PROTOCOL

Standing Frames as part of postural management for children with Spasticity. What is the acceptability of a trial to determine the efficacy of standing frames?

Protocol Version Version 3

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2) Summary of Research: Abstract

Purpose:

To answer the question: What is the likely acceptability of a future trial to determine the efficacy of standing frames? We plan to undertake two surveys and qualitative research to assess the feasibility and inform the design of a trial (or trials) of standing frame use for children with cerebral palsy (CP).

Background:

CP is a common condition affecting 1 in 400 children. It is associated with spasticity and secondary musculoskeletal complications, which impact on mobility, participation and function for activities of daily living. UK consensus recommendations from 2006 for the management of children with severe CP (needing a wheelchair for mobility, GMFCS levels IV and V) advocate a 24 hour postural programme including the use of standing frames from age 12 months. A standing frame is a rigid frame with a wide base. A child is positioned within the frame with fastenings to enable the child to be upright, yet be free to use their arms and hands. Proposed benefits include maintenance of muscle length, reduction in joint contractures and hip dysplasia, improvement in bone mineral density, gastrointestinal motility, respiratory function, and improved participation and self-esteem. Although standing frames are widely used, there is limited evidence of their positive effect on these outcomes. There are also negative effects: some children experience discomfort in standing frames and families and education staff describe practical difficulties in their use. Knowledge is limited about variation in practice, prescription and actual standing frame use.

Design:

Sequential, mixed-methods (quantitative and qualitative) studies.

1) Survey 1: Professionals (health and education staff) and parents: to identify current use of standing frames.

2) Qualitative research: focus group work with parents and professionals; in-depth interviews with children to understand attitudes to standing frame use and acceptability of a trial.

3) Synthesis of findings from survey 1 and qualitative research, and development of potential trial designs with appropriate comparators and outcome measures.

4) Survey 2: Professionals and parents: regarding acceptability and feasibility of potential trial designs, including willingness to recruit (professionals), to have their child randomized (parents), comparators and outcome measures.

5) To propose a design for a substantive trial or trials.

Target population:

Young people with CP GMFCS Level IV or V, parents, health and education staff of these young people.

Health technologies being assessed:

Use of Standing Frames.

Sample size:

Surveys: we aim to survey 450-500 UK professionals and 150-200 parents of children with CP for each survey. The survey distribution will be similar for each survey, even if they do not respond to the first survey. Focus Groups: 6, with 8

people in each. 4 of the focus groups will be "single stakeholder" to include: parents, therapists, medical staff (orthopaedic surgeons and paediatricians, and educational professionals. The final 2 focus groups will be "mulit-stakeholder" and will include representation from all groups In-depth interviews with children and young people: 12-15 interviews.

Timetable:
The study will take place over 22 months: Survey 1 (months 1-4) Qualitative work (months 5-14) Synthesis and preparation of survey 2 (months 15-16) Survey 2 (months 17-18) Final analysis and trial proposal (months 19-22)

Expertise in team:
The team comprises clinical expertise in paediatrics (including Neurodisability), orthopaedics, occupational therapy and physiotherapy from 3 major English centres. Methodological input in trial design, survey design and qualitative methods is provided by the Institutes of Health & Society and Neuroscience and the UK CRC registered Newcastle Clinical Trials Unit at Newcastle University.

Deliverables:
1) Description of current standing frame use for children with CP (including treatment indications, treatment goals, types of frame, durations of intended and actual use; perceptions and practicalities of standing frame use).
2) A proposed design (Population- Intervention-Control/comparator-outcome(s) – Time) for a clinical trial(s) of effectiveness of frames in children with CP, GMFCS levels IV or V.

3) Background and Rationale:

Cerebral palsy (CP) affects 1 in 400 children. It is associated with spasticity and secondary musculoskeletal complications. 25% of children are non-ambulant (GMFCS IV or V) (Palisano 1997,Westbom 2007). These children suffer joint contractures, loss of bone mineral density (BMD), fractures and hip dislocation, leading to pain and progressive disability (Sheval 2009). Postural management, including standing frame use, is recommended.

Whilst a consensus statement (Gericke et al 2006) recommended the use of standing frames as part of a postural management programme for children with CP (GMFCS IV and V) from age 12 months, it also acknowledged the lack of an evidence base for this intervention. Studies are small case series, which are unblinded and not randomized.

Recent reviews (Bush 2010, Fehlings 2012, Paleg 2013) and the HTA brief find the evidence base for standing frame use to be limited. The most recent review (Paleg 2013) claimed a positive effect on BMD, hip stability and joint range of movement at the hip, knee and ankle with variable duration of standing frame use, but Fehlings (2012) found no such convincing evidence. Frames may also be disadvantageous. Children report pain and discomfort and families report increased demands on their time, hence reducing family and child participation. (Bush 2010) Further, standing frames are expensive (they cost between £800 and £2000), require adaptation with child growth, and use therapist time to prescribe and monitor their use.

Gibson et al (2009) undertook a within-group study of the effect of standing frame use for 1 hour daily for 6 weeks in 5 non-ambulant children with CP, aged 6-9 years. Two six-week intervals of standing frame use were alternated with two periods of no standing frame use. There was a suggestion of improvement of hamstring stretch with standing frame use.

Caulton et al (2004) reported a randomized controlled trial of a standing programme on BMD in 26 pre-pubertal children (age 4-11 years) with CP. This was a heterogenous group, paired according to vertebral and tibial BMD scores and then randomised to either their usual standing duration or 50% increased duration of standing. There was an increase of 6% mean vertebral BMD in the intervention group but no significant change in proximal tibial BMD in either group. The authors concluded that by increasing vertebral BMD through increased duration of standing there may be a potential to reduce risk of vertebral fractures.

There is variability in the amount of weight bearing in different standing frames, which may affect BMD outcomes (Herman 2007). Dynamic standing interventions may have more potential to improve bone health than passive standing frames (Gudjonsdottir 2002, Damcott 2013).

Synthesis of the literature and consultation with clinicians, therapists, educational staff, children and their families identifies clinical equipoise in standing frame use, justifying the need for further study. Furthermore there is a need to understand better the impact of interventions at different stages throughout the life course in individuals with long-term conditions (Ben-Shlomo 2002), with respect to participation, quality of life and self-esteem, rather than simply changes in body structure and function.

In the design of this study we will therefore adopt a view of health conceptualized by the International Classification of Functioning Disability and Health. (ICF-CY) (WHO 2008)

The challenges for trial design arise from the heterogeneity of current practice regarding the purpose and delivery of standing frame intervention, and the many variables in each of Population, Intervention, Comparator, Outcome and Timing that need to be considered.

<u>Current practice in the use of standing frames</u>

Little is known about current UK practice with respect to prescribing or actual use of standing frames, at home or in the community. Depending on the child's neurodevelopmental profile and the goal of standing frame use, a variety of different comparators in a trial may be appropriate. For example: "In children with CP aged under 2 years (Population), does introduction of a standing frame (Intervention), compared to non-introduction (Comparator) prevent hip dislocation (Outcome) over three years (Time)?"

Parents and professionals are likely to have strong pre-formed views about standing frame use. Professionals may have opinions informed by clinical experience, and make persuasive arguments to parents despite the weak evidence base. Parents may have invested time, effort and faith in standing frames. Thus, whilst the current paucity of evidence demonstrates a clear need for evaluative research, a substantive trial will be difficult to design.

A robust survey of current standing frame use and consultation regarding participation in a trial is hence required.

Evidence explaining why this research is needed now

The birth prevalence of CP is about 2.5 per 1000 live births. There were 729,000 births in England and Wales in 2012. This amounts to about 32,800 individuals aged 0-18 with CP. Approximately 1/4 are GMFCS level IV or V = 8200 individuals, for whom standing frames are likely to be considered as part of postural management.

The potential impact of standing frame use extends beyond childhood. Life expectancy in those with GMFCS levels IV or V cannot be deduced precisely, because published studies use different classifications of severity; however, 89% of those with only motor impairment and needing a self-propelled wheelchair lived to age 30; and 42% who could not self-propel lived to age 30. If an individual has additional severe impairments of for example cognition, life expectancy reduces (Hutton 2002). Thus there is a large population for whom obtaining clarity of the benefit of standing frame use is important.

For a child, a standing frame may reduce risks of joint contractures, hip dysplasia and scoliosis. It may improve bone mineral density and increase the likelihood, as a non-ambulant adult, of independent ability to stand and transfer. It may reduce pain and make daily care easier. Enabling the child to be vertical, a standing frame may improve head and trunk control, fine motor skills, gastrointestinal, bladder and respiratory function and increase self-esteem and social, communicative and exploratory participation. (Gericke et al 2006, Paleg 2013, Hughes 2014) Maintenance of the ability to stand and transfer for independence has clear practical benefit.

However these are only potential benefits. The NHS needs to know if these benefits are real, given that there are significant cost implications of use and also reported negative effects: some children experience discomfort in standing frames and families and education staff describe practical difficulties in their use (Hutton 2011).

If there is clinical benefit in the use of standing frames, then the costs need to be balanced against the costs of secondary musculoskeletal complications of spasticity in CP such as management of hip migration and dislocation, neuromuscular scoliosis, pathological fractures, pain and respiratory compromise which might have been prevented.

The NICE guideline for spasticity (CG 145 2012) highlighted the limited evidence base for all interventions for children with spasticity and specifically for postural management programmes. NICE proposed a trial of standing frame use for children aged 1-3 years with GMFCS level IV or V. The HTA has widened the brief in this call. We agree that this is appropriate due to clinical indications for frames and changing neurodevelopmental profiles at different ages.

A future trial of standing frame use would fit with the CMO's annual report 2012 (October 2013) highlighting the need for research into effective intervention for long-term conditions in childhood; particularly in neurodevelopmental disorders where health needs may be great but for which the evidence base for interventions is very weak.

Aims and Objectives

The overall purpose is to answer the question: What is the likely acceptability of a future trial to determine the efficacy of standing frames? To do this, we plan to undertake two surveys and qualitative research in order to assess the feasibility and inform the design of a trial (or trials) of standing frame use for children with cerebral palsy (CP).

Aims:

1) To determine current standing frame use in UK practice for the postural management of children age 1-18 years with CP with severe movement impairment (GMFCS levels IV and V).

2) To assess the willingness of parents to have their child randomized to potential trial designs, including the acceptability of different treatment regimens.

3) To assess preparedness of healthcare providers to recruit to potential randomized controlled trial designs.

4) To propose a substantive trial design or designs.

Objectives :

These will be linked to the aims above.

Aim 1 will be addressed by Objective 1.

Aims 2 and 3 will be addressed by Objectives 2, 3 and 4.

Aim 4 will be addressed by Objective 5.

1) To undertake a survey (Survey 1) of parents and carers, healthcare providers and education staff to determine current standing frame use for children with CP with GMFCS levels IV or V. The questions will include: treatment indications, treatment goals, types of frame, duration of intended and actual use; perceptions and practicalities of standing frame use. (Addressing Aim 1)

2) To undertake qualitative research to understand attitudes to standing frame use and acceptability of evaluating their benefit through a trial or trials. This will include: a) focus group work with parents/carers, healthcare providers and education staff, b) in-depth interviews with children. (Addressing aims 2 + 3)

3) To develop, based on the results of Survey 1 and the qualitative research, a small number (three or four) of potential trial designs, structured around a PICOT (Population, Intervention, Comparison, Outcome, Time) framework. (Addressing aims 2 + 3)

4) To complete a further survey (Survey 2) of parents and carers, healthcare providers and education staff regarding acceptability and feasibility of these potential trial designs. (Addressing aims 2 + 3)

5) To develop a substantive trial design or designs. (Addressing aim 4)

Research Plan

1) Survey 1: Professionals (health and education staff) and parents, to identify current use of standing frames.
2) Qualitative research: focus group work with parents and professionals; in-depth interviews with children to understand attitudes to standing frame use and acceptability of a trial.
3) Synthesis of findings from 1 and 2 above and development of potential trial designs with appropriate comparators and outcome measures.
4) Survey 2: Professionals and parents regarding acceptability and feasibility of potential trial designs, including willingness to recruit (professionals), to be randomized (parents), comparators and outcome measures.
5) Proposal of a design for a substantive trial or trials.

Health Technologies being assessed

One of the 'treatments' used widely in the management of children with spasticity is postural management which is an accepted, although very under-researched, aspect of physical therapy.

Postural management programmes include the use of standing frames, as well as resting, lying and seating support systems. Standing frames are part of established clinical practice as a means of improving functional ability, slowing or preventing musculoskeletal deformity and facilitating participation in activities.

There is significant heterogeneity in standing frame design. A frame is usually selected by a therapist, following clinical assessment of the child, but the choice of frame is influenced by a variety of factors both intrinsic to the child (level of motor ability, postural control, acceptability to the family), and extrinsic: related to environment; at school and home and other factors including cost, availability and therapist choice/experience.

Design and Theoretical/ Conceptual Framework

This proposal will use the framework of the UK Medical Research Council (MRC) guidance for how 'complex' (multifaceted) interventions should be developed and evaluated, highlighting in particular the issues that should be addressed as part of the intervention development and feasibility/piloting before a trial. Specifically, the tasks from the framework that will be addressed in the present study are: (i) establishing evidence about the problems and solutions (here evidence about the use of standing frames); and (ii) testing the procedures (here investigating the acceptability of the procedures).

Also as recommended within the framework for development and feasibility/piloting stages, a mixed methods approach with a literature search, survey, focus groups and interviews will be used. The study will have a sequential (quantitative -> qualitative -> quantitative) design where the findings of a previous step will be used to inform the following step.

The detailed study plan is outlined below and linked to the objectives above:

OBJECTIVE 1

"To undertake a survey (Survey 1) of parents and carers, healthcare providers and education staff to determine current standing frame use for children with CP with GMFCS levels IV or V. The questions will include: treatment indications, treatment goals, types of frame, duration of intended and actual use; perceptions and practicalities of standing frame use."

Survey 1:

1) Survey 1:

Sample size estimation

We aim to survey

- 180-200 parents of children with CP (GMFCS IV and V;) via clinical services: 100 via NECCPS and NNPCF; 30 at each of three clinical services and through social media linked to national organisations and charities (e.g. NNPCF, SCOPE, Council for Disabled Children;)
- 450-500 UK child health professionals working with children with CP (GMFCS IV and V.) (110 Orthopaedic consultants via BISCOS, 250 paediatricians; via CDT survey and clinical networks; and 80 - 100 therapists via APCP and clinical networks.)

Conservatively assuming a lower than expected response rate of 40%, these numbers would allow estimates of proportions to have 95%CI of width ±11% in parents, and width ±7% in the combined professional group.

Participants

To achieve the recruitment targets and gain a broad range of views, children with CP, parents/carers, paediatricians, orthopaedic surgeons and physiotherapists will be contacted. The aim is to recruit UK-wide, through the relevant National Royal Colleges, professional bodies, national professional newsletters and UK multidisciplinary Child Development Centres (via BACD). Email or postal invitations to participate in the study will be sent to relevant professionals. Parents will be recruited through a regional database: the North of England Cerebral Palsy Survey (NECCPS), clinical services and national parent support organizations. We have identified support for the application from Dr Karen Horridge: Chair of BACD and NECCPS.

An email/web based flyer will be sent out with a link to complete the survey via survey monkey. Paper forms of the survey questionnaires will be available for respondents who do not have web access or express a preference for a paper version. Contact details to request a paper copy of the survey (either by text, email or telephone) will be provided, and paper copies (with SAE) will be sent if requested. There will be a statement on each survey to confirm that the user has not completed it previously, to avoid duplication.

Materials

Questionnaire development will be undertaken in consultation with parents and child health professionals. Following literature review, the development of ideas and refinement of questions will be via email, telephone or web-based conference. Specific versions of a survey questionnaire will be designed for two user groups: i) professionals ii) parents. These will be piloted using cognitive interviewing techniques, in a small number of people selected by the co-applicant group, to assess comprehensibility and acceptability of the questions and associated instructions.

The draft of the survey will be taken to the 1st PPI group meeting in October 2015 for comment and final review, immediately preceding the 1st co-applicant meeting to finalise the content of the survey.

Most questions will require fixed-choice responses, though for some items there will be the facility for brief free-text responses. The questionnaire will be presented in the following sections: demographic characteristics; experience and use of standing frames as part of a postural management programme for treatment of spasticity in children with CP (GMFCS IV and V); type of standing frame used and factors influencing choice of standing frame design, perceived goals/ advantages of use; perceived barriers or disadvantages in use; and research priorities regarding evaluation of the efficacy of standing frames.

Procedure

The questionnaire will be sent with a request for response by 8 weeks and will be incentivized: a £10 Amazon voucher will be provided to all those who complete it. Evidence suggests that response rates are considerably improved with incentivisation and an immediate incentive is preferable to a prize draw or longer-term incentive, even if the value of the latter is higher. (Drummond et al 2013)

At the end of the questionnaire participants will be asked if they would be happy to be involved in further development of the ideas, such as focus groups or in depth interviews. It will be explained that it is important to obtain views from all user groups and from people with varying experiences of standing frame use, both positive or negative. Contact details will be requested for these people, with their consent.

The analysis of the questionnaire will be descriptive, largely reporting percentages of respondents in each category for each question. A summary of the results of the first survey will be analysed and discussed by the co-applicants via email, telephone and web based conference.

A co-applicants and PPI meeting will be held to discuss the findings of survey 1, and planning of the qualitative part of the study: including the topic guides for the focus groups and interviews. Care will be taken to obtain views without medical focus, and to consider standing frame design within the framework of the World Health Organization International Classification of Functioning, Disability and Health (ICF – CY.) Information will be disseminated to potential participants in focus groups and young people identified for the in depth interviews.

OBJECTIVE 2: Qualitative enquiry

"To undertake qualitative research to understand attitudes to standing frame use and acceptability of evaluating their benefit through a trial or trials. This will include: a) focus group work with parents/carers, healthcare providers and education staff, and b) in-depth interviews with children."

Qualitative methods allow health research to be informed by patient and practitioner views and priorities, and are useful in developing theory or design [Mays 1997]. Focus groups are a means to access consensus attitudes and opinions, (Barbour 2005) while interviews provide rich data on individual perspectives. (Banister 1998)

a) Focus groups and interviews to identify attitudes to a proposed research trial and generate potential trial protocols.

Target Population and sampling

Rrespondents to Survey 1 who have expressed an interest in further contribution to the study will be approached to contribute to the focus groups and in-depth interviews. If there are significant numbers who have agreed to this participation, purposive sampling will be used to try to capture diversity with respect to their experience of standing frame use, their attitude to standing frame use, the type of frame used and the severity of cerebral palsy, age and gender of the young person.

The descriptive data from the results of primary survey will be summarized and disseminated to potential participants in focus groups and young people identified for the interviews in advance of these, with explanation of the aims and purpose of the qualitative aspects of the research. This information will be designed by the co-applicant group and reviewed by the PPI group prior to dissemination. Care will be taken to be factual about the data obtained from the survey and information about example topics for discussion in the focus groups and interviews, without making any suggestion or prior assumption about the participants' spontaneous and independent views that we hope to obtain in the qualitative stages of the research.

Inclusion/Exclusion Criteria

a) Focus groups

INCLUSION: Professionals and parents who have participated in the survey and have expressed a wish to participate in the qualitative data collection through focus groups.

b) Interviews

INCLUSION:
Young people with CP GMFCS IV or V who have used a standing frame as part of their postural management programme. They will be identified either via carers completing Survey 1, or through the clinical services at the clinical centres of members of the co-applicant group.
Age 8 -18 years

EXCLUSION:
Severe cognitive impairment and/or significant communication difficulties resulting in inability to be able to make simple choices or communicate “yes” vs. “no” responses.

Setting/Context
a) Focus groups: to take place in clinical or education settings at each of the 3 centres
b) Interviews: at choice of young person + parent or carer

Data Collection

a) Focus Groups:

Four focus groups (single stakeholder) will be conducted at around the same time, each comprising 6-8 representatives of one of the following groups whose views are important to the study:

1. Physiotherapists & occupational therapists
2. Parents of children with CP
3. Medical professionals (orthopaedic surgeons , paediatricians) involved in the delivery of healthcare to these populations
4. Education staff from special and mainstream schools

Interviewing these groups separately will avoid any influence of perceived status or hierarchy, and facilitate the expression of views about other groups. Two further focus groups (multi-stakeholder) will be run consisting of participants from earlier groups, to gather their views on the researchers’ interpretation of data.

Focus groups will be digitally recorded with the permission of the participants. Sound files will be transcribed verbatim and anonymised.

b) In-depth interviews:
Over a longer period of time, in parallel to the focus groups we will also conduct a minimum of 12 interviews with young people who are current standing frame users aged between 8 and 18. Spreading interviews out will allow for an iterative process of data collection and reflection to inform sampling. Participants will be recruited through existing contacts and from respondents to the survey. Interested individuals will be sampled purposively for interviews seeking variation in gender, age (8-18), experience of frames, educational experience, socio-economic status, severity of condition and level of communication difficulties. Recruitment will continue until data saturation, defined as three consecutive interviews not returning new themes, by agreement among the research team (Francis 2009)

We estimate on the basis of previous work that data saturation may occur at around 15 interviews. Interviews will be conducted wherever convenient for the participant, with a familiar communication partner.

Informed consent from the young person’s parent, and assent from the young person will be obtained by the RA: this will include consent for digital and video recording. The purpose of video-recording is to aid with transcription and take account of non-verbal communication, and the use of augmentative/alternative communication devices and communication strategies, which would not be fully assessable from an

audio interview alone. Sound files will be transcribed verbatim and anonymised, and read back or given to participants to review for correctness.

Regarding interviewing young people:
Clinical members of the research team have significant experience of clinician, parent and young person clinical interactions and have experience in interviewing children with CP for research with a range of communication needs and can support the research associate with this. Nevertheless interviewing children is always a challenge and each child will have individual communication support requirements. Formal training for the RA will be sought.

The researcher needs to be able to build enough of a trusting relationship to feel confident that the children understand the question, and are giving their honest answers rather than the answer they feel the researcher would like to hear. The RA will need to be experienced and confident in communicating with children to be able to build this rapport. We recognize that interviewing young people is time-consuming and that the RA may need to meet an individual child on more than one occasion before the interview, to plan the interview location, environment and communication support. The interview will take place in the location of their choice, and the child and carers will be consulted to inform their positioning and postural support required for the interview to maximize comfort and communication. The child will be given the option to have a communication partner of their choice: this may be a parent, friend, educational support worker or therapist. The choice of the young person's communication partner will be crucial to the success of the interview: to support the YP understanding of the questions and also the researchers understanding of the total communication approach response.

Most research around interviewing young people focuses on power relations and understanding the interviewer's role as an 'adult' and how this might affect how the young person responds. The interviewee's may be crucial to this. Previous co-applicant experience of interviewing young people in school together with a chosen communication partner; showed that often a friend was chosen over their therapist, parent or teaching staff and this was deemed to encourage honesty and independence in their answers.

The interviewer will need to be creative about how they talk to the young people. Answering direct questions may not be possible. As long as young people understand the question, and have a clear yes/no response using a total communication approach, then it will be possible to gain their views, but methods such as role-play, story telling, using pictures or communication prompts may be required; for example pictures of the young person in their standing frame, pictures of different types of standing frame, or pictures of young people participating in different activities whilst in a standing frame may be used. Having the young person's standing frame at the interview may also be a helpful prompt, indeed giving them the choice of standing in it will be part of planning the interview environment.

Using another example from previous experience; "For one young person who was quite shy, we did quite a lot of role play about his views on PE, his equipment, management in school etc as I felt he was able to talk about how he felt more openly when he was talking about a 'new' boy who had come to school and how he might like to have his needs met."

Data Analysis:

We will use a Framework method to analyse all qualitative data (Ritchie and Spencer 1994). This method involves five stages: 1) familiarisation with the data to identify emergent themes with a small number of transcripts; 2) developing a thematic framework; 3) testing and refining the framework with a new batch of transcripts and refine framework accordingly: 4) using the framework to code the transcripts: 5) mapping and interpretation of themes. With the use of NVivo as a tool to manage the data we will also record negative cases as these may be important in the final analysis. A proportion of transcripts will be second-coded by an investigator; any divergence in coding or emergent issues will be discussed with JL and JK and at regular team meetings to inform the analysis and identify areas for closer consideration (including negative case analysis) and reach a robust interpretation consistent with all data. (Barbour 2003, Elliott 1999)

OBJECTIVE 3

"To develop, based on the results of Survey 1 and the qualitative research, a small number (three or four) of potential trial designs, structured around a PICOT (Population, Intervention, Comparison, Outcome, Time) framework."

Data from the previous focus groups, interviews and from Survey 1 will inform the topic guide for the multi-stakeholder focus groups. The PICOT structure will be applied to develop a range of potential trials, with various design elements – such as level of randomization etc., based on plausible scenarios, for discussion in these two focus groups where acceptability, barriers to participation and randomization will be explored for each design. Participants in the multi-stakeholder groups will be selected from the single groups on the basis of their willingness to contribute to the multi-stakeholder groups and asked to act as representatives of the single stake-holder group to express and feedback issues discussed.

For the purposes of Survey 2, to ensure the study keeps to schedule, a brief analysis of the multi-stakeholder focus group data will be conducted. This data analysis will be distributed to all co-applicants for their comment and interpretation, to develop 3-4 potential trial designs for survey 2. Survey 2 will be drafted via email and webex conference and sent for discussion to the PPI advisory group for their 3rd meeting. A co-applicant meeting will follow this, to finalise the content of Survey 2

OBJECTIVE 4

"To complete a further survey (Survey 2) of parents and carers, healthcare providers and education staff regarding acceptability and feasibility of these potential trial designs."

1) Second survey

The second survey will be sent to all previous professionals and parents identified for the first survey, regardless of participation in Survey 1. Survey methods will be as described previously for survey 1. To maximize response rate to survey 2, any survey 1 respondent who has provided contact details will receive information regarding study progress in preparation for the second survey.

The second survey will include sections once again on demographics, current use of standing frames and any recent change in practice or protocol.

The final section will contain summary descriptions ('vignettes') of the designs for 2 or 3 proposed, multi-site randomized controlled trials (RCT) to evaluate the use of standing frames as part of a postural management programme in children with CP (GMFCS IV and V) together with illustrative flow charts.

The vignettes will include an explanation of the need for randomization and the potential comparators. Once survey participants have read the vignettes and flowcharts, they will be asked a series of questions with fixed-choice responses (with space for brief free text comments) to ascertain opinions about the proposed trial designs and levels of clinical support required. This section of the questionnaire will be devised to investigate possible barriers and facilitators to recruitment and retention of families within the proposed research designs. This approach is similar to that used by members of the co-applicant team in surveys of stakeholders regarding proposed designs for trials of dietary interventions in young people with autistic spectrum disorder, and a trial of medications to treat drooling (Parr 2012, Winburn 2013).

OBJECTIVE 5

"*To develop a substantive trial design or designs."*

1) Data integration: The findings from the single and multi-stakeholder focus groups and interview data, particularly themes related to trial participation and design, will be compared with the results from Survey 2. If the findings are favourable with respect to potential feasibility of a trial and willingness of parents and professionals to allow young people to be randomized, this final analysis of the qualitative research and survey 2 will inform generation of a proposed trial protocol with suitable comparators.

2) The results of the data analysis will be distributed to all co-applicants for their scrutiny and discussion.

3) A final meeting of co-applicants and PPI advisory group will be convened to confirm potential trial design(s) and to plan development and potential funding application(s) for a trial(s).

Dissemination and projected outputs

Planned outputs:

1) NIHR HTA report of current standing frame use in children and young people with CP aged 1-18 years, GMFCS levels IV or V; to include treatment indications, treatment goals, types of frame, durations of intended and actual use; perceptions and practicalities of standing frame use.

2) A proposal for a design for a substantive trial or trials to assess clinical and cost effectiveness of standing frame use for children and young people with CP aged 1-18 years, GMFCS levels IV or V with:

a. suitable comparator(s)

b. information about willingness of parents to allow their child to be randomized

c. information about preparedness of healthcare providers to recruit and randomize children

d. personnel required to support such a trial

e. outcome measures and timescale for such a trial

The key impact will be to inform the HTA on whether it is realistic for a trial of standing frame use to be undertaken in the UK. If the HTA decided to put out a call and a successful trial (or trials) was conducted, this would be a major achievement, as it would demonstrate that trials of poorly evidenced therapeutic practices directed to disabled children and young people could be undertaken in the UK, using networks of therapeutic teams supported by the infrastructure of NIHR.

Dissemination:

Dissemination will take place during the study, not just at its end. We want young people, parents/carers and professionals to be aware of this work at an early stage so that they feel their reactions and suggestions are contributing to it and anticipate the results with interest. We will consult with parents who participate in Survey 1 about additional ways to disseminate information, but will include:

1) Information for newsletters and web sites of Cerebra, SCOPE, Contact-a-family, Council for Disabled Children.

2) Information to children and families through leaflets, parent/carer forums and internet social sites such as website/facebook page and Twitter.

3) During the project, presentation at Annual Conferences of:

- European Academy of Childhood Disability
- Royal College of Paediatrics and Child Health
- British Society for Children's Orthopaedic Surgery
- Association of Paediatric Chartered Physiotherapists

4) Open access publication in peer reviewed journals at the end of the project:

a) Results of Survey 1 of current use of standing frames in the UK.

b) The qualitative work on feasibility and acceptability of a trial into the use of standing frames for children with CP with a proposed study protocol (which if funded in the future, will be published in the Trials Journal).

Plan of investigation and timetable

The study period will be over 22 months from October 2015, as shown in the study outline diagram. There will be 4 project management meetings of co-applicants and PPI meetings between each phase of the study, to review progress and finalise plans for the next stage of the study. Throughout the study, there will be close communication between co-applicants using email, web-based and telephone conferencing.

Study management

The CI will have overall responsibility for project management and delivery of outcomes. She will work closely with the PI at each site, at co-applicant qualitative research team at the Institute of Health and Society, Newcastle Unversity which include senior NIHR academics; and the lead for the UK CRC registered Newcastle CTU.

The RA will undertake day to day running of the study, and trial manager role. They will lead data collection and analysis. Other academic and clinical co-applicants will support design and analysis of the surveys, focus groups, in-depth interviews and potential trial designs.

The co-applicant committee, consisting of all co-applicants, will have four meetings over the course of the project. The study management team, consisting of the CI, PI's and RA will have fortnightly face to face, telephone or web-mediated conferences.

The PPI advisory group, co-ordinated by the parent co-applicant, and to include experienced communication support professionals and users will review proposals from the co-applicant Committee; and will scrutinise the content of Survey 1, structure and content of the focus groups, in-depth interviews, Survey 2 and final trial proposal(s).

The clinical team; CI and PI's will be responsible for recruitment to the focus groups and interviews. The parent co-applicant will provide participant and carer support during focus groups and interviews. Other clinical co-applicants will provide clinical expertise and knowledge about standing frame design and potential trial designs.

Research management expertise and administration will be provided by the administration teams, finance and contract teams support in the Institute of Neuroscience and Institute of Health and Society at Newcastle University. The Research Co-ordinator and technical support will ensure efficient running of the project according to appropriate regulations.

An independent Study Steering Committee will be set up to monitor the management of the study according to the guidance and terms of reference of the NIHR HTA programme.

Regulatory approvals

Research Ethics Approval:

All research governance approvals will be sought for this multi-site clinical research study in the pre-study period following confirmation of the award. We will inform the research ethics committee that each stage of the research will inform the subsequent stage; therefore there will be submissions of materials for each stage (including topic guides for the focus groups and interviews, and survey 2) as amendments.

The research may raise questions of clinical equipoise in existing practice with carers and young people. Issues such as future concordance with treatment and questions that carers, young people and professionals may have following the study may arise. Contact details of the study team members willing to provide further information and details of independent organizations such as PALS will be provided on a debriefing sheet.

Informed consent (including agreement for audio and video recordings) will be obtained from focus group participants and parent carers of young people taking part in in depth interviews. For the young people, assent will be obtained. Consent will be taken by the RA once appropriate training, including GCP has been provided. Information and consent forms will be provided for the surveys.

Confidentiality of carers', young people's and professionals' opinions will be maintained. For focus group participants this will be through setting ground rules. Transcripts will be anonymised for both focus groups and interviews. Most survey data will be anonymous, though some people from survey 1 will have provided their contact details for subsequent stages of the study. These details will be stored securely with access to the study team only and used only for the purposes of contacting these potential participants.

Data will be stored in accordance with University and Trust regulations. The CI will act as custodian.

Declaration of interests:
As an MHRA registered medical device manufacturer, the Orthotic Research & Locomotor Assessment Unit (ORLAU) is responsible for managing the relationship between The Robert Jones and Agnes Hunt Hospital (RJAH), ORLAU Publishing Ltd (a commercial offshoot not-for-profit company which owns the IP rights of the ORLAU 1000 and ORLAU Adult patterns of standing frame) and commercial manufacturers. RJAH is a purchaser of ORLAU designed equipment and ORLAU Publishing Ltd receives income from sales. This income is returned to the RJAH Trust in the form of support for ongoing research, development and teaching work within ORLAU. ORLAU also provides a standing frame supply and maintenance service to the Movement Centre, an independent charity based on the RJAH Orthopaedic Hospital site.

Chailey Heritage Clinical Services is an NHS service within Sussex Community Trust. The service has influenced design and manufacture of many types of postural management equipment including standing frames. Chailey Heritage receives royalties from sales of a commercially available standing frame through the manufacturer: Active Design.

Patient and Public Involvement

Parents of children attending postural clinics in Newcastle in October 2013 – March 2014, and parent carers in the Chailey research advisory group have been asked their opinions on the potential study with respect to:

- survey methods: via post or online
- support and venue of child interviews
- agreement to video recording or audio-recording of interviews and focus groups barriers to joining such a study.

The parent co-applicant has also shared a questionnaire using the regional network for NNPCF (National Network of Parent Carer Forums) via Facebook and email.

Parents were very positive regarding:

1) the need for the research: parents feel that standing frames are important and part of their children's lives and they have expressed surprise at the limited evidence base. One parent reported "feeling guilty" when she had been unable to achieve prescribed frame use, especially at the time she was adapting to their child's diagnosis. She said that if she had understood that the evidence base was limited, this guilt would have been less.
2) willingness to support the proposed methods: to complete surveys online, and to support child interviews. No one has been concerned about the prospect of either audio or video recording focus groups or interviews. Most were happy to attend a focus group; only one parent said she didn't like groups per se. Practical considerations were identified for focus groups: to be in school hours, to be local, coffee/tea should be provided and there should be easy access to parking.

Educational staff also provided similar feedback:

1) research into standing frame use is worthwhile: "it will be good to have evidence regarding benefits – why standing frames are necessary and what might happen if children didn't use one?"
2) the proposed methods: "the on-line survey is a good idea but must not be too long. For practical reasons focus groups need to be out of school time but it would be difficult to include teaching assistants as they only work school hours.

A PPI group will be involved throughout the study and led by a parent co-applicant who has extensive experience of standing frame use, working as a family support worker and parent representative/advisor to multi agency professional groups. We have developed specific draft terms of reference for the group in accordance with INVOLVE guidance.

The PPI advisory group will be set up with representation from the CHAILEY Locality and will consist of:

- 2 Parent carers from i) the parent research advisory group at Chailey School or ii) NNPCF.

- 2 young people with cerebral palsy aged 19 -25, resident at the Futures Centre, Chailey,
- 2 representatives of Educational staff from both special schools and mainstream schools.
- A representative with expertise in communication with YP with additional communication needs (e.g.TRIANGLE or a speech therapist.)

The draft terms of reference will be reviewed and finalised at the first meeting. The overall management of the group, and communication between members of the group will be determined by themselves. The group will meet four times in the study period in parallel to the co-applicant project management group. Representation from predominantly one locality will facilitate attendance. While we aim that at the first meeting all group members will meet in person, video link attendance will be an option for later meetings.

All members of the PPI advisory group will be remunerated at INVOLVE recommended rates, and have access to appropriate mechanisms to facilitate communication and advocacy support.

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