

Autosomal Dominant Polycystic Kidney Disease (ADPKD) Priority Setting Partnership

SCOPE

1. Purpose of the PSP and background

The purpose of this scope is to clearly set out the aims, objectives and commitments of the Autosomal Dominant Polycystic Kidney Disease or ADPKD Priority Setting Partnership (PSP) in line with James Lind Alliance (JLA) principles. The Scope is a JLA requirement and will be published on the PSP's page of the JLA website. The Steering Group will review the Scope 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 PKD Charity UK (PKDC) initiated the ADPKD PSP. The PKDC funds 'patient-led' research and has always sought to incorporate the needs of patients, their family members and carers. The PKDC has a Research Advisory Board whose members share those values. Having heard about the benefits of PSPs in other conditions and from experience of partnership on two other PSPs, the PKDC Trustees approved a budget to carry out an ADPKD PSP in the UK.

2. Aims, objectives and scope of the PSP

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

The scope of this PSP differs from the 'traditional' PSP model in that the 'gathering uncertainties' stage will be omitted. When scoping the PSP, it was agreed that sufficient uncertainties had already been gathered during the ADPKD Controversies Conference organised by KDIGO (Kidney Disease: Improving Global Outcomes), held in January 2014.

A global patient/carer group – co-chaired by the PKDC CEO - was invited to participate equally in the KDIGO Conference alongside health professionals. The Conference organisers published a table listing 'gaps in knowledge and a research agenda in ADPKD' (Table 5 in the Conference report). The Conference report was published in *Kidney International* (2015) DOI: <https://doi.org/10.1038/ki.2015.59> and the full report can be accessed here: <https://kdigo.org/conferences/adpkd/>.

The ADPKD PSP will build on the output of the KDIGO Consensus (2014) (specifically Table 5) and two further reviews published after KDIGO. The Cochrane review (2015) of 'Interventions for preventing the

progression of autosomal dominant polycystic kidney disease' concluded that 'although several interventions are available for patients with ADPKD, at present there is little or no evidence that treatment improves patient outcomes in this population and is associated with frequent adverse effects. Additional large randomised studies focused on patient-centred outcomes are needed'.

'Autosomal dominant polycystic kidney disease', published in The Lancet online February 25, 2019, [http://dx.doi.org/10.1016/S0140-6736\(18\)32782-X](http://dx.doi.org/10.1016/S0140-6736(18)32782-X), concluded that 'unresolved questions remain'. These questions were also amongst those listed in the KDIGO Report Table 5.

The ADPKD PSP is to be developed and published therefore 'In Association with the James Lind Alliance'.

The objectives of the PSP are:

- to carry forward and work with patients, carers, and clinicians to rank and prioritise the uncertainties that have been identified about the ADPKD care pathway.
- 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 ADPKD PSP is defined as:

Research and health-care priorities related to: diagnosis and management of renal and extra-renal complications; identification of new treatments to prevent and slow PKD progression; and practical integrated patient support.

The PSP will exclude from its scope questions about:

- Autosomal Recessive Polycystic Kidney Disease or ARPKD

Other considerations:

- Children under the age of 16 affected by ADPKD will be considered in the PSP but not actively consulted.
- The PSP will be focused on the UK and the survey will be in English language; however, it is recognised that international participants will take part and their priorities will be separated and shared with overseas PKD groups.
- The final workshop will be held in 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 clinicians¹, as individuals or representatives from a relevant group.

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

Patients and carers or representative/s:

Mr Patrick Barton	Carer
Mrs Natasha O'Brien	Patient
Mrs Gillian Mundy	Patient
Mr Peter Storey	Kidney Research UK representative

Health professionals:

Nurse Specialist Wendy Brown	Imperial College Healthcare Trust	Steering Group member
Dr Ragada El-Damanawi	Addenbrooke's Hospital (adult nephrologist and clinical research fellow)	Steering Group member
Dr Bert Jindal	GP	Steering Group member
Dr Maryam Khosravi	Royal Free Hospital (adult nephrologist)	Steering Group member
Professor Albert Ong	University of Sheffield (adult nephrology)	Steering Group member
Dr Albert Power	Southmead Hospital Bristol (adult nephrology)	Steering Group member
Dr Richard Sandford	Addenbrooke's Hospital (genetics)	Steering Group member
Professor John Sayer	Institute of Genetic Medicine Newcastle (adult nephrology/genetics)	Steering Group member
Dr Roz Simms	Sheffield Teaching Hospitals (adult nephrologist)	Steering Group member
Professor Pat Wilson	PKD Charity Research Advisory Board & UCL Centre for Nephrology	Steering Group member
Professor Paul Winyard	UCL Great Ormond Street Institute of Child Health (paediatric nephrology)	Steering Group member
Dr Grahame Wood	Salford Royal Hospital (adult nephrology)	Steering Group member

Project coordinator:

Ms Tess Harris	PKD Charity	PSP Lead, Steering Group member (and patient)
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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.

Mrs Jane Pugh	PKD Charity	PSP Project Manager, Steering Group member (and patient)
Maryrose Tarpey	James Lind Alliance Adviser	Chair of the Steering Group
Ann Daly	Independent Information Specialist	Steering Group member

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 *[this section will be updated as partners are confirmed]*. 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 with ADPKD
- carers of people with ADPKD
- health professionals - with experience of ADPKD.

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. However, 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 ADPKD PSP.

Step 2: Awareness raising

The Steering Group will advise on how to raise awareness of the PSP among their patient, carer and clinician communities, in order to secure support and participation. Awareness raising has several objectives including:

- to generate support for the process
- to encourage participation in the process

Step 3: Evidence checking

Existing evidence will be checked to determine whether any uncertainties have been answered by research.

This will be done by Ann Daly. 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 initial evidence check will be on the 'unresolved questions' as referenced in Section 2 above. Questions that are still not adequately addressed by research will be collated and recorded on a standard JLA template by Ann Daly. 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.

Step 4: Refining questions and uncertainties

The uncertainties will be summarised by Ann Daly into indicative questions which are clear, addressable by research, and understandable to all. The Steering Group will have oversight of this process to ensure that the raw data is being interpreted appropriately and that the indicative questions are being worded in a way that is understandable to all audiences. The JLA Adviser will observe to ensure accountability and transparency.

The indicative questions are not research questions - as this 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.

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 ADPKD. 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 by questionnaire. 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.

The Steering Group will also consider how it will collate and report on any additional open-ended comments/questions provided by respondents to the survey, which for example may already have been answered or are out of scope.

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 Tess Harris.

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 ADPKD PSP Steering Group agreed the content and direction of this Scope on 21 October 2019.