Childhood Neurological Conditions Priority Setting Partnership

PROTOCOL December 4 2019

1. Purpose of the PSP and background

The purpose of this protocol is to clearly set out the aims, objectives and commitments of the Childhood Neurological Conditions 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.

Within child and young adult health, successful priority setting partnerships in preterm birth, neurodevelopmental disorders, neurodisability, mental health and cancer care have been completed using such methodologies. Children with neurological conditions contribute significantly to childhood Neurodisability and make up a significant proportion of children with health challenges. Here the rapidly increasing availability of novel and expensive therapies for many genetic conditions risks the agenda of research being driven by the pharmacological and biotechnology industry. As such, it has been important, that a priority setting partnership is developed to set the research priorities in children and young people with neurological conditions.

2. Aims, objectives and scope of the PSP

The aim of the Childhood Neurological Conditions PSP is to identify the unanswered questions, which we refer to as 'uncertainties', about children with neurological conditions 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 Childhood Neurological Disorders Research PSP are to:

- Work with patients, their parents and primary carers, and clinicians to identify uncertainties about the effectiveness of treatments for children with neurological disorders
- Agree by consensus a prioritised list of those uncertainties for research
- Publicise the results of the PSP and process
- Engage research commissioning bodies that will be potential funding source for the identified research priorities

The scope of the Childhood Neurological Disorders PSP is defined as:

To identify key areas where there is no clarity about the effectiveness of interventions, treatments, therapies or procedures aimed at improving the health and wellbeing of children and young adults with neurological conditions.

Subject areas covered by this scope would include but would not be limited to:

- **Epilepsies and Related Paroxysmal Disorders**
- **Movement disorders**, such as dystonia, chorea, tremor, parkinsonism
- **Neuromuscular disorders**, such as congenital myopathy, range of muscular dystrophies, spinal muscular atrophy
- **Neuroinflammatory disorders**, such as relapsing demyelination, neuromyelitis optica spectrum disorder, encephalitis (Infectious and auto immune)
- **Headache disorders** such as migraine, cluster headaches, idiopathic intracranial hypertension
- **Rare metabolic** and genetic conditions affecting central nervous system, such as leukodystrophies, ataxia telangiectasia, interferonopathies
- **Neurovascular and stroke**
- **Acquired Brain and Spinal cord Injury**
- **Sleep Disorders**
- **Functional Neurological Symptoms and Signs**
- **Neurological conditions where treatment evidence and general understanding is weak**

A more comprehensive list of conditions included in the scope of this PSP can found in Appendix 1 of this Protocol.

The PSP will exclude from its scope questions about:

- Subject areas comprehensively covered by other PSPs completed in past 5 years. These currently include autism and neurodevelopmental disorders, multiple sclerosis, mitochondrial disorders and childhood neurodisability.

- Childhood neurological services and care outside of the UK;

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[1], as individuals or representatives from a relevant group.

Name	Role
Suzannah Kinsella	James Lind Alliance, External Chair to the PSP
Dr Ming Lim	PSP Chair/Lead
Philip Levine	PSP Secretary/Administrator
Professor Helen Cross	PSP Co-Lead
Professor Manju Kurian	BPNA Chair of Research
Dr Sam Amin	Consultant Paediatric Neurologist
Dr Richard Chin	Reader in Paediatric Neurosciences
Dr Ava Easton	CEO, The Encephalitis Society
Barbara Babcock	Past Chair Transverse Myelitis Society (Patient reported outcome measures)
Siobhan Hannan	Patient Representative
Molly Brick	Young Adult Representative
Rhys Inward	Young Adult Representative
Harriet Pollard	Young Adult Representative
Carol-Anne Partridge	Patient Representative
Ben Green	Lay Representative, Co-opted
Dr Andrew Mallick	Consultant Paediatric Neurologist
Professor Jeremy Parr	Senior Lecturer Paediatric Neurodisability
Dr Jill Cadwgan	Neurorehabilitation

Priority Setting Partnership Expert Panel – to provide condition specific advice to the PSP as it progresses

Name	Expertise
Professor Robert McFarland	Muscle and mitochondrial
Dr Ruth Williams	neurodegenerative disorders and epilepsy
Dr William Whitehouse	Headache and acute neurology
Dr Evangeline Wassmer	Inflammation and inherited white mater disorder
Dr Vijeya Ganesan	Stroke
Dr Rachel Kneen	Neuro Infectious diseases
Dr Daniel E Lumsden	Movement disorder
Dr Anne-Marie Childs	Neuromuscular

[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.

Dr Anthony Hart	Neonatal Neurology

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:

- children who have neurological conditions
- parents/carers of people who have neurological conditions
- health and social care professionals - with experience of childhood neurological conditions.

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 Childhood Neurological Conditions 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 Childhood Neurological Conditions PSP will carry out a consultation to gather questions that have not been answered by research to date (we refer to these as uncertainties) from patients, carers and clinicians. A period of 3 months will be given to complete this exercise (which may be revised by the Steering Group if required based on the number and range of survey responses).

The Childhood Neurological Conditions PSP recognises that the following groups may require additional consideration:

- ensuring we hear the voices directly from children and young people, as well as their parents/carers
- ensure we have responses from those conditions with communications challenges such asThose with visual and hearing impairment, aphasia or significant motor disability with intact cognition following neurological condition

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

- Online survey with paper copies available;
- Reaching out through colleagues who work with children with neurological conditions and their carers to gather questions face-to-face in a range of situations, including at existing group meetings, and at reviews and clinics;
- Promotion at conferences and meetings.

Existing sources may also be searched for uncertainties. These can include research recommendations in systematic reviews and NICE and/or other college endorsed clinical guidelines; protocols for NHSE reviews being prepared, and registers of ongoing research. Expert panel members of the PSP will have a critical role in supporting the information specialist team to identify key documents within their speciality. The centralised collection and cataloguing of uncertainties will be managed by the PSP administrator, Phillip Levine. Uncertainties relating to treatment which are not adequately addressed by previous research will be finally agreed by the PSP Steering Group.

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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 the Expert Panel 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 chosen members within the committee. 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 the Information Specialist team. 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, considering 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 childhood neurological conditions. 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, parents/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 Ming Lim.

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 Childhood Neurological Conditions PSP Steering Group agreed the content and direction of this Protocol on [insert date].