority setting exercise, taking into account any changes made at the final workshop, in order to ensure that PSP results are publicly available.

The Steering Group will also consider how it will deal with submitted questions that have been answered, and questions that are out of scope.

Step 5: Prioritisation – interim and final stages

The aim of the final stage of the priority setting process is to prioritise through consensus the identified uncertainties about Pernicious Anaemia. This will involve input from patients, carers and clinicians. The JLA encourages PSPs to involve as wide a range of people as possible, including those who did and did not contribute to the first consultation.

There are usually two stages of prioritisation.

1. Interim prioritisation is the stage where the long list of questions is reduced to a shorter list that can be taken to the final priority setting workshop. This is aimed at a wide audience and is done using similar methods to the first consultation. With the JLA's guidance, the Steering Group will agree the method and consider how best to reach and engage patients, carers and clinicians in the process. The most highly ranked questions (around 25) will be taken to a final priority setting workshop. Where the interim prioritisation does not produce a clear ranking or cut off point, the Steering Group will decide which questions are taken forwards to the final prioritisation.
2. The final priority setting stage is generally a one-day workshop facilitated by the JLA. With guidance from the JLA and input from the Steering Group, up to 30 patients, carers and clinicians will be recruited to participate in a day of discussion and ranking, to determine the top 10 questions for research. All participants will declare their interests. The Steering Group will advise on any adaptations needed to ensure that the process is inclusive and accessible.

6. Dissemination of results

The Steering Group will identify audiences with which it wants to engage when disseminating the results of the priority setting process, such as researchers, funders and the patient and clinical communities. They will need to determine how best to communicate the results and who will take responsibility for this. Previous PSPs' outputs have included academic papers, lay reports, infographics, conference presentations and videos for social media.

It should be noted that the priorities are not worded as research questions. The Steering Group should discuss how they will work with researchers and funders to establish how to address the priorities and to work out what the research questions are that will address the issues that people have prioritised. The dissemination of the results of the PSP will be led by Petra Visser.

The JLA encourages PSPs to report back about any activities that have come about because of the PSP, including funded research. Please send any details to jla@soton.ac.uk.

7. Agreement of the Steering Group

The Pernicious Anaemia PSP Steering Group agreed the content and direction of this Protocol on 17th December 2020.