



Priority Setting Partnerships

Neurodevelopmental Disorders Priority Setting Partnership

PROTOCOL April 1st, 2016

1. Purpose of the PSP and background

The purpose of this protocol is to set out the aims, objectives and commitments of the Neurodevelopmental Disorders Priority Setting Partnership (PSP) and the basic roles and responsibilities of the partners therein.

Neurodevelopmental disorders arise from disturbances in the growth and development of the brain and the nervous systems. Significant overlap exists among these disorders in risk factors, underlying molecular mechanisms and clinical outcomes related to emotional regulation, behaviour, learning, and self-control. Often children are given dual or multiple diagnoses dependant on these observed outcomes. In Ontario alone, more than 300,000 children and youth have been diagnosed with one or more neurodevelopment disorders.

The Ontario Brain Institute (OBI) is a provincially-funded, not-for-profit research institution seeking to maximize the impact of neuroscience and establish Ontario as a world leader in brain research, commercialization and care. Convergent partnerships are created between researchers, clinicians, industry, patients, and their advocates to foster discovery and deliver innovative products and services that improve the lives of those living with brain disorders. The Province of Ontario Neurodevelopmental Disorder Network (POND) is a partner research program of OBI, dedicated to addressing the urgent need for new and better targeted therapies in an effort to improve the long-term outcomes for children with neurodevelopmental disorders, including: Attention Deficient Hyperactivity Disorder, Autism Spectrum Disorder, Down Syndrome, Fragile X, Intellectual Disability, Obsessive Compulsive Disorder, Rett Syndrome, and Tourette Syndrome.

Central to improving outcomes for children with one or more neurodevelopmental disorders is ensuring that the research is focused on their needs. The Neurodevelopmental Disorder PSP has been set up to identify research questions that are relevant to patient, family and clinician. This approach provides a unique opportunity to identify uncertainties that are common, as well as unique, across these eight neurodevelopmental disorders, and help to ensure that the POND research program is addressing research questions that are relevant to patients and families.

The Neurodevelopmental Disorder PSP will be conducted based on the guidelines of the James Lind Alliance. The James Lind Alliance (JLA) is a non-profit making initiative funded by the National Institute of Health Research (NIHR – www.nihr.ac.uk). Its aim is to provide an infrastructure and process to help patients and clinicians work together to agree which are the most important treatment uncertainties affecting their particular interest, in order to influence the prioritization of future research.

SCOPE

Any questions pertaining to interventions, such as assessment, diagnosis therapies, care or supports, and the improvement of the health¹ and wellbeing² of persons with a neurodevelopmental disorder³ (see notes below).

1. Health is defined by the 'components of health' described by the World Health Organization in the International Classification of Functioning Disability and Health (figure 1); namely body functions & structures, activities and participation, further details are provided in Appendix 1.

2. Wellbeing is defined by how people feel about their life and their ability to reach their aspirations.

3. For the purposes of this project, neurodevelopmental disorders are a group of conditions which starts early in development, and are characterized by developmental deficits that produce impairments of personal, social, academic, or occupational functioning. The range of developmental deficits varies from very specific limitations of learning to global impairments of social skills or intelligence. Some examples are: Attention Deficient Hyperactivity Disorder, Autism Spectrum Disorder, Down Syndrome, Fragile X, Intellectual Disability, Obsessive Compulsive Disorder, Rett Syndrome, and Tourette Syndrome.

2. Aims and objectives of the Neurodevelopmental Disorders PSP

The aim of the Neurodevelopmental Disorders PSP is to identify the unanswered questions about the health and wellbeing of persons living with a neurodevelopmental disorder from patient, family and clinical perspectives and then prioritize those that patients, families, and clinicians agree are the most important.

The objectives of the Neurodevelopmental Disorders PSP are to:

- work with patient representatives, families and clinicians to identify uncertainties about ways to improve the health and wellbeing of persons with a neurodevelopmental disorder and the treatment and management of neurodevelopmental disorders
- agree by consensus on a prioritized list of those research uncertainties
- publicize the process and results of the PSP
- share the results with the POND Executive Committee and funding bodies to be considered for funding.

3. The Steering Group

The Neurodevelopmental Disorders PSP will be led and managed by the following:

Patient representatives:

- Sue Lynch, Community Living Toronto
- James Mitchell, ADHD patient advocate
- Margaret Spoelstra, Autism Ontario

Clinical representatives:

- Evdokia Anagnostou (Steering Group Chair), Holland Bloorview Kids Rehabilitation Hospital
- Brendan Andrade, Centre for Addition and Mental Health
- Rob Nicolson, Children's Hospital at London Health Sciences Centre

Not-For-Profit representatives:

- the Ontario Brain Institute (OBI):

- Carla Arasanz
- Jessica Jordao

The Partnership and the priority setting process will be supported and guided by:

- The James Lind Alliance (JLA) senior advisor:
 - Katherine Cowan

The Steering Group includes representation of patient/carer groups and clinicians.

The Steering Group will agree to the resources, including time and expertise that they will be able to contribute to each stage of the process. The JLA will advise on this.

4. The Wider Partners

Organizations and individuals will be invited to be involved with the PSP as partners. Partners are groups or individuals who will commit to supporting the PSP by disseminating the PSP survey and helping the PSP to gather questions and uncertainties of practical clinical importance relating to the treatment and management of the health problem in question. Partners represent the following groups:

- people who have had neurodevelopmental disorders including:
 - Attention Deficit Hyperactivity Disorder
 - Autism Spectrum Disorder
 - Down Syndrome
 - Fragile X Syndrome
 - Intellectual Disability
 - Obsessive Compulsive Disorder
 - Rett Syndrome
 - Tourette Syndrome
 - Learning disabilities
 - Other genetic syndromes related to intellectual disability
- carers of people who have had neurodevelopmental disorders
- medical doctors, nurses and professionals allied to medicine with clinical experience of neurodevelopmental disorders.
- not-for-profit groups dedicated to advocacy for people who have had neurodevelopmental disorders

It is important that all organizations that can reach and advocate for these groups should be invited to become involved in the PSP. The JLA Advisor will take responsibility for ensuring the various stakeholder groups are able to contribute equally to the process.

Exclusion criteria

Some organizations may be judged by the JLA or the Steering Group to have conflicts of interest. These may be perceived to adversely affect those organizations' views, causing unacceptable bias. As this is likely to affect the ultimate findings of the PSP, those organizations 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 stages through which the PSP aims to fulfil its objectives. The process is iterative and dependent on the active participation and contribution of different groups. The methods adopted in any stage will be agreed through consultation between the Steering Group members, guided by the PSP's aims and objectives. More details can be found in the Guidebook section of the JLA website at www.jla.nihr.ac.uk where examples of the work of other JLA PSPs can also be seen.

Step 1: Identification and invitation of potential partners

Potential partner organizations 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 Neurodevelopmental Disorders PSP and invited to attend and participate in an initial stakeholder meeting.

Step 2: Identifying research uncertainties

Each partner will identify a method for soliciting from its members questions and uncertainties of practical clinical importance relating to the treatment and management of Neurodevelopmental Disorders. A period of three months will be given to complete this exercise.

The methods may be designed according to the nature and membership of each organization, but must be as transparent, inclusive and representative as practicable. Methods may include membership meetings, email consultation, postal or web-based questionnaires, internet message boards and focus group work.

Existing sources of information about treatment and management uncertainties for patients and clinicians will be searched. These can include question-answering services for patients and carers and for clinicians; research recommendations in systematic reviews and clinical guidelines; protocols for systematic reviews being prepared and registers of ongoing research.

The starting point for identifying sources of uncertainties and research recommendations is NHS Evidence: www.evidence.nhs.uk.

Step 3: Refining questions and uncertainties

The Steering Group will need to have agreed exactly who will be responsible for this stage – the JLA can advise on the amount of time likely to be required for its execution. The JLA will participate in this process as an observer, to ensure accountability and transparency.

The consultation process will produce "raw" unanswered questions about diagnosis and the effects of treatments. These raw questions will be assembled and categorised and refined by the Information Specialist and Steering Group into "collated indicative questions" which are clear, addressable by research and understandable to all. Similar or duplicate questions will be combined where appropriate.

Systematic reviews and guidelines will be identified and checked by the Information Specialist to see to what extent these refined questions have, or have not, been answered by previous research. Sometimes,

uncertainties are expressed that can in fact be resolved with reference to existing research evidence – *i.e.* they are "unrecognised knowns" and not uncertainties. If a question about treatment effects can be answered with existing information but this is not known, it suggests that information is not being communicated effectively to those who need it. Accordingly, the JLA recommends strongly that PSPs keep a record of these 'answerable questions' and deal with them separately from the 'true uncertainties' considered during the research priority setting process.¹

Uncertainties which are not adequately addressed by previous research will be collated and recorded (on a template supplied by the JLA) by the Information Specialist. This will demonstrate the checking undertaken to make sure that the uncertainties have not already been answered. This is the responsibility of the Steering Group, which will need to have agreed on personnel and resources to carry out this accountability. 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.

Step 5: Prioritization – interim and final stages

The aim of the final stage of the priority setting process is to prioritize through consensus the identified uncertainties relating to the treatment or management of neurodevelopmental disorders. This will be carried out by members of the Steering Group and the wider partnership that represents patients and clinicians.

- The interim stage, to proceed from a long list of uncertainties to a shorter list to be discussed at the final priority setting workshop (e.g. up to 30), may be carried out over email or online, whereby organizations consult their membership and choose and rank their top 10 most important uncertainties. There are examples of how other PSPs have achieved this at www.jla.nihr.ac.uk in the Key Documents of the [Anaesthesia and Perioperative Care PSP](#) section and the [Childhood Disability PSP](#) section.
- The final stage to reach, for example, 10 prioritized uncertainties is likely to be conducted in a face-to-face meeting, using group discussions and plenary sessions.
- The methods used for this prioritization process will be determined by consultation with partner organizations and with the advice of the JLA Adviser. Methods which have been identified as potentially useful in this process include: adapted Delphi techniques; expert panels or nominal group techniques; consensus development conference; electronic nominal group and online voting; interactive research agenda setting and focus groups.

The JLA will facilitate this process and ensure transparency, accountability and fairness. Participants will be expected to declare their interests in advance of this meeting.

6. Dissemination of findings and research

Findings and research

It is anticipated that the findings of the Neurodevelopmental Disorders PSP will be reported to the POND Executive Committee and research funding charities. Steering Group members and partners are expected to develop the prioritized uncertainties into research questions, and to work to establish the research needs of those unanswered questions to use when approaching potential funders, or when allocating funding for research themselves, if applicable.

Publicity

As well as alerting funders, partners and Steering Group members are encouraged to publish the findings of the Neurodevelopmental Disorders PSP using both internal and external communication mechanisms. The Steering Group may capture and publicize the results through descriptive reports of the process itself in Plain

¹ Steering Group members should insert information on how they intend to do this.
James Lind Alliance: Priority Setting Partnership Protocol – April 2016

English. This exercise will be distinct from the production of an academic paper. However, production of an academic paper should not take precedence over publicizing of the final results.

7. Agreement of the Steering Group

Signed by the Steering Group

The undersigned agree to follow the Neurodevelopmental Disorders Priority Setting Protocol.

Evdokia Anagnostou (Steering Committee Chair), Holland Bloorview Kids Rehabilitation Hospital

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Date:

Brendan Andrade, Centre for Addiction and Mental Health

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Date:

Susan Lynch, Community Living Toronto

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Date:

James Mitchell, ADHD Patient Advocate

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Date:

Rob Nicolson, Children's Hospital at London Health Sciences Centre

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Date:

Margaret Spoelstra, Autism Ontario

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Date:

Carla Arasanz, Ontario Brain Institute

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Date:

Jessica Jordao, Ontario Brain Institute

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Date:

Katherine Cowan, The James Lind Alliance

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Date:

APPENDIX 1 - WAYS OF THINKING ABOUT HEALTH

The following are 'components of health' as defined by the World Health Organization in the International Classification of Functioning Disability and Health.

BODY FUNCTIONS & STRUCTURES

- Mental functions
- Nervous system
- Sensory functions & pain
- Ear & eye
- Voice and speech
- Cardiovascular, haematological, immunological and respiratory systems
- Immunological & respiratory systems
- Digestive, metabolic & endocrine systems
- Genitourinary & reproductive systems
- Neuromusculoskeletal & movement
- Skin & related structures
- Movement

ACTIVITY & PARTICIPATION

- General tasks & demands
- Communication
- Mobility
- Self care
- Domestic life
- Interpersonal interactions & relationships
- Major life areas
- Community, social & civic life
- Any other activity & participation

These 'components of health' are described in a little more detail on the following pages.

BODY FUNCTIONS & STRUCTURES

Mental functions - both global mental functions, such as consciousness, energy and drive, and specific mental functions, such as memory, language and calculation mental functions

Nervous system – brain, spinal cord and nervous system

Ear & eye structures, sensory functions and pain - senses, seeing, hearing, tasting and so on, as well as the sensation of pain.

Voice & speech - functions of producing sounds and speech

Cardiovascular, haematological, immunological systems - functions involved in the cardiovascular system (functions of the heart and blood vessels), the haematological and immunological systems (functions of blood production and immunity), and the respiratory system (functions of respiration and exercise tolerance).

Immunological & respiratory systems - functions involved in the cardiovascular system (functions of the heart and blood vessels), the haematological and immunological systems (functions of blood production and immunity), and the respiratory system (functions of respiration and exercise tolerance).

Digestive, metabolic & endocrine systems - functions of ingestion, digestion and elimination, as well as functions involved in metabolism and the endocrine glands and the growth maintenance functions

Genitourinary & reproductive systems - functions of urination and the reproductive functions, including sexual and procreative functions.

Neuromusculoskeletal & movement - functions of movement and mobility, including functions of joints, bones, reflexes and muscles.

Skin & related structures - functions of skin, nails and hair

Movement - moving by changing body position or location or by transferring from one place to another, gait functions including walking, running or climbing; also carrying or manipulating objects.

ACTIVITY & PARTICIPATION

General tasks and demands - carrying out single or multiple tasks, organizing routines and handling stress. These items can be used in conjunction with more specific tasks or actions to identify the underlying features of the execution of tasks under different circumstances.

Communication - general and specific features of communicating by language, signs and symbols, including receiving and producing messages, carrying on conversations, and using communication devices and techniques.

Mobility - moving by changing body position or location or by transferring from one place to another, by carrying, moving or manipulating objects, by walking, running or climbing, and by using various forms of transportation.

Self care - caring for oneself, washing and drying oneself, caring for one's body and body parts, dressing, eating and drinking, and looking after one's health.

Domestic life - carrying out domestic and everyday actions and tasks. Areas of domestic life include acquiring a place to live, food, clothing and other necessities, household cleaning and repairing, caring for personal and other household objects, and assisting others

Interpersonal interactions & relationships - carrying out the actions and tasks required for basic and complex interactions with people (strangers, friends, relatives, family members and lovers) in a contextually and socially appropriate manner.

Major life areas - carrying out the tasks and actions required to engage in education, work and employment and to conduct economic transactions.

Community, social & civic life - the actions and tasks required to engage in organized social life outside the family, in community, social and civic areas of life.