The Treatment of Pemphigus and Pemphigoid Priority Setting Partnership

PROTOCOL [September 2019][1] Version [5][2]

1. Purpose of the PSP and background

The purpose of this protocol is to set out clearly the aims, objectives and commitments of the Treatment of Pemphigus and Pemphigoid 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 focussed 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 BLISTER trial which looked at the role of doxycycline in treating bullous pemphigoid was recently conducted by the UK Dermatology Clinical Trials Network and led by a team from the Centre of Evidence based Dermatology in Nottingham. BLISTER was conducted in response to a research gap highlighted by a Cochrane review on treatments for bullous pemphigoid. Before undertaking any further trials in the area, it was decided that a PSP should be conducted to determine the most important questions to answer.

2. Aims, objectives and scope of the PSP

The aim of the Treatment of Pemphigus and Pemphigoid PSP is to identify the unanswered questions about bullous pemphigoid, pemphigus vulgaris and mucous membrane pemphigoid 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 this PSP are to:

- work with patients, carers and clinicians to identify uncertainties about treatments for bullous pemphigoid, pemphigus vulgaris and mucous membrane pemphigoid.
- to agree by consensus a prioritised list of those uncertainties, for research.

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[1] This protocol template should be modified with agreement from the JLA Adviser to reflect the make-up of different PSPs and the organisations driving them. This protocol template document was last updated by the JLA in November 2018.
[2] The Steering Group is responsible for ensuring any updates or amendments to the PSP plan are included in subsequent versions of the Protocol and sent to the JLA for publication on the website.

- 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 this PSP is defined as:

- Treatments; to include topical and systemic treatments, dressings, procedures and side-effects
- Treatment of both the skin and mucous membranes

This PSP will focus on the treatments for of pemphigus and pemphigoid to keep the project manageable within the available resources. It is recognised by the steering committee that there are other areas of potential research that are of equal importance, for example pathogenesis, prevention, diagnosis, service delivery, education and awareness, but they will be excluded from the scope of this PSP. Better diagnosis, increased awareness and pathology were all highlighted as important areas that require further research in a recent study[1] and any data that is submitted on these topics will be collated and made available for any future PSPs covering these topics. 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 clinicians[3], as individuals or representatives from a relevant group.

This PSP will be led and managed by a Steering Group involving the following:

Patient and carer representative/s:

Ingrid	Thompson	Bullous pemphigoid
Lisa	Shendge	Non-bullous pemphigoid
Isobel	Davies	Mucous membrane pemphigoid
Badj	Rahman	Mucous membrane pemphigoid
Jag	Barha	Pemphigus vulgaris
Sharon	Hockey	Pemphigus vulgaris

Clinical representative/s:

Debra	Brown	Dermatology Specialist Nurse
John	Dart	Ophthalmologist
Phil	Hampton	Dermatologist
Sharleen	Hill	Dermatologist
Gudula	Kirtschig	Dermatologist
Antonia	Lloyd-Lavery	Dermatologist
Ruth	Murphy	Dermatologist
Saaeha	Rauz	Ophthalmologist

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[3] 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.

Jane	Setterfield	Dermatologist
Christina	Waistell	Dermatology Specialist Nurse
Melanie	Westmoreland	Dermatology Research Nurse

Project Leads:

Joanne Chalmers, Senior Research Fellow, University of Nottingham
Karen Harman, Consultant Dermatologist, University Hospitals Leicester and Clinical Associate Professor, University of Nottingham

Information Specialist:
Douglas Grindlay, University of Nottingham

Project Coordinator:

Margaret McPhee, University of Nottingham

James Lind Alliance Adviser and Chair of the Steering Group:

Sheela Upadhyaya, 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.

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 bullous pemphigoid, pemphigus vulgaris or mucous membrane pemphigoid
- Carers of people who have bullous pemphigoid, pemphigus vulgaris and mucous membrane pemphigoid. The terms ‘carer’ is used here to mean anyone involved in supporting the care of the person with pemphigus or pemphigoid and could include family members, friends or official carers
- Health and social care professionals - with experience of bullous pemphigoid, pemphigus vulgaris and mucous membrane pemphigoid
- British Association of Dermatologists
- British Society for Geriatric Dermatology
- British Dermatology Nursing Group
- Primary Care Dermatology Society
- British Society for Medical Dermatology

- Patient support groups - PEM Friends, Pemphigus Vulgaris Network, MMP Group
- British Society for Oral Medicine
- Royal College of Ophthalmologists
- British Geriatrics Society
- UK Dermatology Clinical Trials Network

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 Treatment of Pemphigus and Pemphigoid 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 Treatment of Pemphigus and Pemphigoid PSP will carry out a consultation to gather uncertainties from patients, carers and clinicians. A period of 18 months will be given to complete this exercise (which may be revised by the Steering Group if required).

The Treatment of Pemphigus and Pemphigoid PSP recognises that patients with no internet access or who are not comfortable completing the survey 'online', or the very elderly or frail, may need additional consideration and support to complete the surveys

The Steering Group will use the following methods to reach the target groups:

- Online survey
- Paper survey
- Face to face survey completion on hospital ward or during clinic visits

Research recommendations in systematic reviews and clinical guidelines; protocols for systematic reviews being prepared and systematic reviews being prepared (Prospero search) and registers of ongoing trials (WHO Trials register) will also be used to identify uncertainties.

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 Karen Harman, Joanne Chalmers, Douglas Grindlay and Maggie McPhee 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. The evidence will be collated by Douglas Grindlay. 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 Karen Harman. 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 the Treatment of Blistering Skin Diseases. 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 Joanne Chalmers.

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 Treatment of Blistering Skin diseases PSP Steering Group agreed the content and direction of this Protocol on update Sept 2019.

1. Lamberts A, Yale M, Grando SA, Horvath B, Zillikens D, Jonkman MF. Unmet Needs in Pemphigoid Diseases: An International Survey Amongst Patients, Clinicians and Researchers. Acta Derm Venereol 2019;99:224-5.