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Improvement of physical abilities in patients with cystinosis

Submission date
19/09/2023

Recruitment status
No longer recruiting

Registration date
21/09/2023

Overall study status
Completed

Last Edited
21/09/2023

Condition category
Genetic Diseases

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[ ] Results
[ ] Individual participant data
[ ] Record updated in last year

Plain English summary of protocol

Background and study aims
Infantile nephropathic cystinosis is a rare condition that affects the body's ability to store certain substances. In the early 1980s, a way to help people with this condition live longer was introduced, using a treatment called cysteamine and kidney therapy. But even with treatment, this disease is pretty tough. It keeps getting worse over time, and there's no cure for it. It affects your muscles, which can be a big deal for how the disease goes, how long you live, and how good your life is.

When you're a teenager, the disease can make your muscles weak, especially in your hands. As it gets worse, it can affect the muscles in your chest, making it hard to breathe, talk, and swallow. So, the condition of your muscles is super important for how the disease goes and how your life is.

We are looking at an exercise plan based on something called "Auf die Beine," which was made in Cologne for kids and teens who can't move around well because of cerebral palsy (you can check it out here: https://unireha.uk-koeln.de/kinder-jugendreha/behandlungskonzept-auf-die-beine/). The plan we're talking about involves exercises you can do at home and short workouts. They know that people with this disease already have a lot to deal with, like taking medicine, eating a special diet, and maybe even having to do dialysis.

So, what we're trying to find out is if doing these exercises on a special vibrating platform called a 'Galileo plate' can help people with cystinosis get stronger and have better heart and lung health. We also want to see if it makes them more active in their everyday lives and if it makes life better for them. We're also trying to figure out if these exercises can fit into the lives of people who are sick for a long time.

Who can participate?
Patients with a confirmed diagnosis of infantile nephropathic cystinosis from 6 to 13 years of age or from the age of 18 years are eligible to participate.

What does the study involve?
Patients in the intervention group train with Galileo vibration plates according to a fixed training

schedule that includes ten short training sessions per week, with a maximum of two sessions per day. Each training session consists of four specific exercises. In contrast, the control group currently performs the same exercises without vibration plates but uses dumbbells instead.

At the outset of the study, patients undergo an intensive training course and receive ongoing supervision during the three-month home training phase. This study follows a randomized controlled trial design. To ensure comparability between the two groups, a matched pair design is chosen due to the rarity of the underlying disease, with patients currently being matched based on their age, gender, and major previous surgeries.

A total of 24 adult patients and 16 children are currently participating in the trial. The study currently includes three clinical assessments: one at the start (baseline), another after the three-month home training phase, and a final assessment after the follow-up phase.

What are the possible benefits and risks of participating?
Potential benefits
Patient orientation: empowering patients by supporting an active lifestyle and enabling patients to positively influence the course of the disease themselves.
Optimization of clinical outcomes: improving cardiorespiratory performance and increasing muscle strength in patients.
Improvement of patient-oriented end points of care: improving quality of life.

Risk of participating
Patients who are likely to undergo a serious intervention (e.g. kidney transplant, orthopedic surgery) during the duration of the study or for whom there are contraindications for training on Galileo vibration platforms according to the official recommendation were excluded from the study. However there was no specific risk for the participants.

Where is the study run from?
Cystinosis Center Rosenheim (Germany)

When is the study starting and how long is it expected to run for?
June 2020 to September 2022

Who is funding the study?
Cystinosis Foundation (Germany)

Who is the main contact?
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Study information

Scientific Title
Prospective study to improve cardiorespiratory performance and muscle strength in patients with cystinosis using the Galileo vibration plate

Acronym
IMPACT

Study objectives
As a primary working hypothesis, this project aims to investigate whether regular home practice using Galileo vibration training platforms positively impacts muscle strength and increases cardiorespiratory performance in cystinosis patients. Secondary objectives include whether the training results in a general increase in everyday activity and an improvement in the quality of life and whether it is possible to integrate the therapy into the day-to-day life of chronically ill patients. The short-term scientific objectives of the project are directly related to the scheduled home training sessions and involve the assessment of muscle strength and cardiorespiratory performance as well as the evaluation of integrability into everyday life. Long-term objectives of the project are to increase everyday activity levels and to achieve a sustainable improvement in the quality of life.

Ethics approval required
Ethics approval required

Ethics approval(s)

Approved 16/06/2020, Bayerische Landesärztekammer (Mühlbaurstr.16, Munich, 81677, Germany; +49 894147212; ethikkommission@blaek.de), ref: 20005

Primary study design
Interventional

Study design
Interventional randomized controlled matched-pair design

Study type(s)
Prevention, Quality of life, Treatment, Efficacy

Health condition(s) or problem(s) studied
Improvement of cardiorespiratory performance and muscle strength in patients with cystinosis

Interventions
The patients will be divided into two age groups depending on their age at the time of randomization. Due to the very rare underlying disease, a matched-pair design was chosen as the study design. Patients will be matched according to their age at the time of randomization, sex and major previous surgeries (kidney transplant, axis corrections of lower legs) and randomly assigned to an intervention group (IG, training on vibration platforms) or a control group (CG, training with vibration dumbbells). Randomization will be performed before the enrollment of all participants in the study with a small self-written R - programme.

The training concept is based on the established approach for the rehabilitation of children “Auf die Beine” developed by UniReha Cologne and on the official recommendations for the use of a vibration platform (4,5). One group (IG) trains while standing on a Galileo vibration platform, the other group (CG) exercises the arms in a sitting position using a vibration dumbbell (Galileo-Mano). Both groups train according to a fixed training schedule which involves 10 short training sessions in a week (maximum 2 per day).

Patients in the IG will initially undergo a one-day intensive training course and will receive regular supervision by the responsible physiotherapists during the training phase (once a month, more if required).
Both the training program with the Galileo vibration platforms (IG) as well as the one with the dumbbells (CG) are tailored to the disease.

The training program for the IG comprises four basic exercises:
• deep squats with attention to the correct leg axis
• heel rise (stand on toes and forefeet)
• push-ups from the quadruped position with both hands resting on the vibration platform
• lifting off and stretching forward one hand from the quadruped position with both knees resting on the vibration platforms

The training program with the Galileo-Mano vibration dumbbells (CG) consists of three basic exercises, each to be performed with both the right and left hand:
• while seated with the forearm horizontal, holding the vibration dumbbell and rotating the forearm towards pronation/supination
• while seated, holding the vibration dumbbell, and lifting it outwards to maximum shoulder

height, then lowering it again towards the knee
• while seated, holding the vibration dumbbell, and lifting it upwards, towards the opposite shoulder, then lower in it again towards the knee.

Each exercise lasts 30 seconds and can be extended to up to one minute depending on the training progress. The duration of vibration per training session is 2 to 4 minutes. Including short breaks each training session thus lasts 5 to 8 minutes. The training frequency of the vibration platform will be progressively increased from 20 to up to 33 Hz (4). Frequencies of 20 Hz or higher induce a strengthening of the musculature; according to the manufacturer this effect is enhanced with increasing frequency.

Intervention Type
Device

Phase
Phase I

Drug/device/biological/vaccine name(s)
Galileo Plate

Primary outcome(s)
At baseline, 3, 6, and 12 months:
1. Muscle function tests (power & strength) using Leonardo Mechanograph (Novotec) (Measurement of ground reaction forces) with s2LJ (single two-legged jumps)
2. HRT (Heel-Rise test)
3. m1LH (multiple one-legged hopping on the forefoot with the knee outstretched)
4. Grip strength
5. 6-minute walk test were performed

Key secondary outcome(s)
Clinical parameters at baseline, 3, 6, and 12 months:
1. Blood pressure (sphygmomanometer)
2. Pulse oximetry
3. Quality of life and wellbeing (children and adolescents: KINDL-R questionnaire, adults SF-36 questionnaire)
4. Clinical (orthopedic surgery, fractures) and laboratory assessment (kidney function, end stage renal disease, dialysis, transplantation, cystine score) physiotherapeutic and orthopedic examination at baseline and 12 months only

Completion date
24/09/2022

Eligibility

Key inclusion criteria
1. Confirmed diagnosis of infantile nephropathic cystinosis
2. Prepubertal patients from 6 to 13 years of age or from the age of 18
3. Consent of the patient (18 years and older) or legal representative (for minors)

Participant type(s)

Patient

Healthy volunteers allowed
No

Age group
Mixed

Lower age limit
6 Years

Upper age limit
45 Years

Sex
All

Total final enrolment
40

Key exclusion criteria
1. Patients who are likely to undergo a serious intervention (e.g. kidney transplant, orthopedic surgery) during the duration of the study or for whom there are contraindications for training on Galileo vibration platforms according to the official recommendation
2. Adolescents who have started puberty (boys: testicular volume 3 ml, girls: Tanner stage from B2). Since puberty in these patients starts late, the prepubertal age group was extended to the age of 13 years. The pubertal age group was excluded due to the hormonal changes during this period.

Date of first enrolment
01/07/2020

Date of final enrolment
31/08/2020

Locations

Countries of recruitment
Germany

Italy

Study participating centre
Cystinosis Center Rosenheim
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Sponsor information

Organisation
Cystinose Stiftung

Organisation
Cystinosis Research Network

Organisation
Cystinosis Ireland

ROR
https://ror.org/05varta73

Funder(s)

Funder type
Charity

Funder Name
Cystinose Stiftung

Results and Publications

Individual participant data (IPD) sharing plan
The data-sharing plans for the current study are unkown and will be made available at a later date.

IPD sharing plan summary
Data sharing statement to be made available at a later date

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet	Adults (in English)	15/09/2020	21/09/2023	No	Yes
Participant information sheet	Adults (in German)	15/09/2020	21/09/2023	No	Yes
Participant information sheet	Children (in English)	15/09/2020	21/09/2023	No	Yes

Participant information sheet	Children (in German)	15/09/2020	21/09/2023	No	Yes