

## Liver Glycogen Storage Disease Priority Setting Partnership

PROTOCOL 19<sup>th</sup> of July 2017<sup>1</sup>

### 1. Purpose of the PSP and background

The purpose of this protocol is to set out the aims, objectives and commitments of the International Liver Glycogen Storage Disease (IGSD) Priority Setting Partnership (PSP) and the basic roles and responsibilities of the partners therein. It is recommended that the Protocol is reviewed by the Steering Group and updated on at least a quarterly basis.

The James Lind Alliance (JLA) is a UK-based non-profit making initiative, established in 2004. It brings patients, carers and clinicians together in Priority Setting Partnerships (PSPs). These partnerships identify and prioritise uncertainties, or 'unanswered questions', about the effects of care and management that they agree are the most important. The aim of this is to help ensure that those who fund health research are aware of what really matters to both patients and clinicians. The UK's National Institute for Health Research (NIHR – [www.nihr.ac.uk](http://www.nihr.ac.uk)) funds the infrastructure of the JLA to oversee the processes for priority setting partnerships, based at the NIHR Evaluation, Trials and Studies Coordinating Centre (NETSCC), University of Southampton.

The IGSD PSP is an initiative from Terry Derks, MD, PhD, a consultant in paediatric metabolic medicine. Liver GSD is a rare metabolic disease for which international collaboration is necessary to achieve more knowledge for treating these patients. 15-17 June 2017, the 4<sup>th</sup> International Conference on Glycogen Storage Diseases will be held in Groningen. This is an opportunity to give a platform to physicians, dieticians and patients to come together and discuss management and care of Liver GSD.

To prioritise further research goals, the JLA was contacted to set up an international Priority Setting Partnership to uncover and report these shared research goals.

### 2. Aims and objectives of the Liver Glycogen Storage Disease PSP

The aim of the IGSD PSP is to identify the unanswered questions about Liver GSD management and care from patient and clinical perspectives and then prioritise those that patients and clinicians agree are the most important.

The objectives of the IGSD PSP are to:

- work with patients and clinicians internationally to identify uncertainties about the management and care of Liver GSD
- 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 international research commissioning bodies to be considered for funding.

### 3. The Steering Group

Name	Country of residence	Healthcare professional	Representative of patient organization	Caretaker of patient	Role
Terry Derks	The Netherlands	Physician (Pediatrician Metabolic Diseases)			PSP-lead
Katherine Cowan	UK				JLA Adviser
Fabian Peeks	The Netherlands	MD-PhD Student			Information Specialist
Elma Daanje	The Netherlands				PSP Project Co-ordinator
Lut de Baere	Belgium		X		PSP-steering group member
Ute Stachelhaus-Theimer*	Germany	Physician (Internal Medicine)	X	X	PSP-steering group member
Nerea López Maldonado*	Spain	Physician	X	X	PSP-steering group member
Niccòlo Seidita	Italy		X	X	PSP-steering group member
Carina Heidenborg	Sweden	Dietician			PSP-steering group member
Antal Nemeth	Sweden	Physician (Pediatric Gastro-enterologist)		X	PSP-steering group member
Thomas Casswall	Sweden	Physician (Pediatric Gastro-enterologist)			PSP-steering group member
Marcus Landgren*	Sweden	Physician (Orthopedic Surgeon)	X	X	PSP-steering group member
Damian Cohen*	Argentina	Physician	X	X	PSP-steering group member
Alberto Ferriani	Brasil		X	X	PSP-steering group member
David Weinstein	USA	Physician (Pediatric Endocrinologist)			PSP-steering group member
Iris Ferrechia*	USA	Nurse	X	X	PSP-steering group member

The Steering Group includes representation of patient/carer groups and clinicians<sup>2</sup>.

The Steering Group will agree 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

Organisations 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 management and care of Liver GSDs. Partners represent the following groups:

- People who have Liver GSD.
- Carers of people who have Liver GSD.
- Health care professionals (including medical doctors, nurses and dietitians) with clinical experience of Liver GSD.

Partners will be identified from across the world. It is important that all organisations which can reach and advocate for these groups should be invited to become involved in the PSP. The JLA Adviser will take responsibility for ensuring the various stakeholder groups are able to contribute equally to the process.

### Exclusion criteria

Some organisations may be judged by the JLA or the Steering Group to have conflicts of interest. These may be perceived to adversely affect those organisations' views, causing unacceptable bias. 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 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](http://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

First, potential partner organisations have been identified through a process of peer knowledge and consultation, through the Steering Group members' international networks. Potential partners have been contacted and informed of the establishment and aims of the Liver GSD PSP and may be invited to attend and participate in the initial stakeholder meeting during the International GSD conference in Groningen, the Netherlands 15-17 June 2017 (IGSD2017).

### Step 2: Initial awareness meeting

The initial stakeholder meeting / awareness raising will have several key objectives:

- to welcome and introduce potential members of the Liver GSD PSP
- to present the proposed plan for the PSP
- to initiate discussion, answer questions and address concerns
- to identify those potential partner organisations which will commit to the PSP and identify individuals who will be those organisations' representatives and the PSP's principal contacts
- to establish principles upon which an open, inclusive and transparent mechanism can be based for contributing to, reporting and recording the work and progress of the PSP.

Furthermore, awareness will be raised for the Liver GSD PSP before and during IGSD2017. On the 16<sup>th</sup> of June, the JLA Advisor Katherine Cowan will provide a plenary lecture.

### **Step 3: Identifying uncertainties**

Each partner will identify a method for soliciting from its members' questions and uncertainties of practical clinical importance relating to the management and care of patients with Liver GSD. A period of approximately 3 months will be given to complete this exercise.

Given the international nature of the Liver GSD PSP, the methods will be designed accordingly. The Steering Group Meetings will be held during international conferences. The First Steering Group Meeting will be held during the IGSD2017, Groningen, The Netherlands, 15-17 June 2017. The intermediate meeting will be held during the SAGSD conference in Ängelholm, Sweden, 28-29 April 2018. The Final PSP meeting will be held either during the SSIEM 2019 in Rotterdam, the Netherlands, September 2019 or the next International GSD meeting.

Furthermore, the survey will be translated into English, German, French, Portuguese, Spanish and Dutch by various health care workers or patient representatives and will be spread through different patient organizations and the networks of participating health care professionals.

Existing sources of information about 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 Pubmed:  
<https://www.ncbi.nlm.nih.gov>

### **Step 4: 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 care and management of Liver GSDs. These raw questions will be assembled and categorised and refined by Fabian Peeks, the Information Specialist, 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 Fabian Peeks, 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 management and care 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.<sup>3</sup>

Uncertainties which are not adequately addressed by previous research will be collated and recorded on a template supplied by the JLA) by Fabian Peeks, 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 personnel and resources to carry this accountability. 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, to ensure that PSP results are publicly available.

### **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 relating to the management and care of Liver GSD. 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), will take place at the SAGSD in Ängelholm, Sweden, 28-29 April 2018. In this meeting, organisations 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](http://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 prioritised uncertainties, will take place during either the SSIEM in Rotterdam, The Netherlands, September 2019, or at the next International GSD meeting.
- The methods used for this prioritisation process will be determined by consultation with the partner organisations 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 Liver GSD PSP will be reported to funding and research agenda setting organisations such as the NIHR and the major research funding charities. Steering Group members and partners are expected to develop the prioritised 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.<sup>4</sup>

### Publicity

As well as alerting funders, partners and Steering Group members are encouraged to publish the findings of the Liver GSD PSP using both internal and external communication mechanisms. The Steering Group may capture and publicise the results through descriptive reports of the process itself in Plain English. This exercise will be distinct from the production of an academic paper, which the partners are also encouraged to do. However, production of an academic paper should not take precedence over publicising of the final results.

## 7. Agreement of the Steering Group

The Steering Group agreed approved this protocol on 16<sup>th</sup> of June 2017 during the First Steering Group Meeting during the International GSD Conference 2017.

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<sup>4</sup> Add further detail here about how and where the priorities will be developed and researched.