Trial Review

COVID-19 studies are our top priority.

For new and updated trial submissions, we are processing trials as quickly as possible and appreciate your patience. We recommend submitting your trial for registration at the same time as ethics submission.

VIEW TRIAL AT REGISTRATION VIEW HISTORY

The safety and scientific validity of this study is the responsibility of the study sponsor and investigators. Listing a study does not mean it has been endorsed by the ANZCTR. Before participating in a study, talk to your health care provider and refer to this information for consumers

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Trial registered on ANZCTR

Registration number	ACTRN12620000416998
Ethics application status	Approved
Date submitted	12/03/2020
Date registered	27/03/2020
Date last updated	21/10/2021
Date data sharing statement initially provided	27/03/2020
Type of registration	Prospectively registered

Titles & IDs

Public title	Improving muscle strength in young people with Prader-Willi syndrome: a phase II randomised trial
Scientific title	The effect of exercise on muscle strength in young people with Prader-Willi syndrome: a phase II randomised trial
Secondary ID [1]	None
Universal Trial Number (UTN)	
Trial acronym	PRESTO
Linked study record	

Health condition

Health condition(s) or problem(s) studied:

Prader-Willi syndrome

Condition category	**Condition code**
Human Genetics and Inherited Disorders	Other human genetics and inherited disorders

Intervention/exposure

Study type	Interventional
Description of intervention(s) / exposure	Participants will be randomised to receive one of two exercises programs. Intervention group participants will complete an exercise program, supervised 1:1 by an exercise professional. Participants will exercise twice a week for 24 weeks (48 sessions in total). Each exercise session will last

	approximately 60 minutes. All exercise sessions will take place in a community gym local to the participant. The exercise program will be supervised by an exercise professional (usually a physiotherapist, exercise physiologist or personal trainer). Exercise professionals will be invited to participate based on their location and typical practice (e.g. working in paediatrics, neurological, or musculoskeletal areas). They will receive a training manual that includes details about the trial protocol, specialist advice on Prader-Willi syndrome, how to facilitate exercise in people with Prader-Willi syndrome, communication strategies, and behaviour management. The exercise professional will complete an exercise log (either in hard copy or online) on behalf of the participant to document the exercises completed and any adverse events that occur. Participants will also receive 2 planning sessions of 1-hour duration following the intervention period with a facilitator to encourage their ongoing participation in community exercise. These sessions will be conducted by an exercise professional either in person or via videoconference. The content of these sessions will be individualised and will aim to address barriers to taking part in ongoing community exercise. These sessions will take place approximately 1 month and 3 months after the end of the intervention.
Intervention code [1]	Rehabilitation
Comparator / control treatment	Control group participants will also complete an exercise program, supervised 1:1 by an exercise professional. Participants will exercise twice a week for 24 weeks (48 sessions in total). Each exercise session will last approximately 60 mins, All exercise sessions will take place in a community gym local to the participant. The exercise program will be supervised by an exercise professional (usually a physiotherapist, exercise physiologist or personal trainer). Exercise professionals will be invited to participate based on their location and typical practice (e.g. working in paediatrics, neurological, or musculoskeletal areas). They will receive a training manual that includes details about the trial protocol, specialist advice on Prader-Willi syndrome, how to facilitate exercise in people with Prader-Willi syndrome, communication strategies, and behaviour management, The exercise professional will complete an exercise log (either in hard copy of online) on behalf of the participant to document the exercises completed and any adverse events that occur. Participants will also received 2 planning sessions of 1-hour duration following the intervention period with a facilitator to encourage their ongoing participation in community exercise. These sessions will be conducted by an exercise professional either in person or via videoconference. The content of these sessions will be individualised and will aim to address barriers to taking part in ongoing community exercise. These sessions will take place approximately 1 month and 3 months after the end of the intervention.
Control group	Active

Outcomes

Primary outcome [1]	Muscle strength- of the arms and legs will be assessed using 1 repetition maximum (1RM) force generation tests. Composite measures of arm (chest press) and leg (leg press) strength will establish the amount of weight each participant can lift once.
Timepoint [1]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [1]	Lean muscle mass will be assessed using a dual energy x-ray absorptiometry (DXA) whole body scan for total lean (muscle) mass and regional lean mass. DXA scans will be carried out on the same equipment at each time point for each participant at each site.
Timepoint [1]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [2]	Sit-to-stand test: measures how long it takes to stand up and sit down 5 times
Timepoint [2]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [3]	Weighted box-stacking test: measures how many boxes weighing 10kg can be stacked in one minute
Timepoint [3]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [4]	Timed stair climb test: measures how long it takes to go up and down a standard flight of stairs
Timepoint [4]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [5]	6-minute walk test: measures distance walked by the participant in 6 minutes
Timepoint [5]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [6]	Physical activity levels (accelerometry): Actigraph GT3X+ accelerometers will be used to measure total physical activity, total sedentary time and the number of steps participants take during waking hours over 7 consecutive days.
Timepoint [6]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [7]	Community participation (attendance): will be measured using the Adolescent Physical Activity Recall, the Adolescent Sedentary Activity, and the community section of the Participation and Environment Measure-Children and Youth questionnaires. These questionnaires measure what sports and other physical activities the participant does, how often and for how long and will be completed by participants and/or their family member or residential caregivers where necessary.
Timepoint [7]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [8]	Community participation (involvement): will be measured using the community section of the Participation

	and Environment Measure-Children and Youth questionnaire. This questionnaire measures how involved participants feel in 10 activities and will be completed by participants and/or their family member or residential caregivers where necessary.
Timepoint [8]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [9]	Health related quality of life: will be measured using the 9-item Child Health Utility (CHU-9D) instrument and the Quality of Life Inventory-Disability questionnaire. The CHU-9D will be completed by participants and/or their family members or residential caregivers where necessary. The Quality of Life Inventory-Disability questionnaire will be completed by family members or caregivers.
Timepoint [9]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [10]	Healthcare utilisation: will be assessed via a health service utilisation questionnaire developed for the trial and completed by participants and/or their family members or residential caregivers where necessary. The questionnaire will collect data on hospital admissions and community allied health visits.
Timepoint [10]	Week O (baseline, week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [11]	Adverse events: will be categorised as serious or non-serious, expected or unexpected, and related or unrelated to the trial will be documented in the participant's exercise logbook completed by the health professional (usually a physiotherapist) supervising the intervention. Examples of possible adverse events are delayed onset muscle soreness, increased anxiety resulting in skin picking or a temper outburst (behavioural features of Prader-Willi syndrome) and food stealing.
Timepoint [11]	During intervention phase of the trial (compiled at week 25, immediately post intervention)
Secondary outcome [12]	Diet: will be documented by parents and carers (not participants) using the online version of the Australian Eating Survey
Timepoint [12]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [13]	Gym experience: qualitative data about the participants' experience of exercise will be collected from both groups (intervention and control). Data on acceptability, benefits and social interactions with gym users during training will be documented from semi-structured interviews with participants and their families. Photographs and video diaries will also be collected by participants using an iPod touch given to them on loan by the research team at trial commencement. Data on social interactions with other gym users will be documented in the participant's exercise log during training by the health professional delivering the intervention. Data collection will be supplemented by observation (using ethnographic methods) for a subgroup of participants (n=10 participants), where 3 training sessions (one session during initial weeks, middle weeks and final weeks of training) will be observed.
Timepoint [13]	During intervention phase of the trial (compiled at week 25, immediately post intervention)
Secondary outcome [14]	Behaviour will be measured using the Developmental Behaviour Checklist questionnaire. The Developmental Behaviour Checklist -Parent version (DBC-P) will be completed by family members or residential caregivers of adolescents (aged 13-17 years) and the Developmental Behaviour Checklist -Adult version (DBC-A) will be completed by family members or residential caregivers of adults (aged 18 years and over).
Timepoint [14]	Week 0 (baseline), week 25 (immediately post intervention) and week 52 (6-months post intervention)
Secondary outcome [15]	Medicare Australia records will be retrieved with participant consent to determine medical services and pharmaceutical use over 12 months.
Timepoint [15]	Week 52 (6-months post-intervention)

Eligibility

Key inclusion criteria	Each participant must meet all of the following criteria to be enrolled in this trial: • Have genetically confirmed Prader-Willi syndrome, • Aged between 13 and 60 years (inclusive) at the time of randomisation, • Able to follow simple verbal instructions in English, • Medical clearance from their general practitioners or physician certifying they can participate (where considered necessary based on answers to the pre-exercise screening questionnaire PAR-Q+), • Provide a signed and dated informed consent form or has a legally acceptable representative capable of understanding the informed consent document and providing consent on the participant's behalf.
Minimum age	13 Years
Maximum age	60 Years
Gender	Both males and females
Can healthy volunteers participate?	No
Key exclusion criteria	People meeting any of the following criteria will be excluded from the trial: • Has participated in progressive resistance training in the 3 months prior to randomisation • Has a concurrent physical (e.g. severe arthritis), psychological (e.g. severe psychosis) or behavioural issue (e.g. violent behaviour) that might affect their ability to participate in a 24-week exercise program. • Inability or unwillingness of participant or legally acceptable representative to give written informed consent.

Study design

Purpose of the study	Treatment
Allocation to intervention	Randomised controlled trial
Procedure for enrolling a subject and allocating the treatment (allocation concealment procedures)	
Methods used to generate the sequence in which subjects will be randomised (sequence generation)	
Masking / blinding	Blinded (masking used)
Who is / are masked / blinded?	The people receiving the treatment/s The people assessing the outcomes The people analysing the results/data
Intervention assignment	Parallel
Other design features	
Phase	Not Applicable
Type of endpoint(s)	
Statistical methods / analysis	

Recruitment

Recruitment status	Recruiting

Date of first participant enrolment

Anticipated	3/04/2020	**Actual**	24/02/2021

Date of last participant enrolment

Anticipated		**Actual**	

Date of last data collection

Anticipated		**Actual**	

Sample size

Target	60	**Accrual to date**	24	**Final**	

Recruitment in Australia

Recruitment state(s)	NSW,QLD,VIC
Recruitment hospital [1]	The Royal Childrens Hospital - Parkville
Recruitment hospital [2]	Royal Prince Alfred Hospital - Camperdown
Recruitment hospital [3]	Princess Alexandra Hospital - Woolloongabba
Recruitment hospital [4]	Queensland Children's Hospital - South Brisbane
Recruitment hospital [5]	Austin Health - Austin Hospital - Heidelberg
Recruitment postcode(s) [1]	3052 - Parkville
Recruitment postcode(s) [2]	2050 - Camperdown
Recruitment postcode(s) [3]	4102 - Woolloongabba
Recruitment postcode(s) [4]	4101 - South Brisbane
Recruitment postcode(s) [5]	3084 - Heidelberg

Funding & Sponsors

Funding source category [1]	Government body
Name [1]	Medical Research Future Fund
Address [1]	Department of Health GPO Box 9848 Canberra ACT 2601 Australia
Country [1]	Australia
Primary sponsor type	University
Name	La Trobe University

Address	Kingsbury Drive, Bundoora, VIC 3086
Country	Australia
Secondary sponsor category [1]	None
Name [1]	
Address [1]	
Country [1]	

Ethics approval

Ethics application status	Approved
Ethics committee name [1]	The Royal Children's Hospital Melbourne Human Research Ethics Committee
Ethics committee address [1]	50 Flemington Rd, Parkville VIC 3052
Ethics committee country [1]	Australia
Date submitted for ethics approval [1]	
Approval date [1]	18/04/2019
Ethics approval number [1]	2019.048

Summary

Brief summary	We will investigate if exercise is effective in increasing muscle strength in people with Prader-Willi syndrome (PWS). We will conduct a phase II, multi-site, double-blind, randomised controlled trial with 6-month follow-up. Sixty participants with PWS aged 13 to 60 years will be randomised to receive one of two exercise programs. Participants will exercise twice a week for 24 weeks at their local gym supervised by an exercise health professional (usually a physiotherapist). We will measure muscle strength, muscle mass, functional strength, physical activity, community participation, and health-related quality of life at baseline (week 0), after the intervention (week 25) and 6 months later (week 52): We will recruit participants through PWS advocacy groups, specialist PWS clinics, and PWS registries and clinical databases.
Trial website	
Trial related presentations / publications	
Public notes	

Contacts

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Data sharing statement

Will individual participant data (IPD) for this trial be available (including data dictionaries)?	Yes
What data in particular will be shared?	Individual participant data for published primary and secondary quantitative outcome measures.
When will data be available (start and end dates)?	Following the publication of the main trial outcomes (circa 2024), no end date.
Available to whom?	Data will be open access.
Available for what types of analyses?	Data will be available for any purpose including meta-analyses.
How or where can data be obtained?	Data will be deposited in the La Trobe University library repository.
What supporting documents are/will be available?	Study protocol Ethical approval

How or where can supporting documents be obtained?

Type [1]	Ethical approval
Citation [1]	
Link [1]	
Email [1]	
Other [1]	
Attachment [1]	/Steps11and12/377484-(Uploaded-08-07-2019-12-31-15)-Study-related document.pdf
Type [2]	Study protocol
Citation [2]	
Link [2]	
Email [2]	
Other [2]	We aim to publish a study protocol in an open access journal.
Attachment [2]	

Summary results

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