

James Lind Alliance
Priority Setting Partnership
Paediatric Lower Limb Surgery

Summary

This partnership brought together children, parents/carers and health professionals aiming to identify and prioritise research questions on the orthopaedic management of lower limb conditions (hip to toe) in children aged 0-16 years. A Priority Setting Partnership (PSP) was set up using prospective surveys and consensus meetings following the James Lind Alliance methodology.

The process took eighteen months (July 2017-January 2019), involved 388 people, generated 1023 questions, and a total of 801 research questions were classified as true uncertainties (in scope questions). 234 individuals participated in the interim prioritisation survey selecting the most important questions from a group of 75 uncertainties. 30 individuals attended the final face-to-face workshop and ranked the top priorities representing the objectives of the Paediatric Lower Limb Surgery Priority Setting Partnership.

This is the first James Lind Alliance priority setting partnership in children's orthopaedic surgery, a particularly under-researched and under-funded area. We hope it will represent an invaluable resource to guide researchers and funders into future paediatric orthopaedic research.

The top 10 priorities

Our top-10 research priorities:

- 1** What are the best ways to measure the outcome following lower limb orthopaedic surgery in children?
- 2** Following orthopaedic surgery to the lower limbs, what should children's rehabilitation include, how long is it expected to last and how does it affect the result of treatment?
- 3** What is the short-term and long-term clinical and cost effectiveness of orthopaedic lower limb surgery (including best timing and technique) for children with Cerebral Palsy who can walk?
- 4** What are the short-term and long-term outcomes of surgery compared to non-surgical care in the treatment of Perthes' disease of the hip?
- 5** What is the clinical and cost effectiveness of pre-operative rehabilitation in children presenting with lower limb orthopaedic conditions?
- 6** What is the short-term and long-term clinical and cost effectiveness of Selective Dorsal Rhizotomy (SDR) in children with Cerebral Palsy who can walk?
- 7** Can surveillance and non-surgical treatment (e.g. physiotherapy, botulinum toxin injections, functional electrical stimulation, orthotics, casting) prevent the development of deformity requiring surgery in children with Cerebral Palsy?
- 8** What is the best method of screening for Developmental Dysplasia of the Hip (DDH) in terms of clinical and cost effectiveness?
- 9** What are the best strategies to optimise communication of information between patients/carers and clinicians in order to enable shared decision-making?
- 10** What is the best management for hip displacement in children with Cerebral Palsy?

Foreword

A.

On behalf of the Steering Group of the Paediatric Lower Limb Surgery JLA Partnership I would like to thank the participants representing the children, their families and carers and the charities who made this project possible. Further to that, I am grateful to everyone who responded to the surveys through their professional organisations.

We are particularly grateful to JLA Adviser, Patricia Ellis, for her guidance and advice throughout the project. Her calm, professional attitude and firm adherence to the JLA methodology helped complete the project to a high standard in a timely manner. I would also wish to thank her colleagues, Katherine Cowan and Toto Gronlund, who assisted her in the smooth running of the final prioritisation meeting.

The steering group was strongly supported by Camille Rougelot, administrator of the project and Martinique Vella-Baldacchino a trainee surgeon who undertook the data management and literature reviews for the project. I am very grateful to both for their enthusiasm and hard work.

This project would not have been possible without adequate funding. I am grateful to the British Society for Children's Orthopaedic Surgery, the Nuffield Department of Orthopaedics, Rheumatology and Musculoskeletal Science, the Oxford Biomedical Research Centre and the British Orthopaedic Association who all contributed to the funding of this work.

Tim Theologis

Consultant Orthopaedic Surgeon and PSP Lead

On behalf of the JLA Paediatric Lower Limb Surgery Partnership

B.

In our capacity representing patients and their parents and carers on the steering committee of this JLA partnership we were given the opportunity to advocate for the interests and concerns of those at the receiving end of lower limb paediatric orthopaedic surgery. The JLA methodology is specifically designed to ensure the active participation of all stakeholders. In this context our views, along with those of

the other patients and carers who participated in the surveys, steering committee, and prioritisation workshop, made a significant contribution to the formulation of the questions and their final ranking. Debates over certain questions and the relative priority of the different diagnoses represented by participants, both clinicians and patients/parents, were lively and sometimes passionate. However, the experienced guidance of Patricia Ellis and her JLA colleagues ensured that everyone's voice was heard. Throughout the process both Patricia and our professional colleagues made sure we understood some of the medical/complicated terms and references making sure we were fully informed of the meaning of the questions and content. We were also impressed by how willing parents/patients were to respond to our surveys, understanding the importance of this piece of work and how they eagerly awaited the outcome and final questions.

By the end of the final workshop it was generally agreed that we had settled upon a ranking of research priorities, which reflected a good compromise between the interests of all involved. Given the importance of the concerns of patients and their carers in determining what counts as successful and appropriate treatment, and the relatively few opportunities for us to influence the direction of research, the JLA partnership provided us with an invaluable forum by which to do so.

Helen Gregory-Osborne and Daniel Dolley

*Steering Group Members of the JLA Paediatric Lower Limb Surgery Partnership
Parent and Patient Representatives*

The Team at work: Final Prioritisation Meeting, Oxford 17th November 2018

Photographs from the workshop are reproduced with the kind permission of all participants.



Introduction and background

James Lind was a Scottish naval surgeon who enrolled individuals onto the first clinical trial in an attempt to establish a treatment for scurvy, succeeding and publishing his discovery in 1753. The James Lind Alliance (JLA) was created to offer the opportunity to patients and members of the public to have an equal voice to clinicians and researchers in influencing the research agenda. The JLA's infrastructure is funded by the National Institute for Health Research (NIHR) and oversees the overall process in a transparent and structured manner.

Musculoskeletal (bone and joint) symptoms are the primary reason for referral to outpatient paediatric clinics. Each year, one in eight children visits the doctor for a musculoskeletal disorder, some of which are responsible for long-term impairment and disability. Treatment options offered depend on the effect on children's future growth and development, therefore the outcome is often unclear due to the lack of good quality research and long-term studies.

The JLA has provided a platform to explore and identify the most pertinent uncertainties, which affect children aged 0-16 years presenting with bone and joint conditions affecting the lower limb. This will help establish a research agenda in this field of clinical practice. The orthopaedic surgical practice in children is not based on good quality evidence. Poor evidence has led to significant variation in surgical practices nationally and internationally. This variation has resulted in conflicting information and loss of confidence in treatment pathways and in the clinicians that deliver them. In 2017, the British Society of Children's Orthopaedic Surgery (BSCOS), the British Orthopaedic Association (BOA) and the Oxford Biomedical Research Centre recognised the importance of establishing research priorities and jointly agreed to fund this partnership.

Whilst setting up this partnership, it became clear that there is considerable common ground in paediatric orthopaedic conditions that affect the lower limb(s): deformity, joint stiffness, pain and impaired mobility are the usual problems associated with these conditions. Examples include developmental dysplasia of the hip, clubfoot, congenital limb deficiencies, neuromuscular conditions and bone dysplasias. Although the aetiology of these conditions varies considerably, the clinical problems and the resulting motor disability are similar and this is what matters from the patient/family perspective. Therefore, the clinical subject of this work was chosen to

include the orthopaedic treatment of lower limb conditions in children. Trauma to the lower limb was excluded, as this usually causes short-term disability and rarely the long-term problems related to orthopaedic conditions of the lower limb. Upper limb orthopaedic or traumatic conditions also cause a different type of disability and were not included in the scope of this project.

The aim of the Paediatric Lower Limb Surgery (PLLS) Priority Setting Partnership (PSP) was to identify the unanswered questions on elective lower limb orthopaedic surgery in the paediatric population by:

- working with patients, carers and clinicians to identify uncertainties about the effects of lower limb orthopaedic surgery in children.
- agreeing by consensus on a top-10 prioritised list of those uncertainties, for research
- publicising the results of the PSP and process and presenting these results to research commissioning bodies to be considered for funding.

The Steering Group

The James Lind Alliance has developed a step-by-step guide outlining the tested methods for PSPs to work effectively and reach credible and useful outcomes. In order for the JLA to fully endorse the final top 10 research priorities, the founding principles of patient and clinician involvement, transparency and systematic rigour must be respected. A JLA Adviser (Patricia Ellis) was appointed to support and guide the PSP setting process whilst liaising with the clinical specialist lead, Tim Theologis. The steering group consisted of:

- **Patient/Parent Charity** representatives (Loredana Guetg-Wyatt, Managing Director of Steps Charity Worldwide, Emma Morley, Research and Information Officer for Steps),
- **Parents** (Helen Gregory-Osborne, Catherine Ann Greaves),
- **Patient** (Daniel Dolley)
- **Paediatric orthopaedic surgeons** (Andreas Roposch, Nick Nicolaou, Steve Cooke, Dan Perry),
- **A surgical trainee** and information specialist (Martinique Vella-Baldacchino),
- **Physiotherapists** (Christine Douglas, Catherine Barry),
- **Clinical Nurse Specialist** (Craig Walsh)

- **Advanced Nurse Practitioner** (Elizabeth Wright)
- **The JLA Adviser** (Patricia Ellis)
- **Administrative support** (Camille Rougelot)
- **PSP Lead** (Tim Theologis).

Details of the steering group members can be found on the Partnership website (<https://www.ndorms.ox.ac.uk/research-groups/paediatric-lower-limb-surgery>).

The Process

Partner organisations

Partner organisations were identified through a process of peer knowledge and through the steering group members' networks. The organisations were invited to participate via a communication package describing the JLA Paediatric Lower Limb Surgery PSP objectives and process. Partners were firstly asked to provide their views and feedback on the proposed protocol.

Organisations represented paediatric orthopaedic patients and their families or carers as well as relevant healthcare professionals, including medical doctors, nurses, physiotherapists and other allied health professionals with clinical experience of paediatric orthopaedic surgery to the lower limbs. Children and young people under the age of 16 years were encouraged to voice their views separately to those of their parents. Parents were asked to encourage their children to fill in a separate survey form. Partner organisations invited to participate in the project are presented in Appendix A.

Identifying treatment uncertainties

An online survey, agreed by the Steering Group, was set up and the link distributed to partner organisations. These organisations were encouraged to freely distribute the survey link to solicit research questions and uncertainties from their members. The steering group further encouraged the submission of questions from a broad array of individuals from across society using a variety of media, including newsletters, internet message boards and postal questionnaires. The link for the survey was available through the PLLS PSP website. Printed copies of the questionnaire were made available, the data was then entered into the online survey. The Steering group monitored responses to the survey and under-represented groups were targeted whilst the survey was live (January 2018 – March 2018).

Example of survey questionnaire for children

Are you thinking about having an operation on your legs, hips or feet? Can you help us?

There is still a lot that we don't know about helping children who have problems with the bones and joints in their legs, hips and feet.

If you have any questions like the ones below, then we need you to get your *thinking caps on!*

When should the operation be done? Do I need to have an operation at all? What kind of operation? How do the Doctors help me get better?

You can write your questions here, or ask a grown up to write them onto our questionnaire, or even take a picture and to send with it to :
Camille.rougelot@ndorms.ox.ac.uk, or Twitter page for the JLA project @PLLS_JLA_PSP

Your Questions:

This project is sponsored by the James Lind Alliance and co-funded by the British Society for Children's Orthopaedic Surgery, the British Orthopaedic Association and the Oxford University Biomedical Research Centre

Refining questions and uncertainties

The raw questions collected during the initial survey were organised into broad lower limb condition categories. Questions, which did not relate to the JLA PLLS objectives were excluded and labeled as 'Out of Scope'. The in-scope raw questions were then further grouped under general indicative questions. The in-scope questions were then searched using evidence published by the National Institute for Health and Care Excellence (NICE), Cochrane Library, systematic reviews and randomized control

trials (Level I and Level II). Evidence was only included if published in the English language and over the past 10 years. A question was confirmed as a genuine uncertainty if it could not be answered using the literature search method above. Several topic experts in various fields were consulted to finalise decisions.

In-scope questions which could be resolved with reference to existing literature, the ‘unrecognised knowns’, were identified and listed. This was then passed on to respective partners within the steering group in order to communicate the information to the respective patients groups. By merging and grouping the remaining true uncertainties on similar themes together, the final number of research uncertainties was reduced to 75. These 75 questions were agreed by the Steering Group and were entered into the next stage of prioritisation. A list of the 75 questions can be found at <http://www.jla.nihr.ac.uk/priority-setting-partnerships/Paediatric-lower-limb-surgery/>.

Prioritisation – interim stage

The long list of 75 questions was reduced to a shorter list by a further online survey of the same partners, whereby respondents (a mixture of patients, parents and health care professionals) were asked to identify the 10 most important questions. Participants were asked to submit their preferences between August 2018 – September 2018. The Steering Group reviewed the results of the second survey and agreed the top 26 questions to be taken to the final prioritisation meeting. This followed the James Lind Alliance recommendation to select approximately 25 questions for the final prioritisation. Care was taken to adequately represent the top choices of children, parents/carers and professional groups.

Prioritisation – final prioritisation workshop

The top 26 questions were taken to the final face-to-face workshop. Thirty individuals, representing children, parents/carers and professional groups, were invited. The meeting took place at Worcester College in Oxford.



Three independent JLA facilitators ensured that each individual had their views represented during the prioritisation workshop. Following the JLA methodology, the top 10 research priorities were selected.

The whole prioritisation process is summarised in Appendix B. The full list of the 26 questions included in the final prioritization is presented in Appendix C.

The Top 10 priorities

This JLA process has identified the top 10 unanswered research priorities in paediatric lower limb orthopaedics. These questions require appropriate attention to enable large scale research that will definitively address these uncertainties. The notable engagement of professionals, patients and the public, will ensure that the questions have a broad-reach in terms of real-world impact.

The number one priority was to identify the best way to measure outcomes following lower limb paediatric orthopaedic surgery. This highlights the importance of developing high-quality tools to be used in research to process, and make informed decisions about clinical effectiveness.

Four of the top-10 priorities were directly related to the management of children with Cerebral Palsy. This is not surprising as children with cerebral palsy often undergo orthopaedic interventions. The important hip conditions of childhood (Perthes disease, Developmental Dysplasia of the Hip), as well as rehabilitation techniques and methods to improve shared decision making between clinicians and patients/families, all contributed to the top 10 list (please see below).

| | |
|----------|---|
| 1 | What are the best ways to measure outcome following lower limb orthopaedic surgery in children? |
| 2 | What should children's rehabilitation following orthopaedic surgery to the lower limbs include, how long is it expected to last and how does it affect the result of treatment? |
| 3 | What is the short-term and long-term clinical and cost effectiveness of orthopaedic lower limb surgery for children with Cerebral Palsy who can walk (considering best timing and technique)? |
| 4 | What are the short term and long term outcomes of surgery compared to non-surgical care in the treatment of Perthes disease? |
| 5 | What is the role of pre-operative rehabilitation in children presenting with lower limb orthopaedic conditions? |
| 6 | What is the short-term and long-term clinical and cost effectiveness of Selective |

| | |
|-----------|--|
| | Dorsal Rhizotomy (SDR) in children with Cerebral Palsy who can walk? |
| 7 | Can surveillance and non-surgical treatment (physiotherapy, botulinum toxin injections, functional electrical stimulation, orthotics, casting) prevent the development of deformity requiring surgery in children with Cerebral Palsy? |
| 8 | What is the best method of screening for Developmental Dysplasia of the Hip (DDH) in terms of clinical and cost effectiveness? |
| 9 | What are the best strategies to optimise communication of information between patients/carers and clinicians in order to enable shared decision making ? |
| 10 | What is the best management for hip displacement in children with Cerebral Palsy? |

Out of Scope questions

As part of the dissemination process, the Steering Group decided to distribute the out-of-scope questions to all Partner organisations that contributed to this project. These questions could give rise to future JLA projects. They will also alert our Partners to other themes and questions that children and families have. This may be helped by future research or simply by information campaigns.

The full list of out of scope questions can be found here:

<http://www.jla.nihr.ac.uk/priority-setting-partnerships/Paediatric-lower-limb-surgery/>

Next steps

The steering group agreed on a strategy, which would include dissemination of results through the established networks of partners, using a variety of media such as an infographic, project report, conference presentations, online social media platforms and the use of video clips.

Future research arising from this project must be high quality in order to provide definitive answers to these research priorities. Appropriate study design and an adequately formulated research question, following the EQUATOR network and IDEAL guidelines will shape the future of paediatric orthopaedic research.

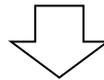
The results of the lower limb paediatric JLA PSP were presented at the British Society for Children's Orthopaedic Surgery annual conference on 7 March 2019. Results are now being disseminated at national and international conferences, social media and a formal publication. The findings will be reported to funding research organisations such as the NIHR, Medical Research Council and major research funding charities.

Appendix A – Partner organisations that contributed to the Surveys and their links with the Steering Group

| | |
|-----------------------|--|
| Loredana Guetg-Wyatt | Limb power (http://limbpower.com) |
| Emma Morley | Bone Cancer Research Trust (http://www.bcrct.org.uk) |
| Loredana Guetg-Wyatt | Meningitis Research Foundation (https://www.meningitis.org) |
| Helen Gregory-Osborne | SCOPE (https://www.scope.org.uk) |
| | Action Cerebral Palsy (https://www.actioncp.org) |
| Tim Theologis | British Society for Children's Orthopaedic Surgery - BSCOS (https://bscos.org.uk) |
| Nicolas Nicolaou | British Limb Reconstruction Society – BLRS (http://blrs.org.uk) |
| Daniel Perry | British Orthopaedic Association Research Committee (https://www.boa.ac.uk/committee/research/) |
| Craig Walsh | Royal College of Nursing (https://www.rcn.org.uk) |
| Christine Douglas | Royal College of Occupational Therapists (https://www.rcot.co.uk) |
| Stephen Cooke | General Practitioners (Alison Marsh – research programme manager - Alison.Marsh@rcgp.org.uk) |

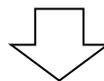
Appendix B – Summary of Prioritisation Process

Survey (January 2018 – March 2018)
388 individuals submitted 1023 questions
47% individuals were patients, parents or carers



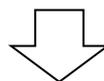
Organising and identifying uncertainties (March 2018 – July 2018)

801 questions classified as in-scope
222 questions were out of scope
75 indicative research questions were generated, all of which were confirmed uncertainties following literature searches



Interim prioritisation online survey (August 2018 – September 2018)

234 individuals selected their top 10 questions from the 75 indicative questions



Final Prioritisation face-to-face workshop (November 2018)

Attended by 30 individuals (6 physiotherapists, 9 parents, 4 patients, 7 orthopaedic surgeons, 1 clinical scientist, 1 advanced nurse practitioner and 2 charity representatives)

Participants asked to rank the top 26 questions from the interim prioritisation survey.

Appendix C – The 26 questions discussed at the final workshop

1. What are the short term and long term outcomes of surgery compared to non-surgical care in the treatment of Perthes disease?
2. What are the best ways to measure outcome following lower limb orthopaedic surgery in children?
3. Which operation leads to best results in the treatment of Perthes disease?
4. Does vitamin D supplementation or other diet supplements increase recovery rates following lower limb bone surgery, such as osteotomy or leg lengthening?
5. Can surveillance and non-surgical treatment (physiotherapy, botulinum toxin injections, functional electrical stimulation, orthotics, casting) prevent the development of deformity requiring surgery in children with Cerebral Palsy?
6. What is the outcome of hip replacement in a child compared to hip reconstruction. (eg. functionality, how long it lasts, how it is affected by age and underlying diagnosis) ?
7. What are the best strategies to optimise communication of information between patients/carers and clinicians in order to enable shared decision making ?
8. What is the short-term and long-term clinical and cost effectiveness of orthopaedic lower limb surgery for children with Cerebral Palsy who can walk (including best timing and technique)?
9. What are the long-term outcomes of treatment in Developmental Dysplasia of the Hip (DDH) presenting late?
10. What is the optimal management for severe stable Slipped Upper Femoral Epiphysis (SUFE)?
11. What is the short-term and long-term clinical and cost effectiveness of Selective Dorsal Rhizotomy (SDR) in children with Cerebral Palsy who can walk?
12. What is the best management for hip displacement in children with Cerebral Palsy?
13. What is the best method of screening for Developmental Dysplasia of the Hip (DDH) in terms of clinical and cost effectiveness?
14. What is the optimal surgical treatment and timing of surgery for unstable Slipped Upper Femoral Epiphysis (SUFE)?
15. What should children's rehabilitation following orthopaedic surgery to the lower limbs include, how long is it expected to last and how does it affect the result of treatment?
16. What are the most effective interventions (including type and timing of the procedure) for Developmental Dysplasia of the Hip (DDH) presenting late?
17. What is the role of pre-operative rehabilitation in children presenting with lower limb orthopaedic conditions?
18. What are the indications for surgical treatment of flatfeet (including implants) and what are the long-term results of surgery?
19. Does gait analysis alter surgical decision making in ambulant children with Cerebral Palsy?
20. Why is there geographical variation in the management of lower limb orthopaedic conditions in children?
21. What is the effect of surgeon and centre experience on the outcome of orthopaedic lower limb surgery in Cerebral Palsy children?
22. What are the indications for metalwork removal in children who have previously undergone lower limb orthopaedic surgery?
23. What is the efficacy of treatment for patello-femoral instability (an unstable knee-cap) in children?
24. What degree of lower limb anatomical variation (eg. knock knees, bow legs, in-toeing) justifies treatment to prevent long-term problems ?
25. What are the indications and most effective treatment for relapsed clubfoot?
26. What is the best way to treat idiopathic (unexplained) tip toe walking?

Appendix D - References

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