Empowering children with mitochondrial myopathy in dealing with fatigue.

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Inborn errors of metabolism
Study type	Interventional

Summary

ID

NL-OMON46370

Source
ToetsingOnline

Brief title
PowerMe study

Condition

- Inborn errors of metabolism
- Psychiatric and behavioural symptoms NEC
- Lifestyle issues

Synonym
metabolic disease, mitochondrial diseases, oxidative phosporylation diseases

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum
Source(s) of monetary or material Support: Prinses Beatrix Spierfonds

Intervention

Keyword: Cognitive behavior therapy, E-health, Fatigue, Mitochondrial disease

Outcome measures

Primary outcome

The primary outcome is perceived fatigue.

Secondary outcome

Secondary outcomes in children with mitochondrial disease are quality of life,

physical functioning and school attendance.

Study description

Background summary

Mitochondrial myopathies are heterogeneous in clinical expression, with an unpredictable course ranging from mild to lethal. The disease is often hereditary and the expression can vary from very mild to life threatening pathology. This means children with mitochondrial myopathy and their parents have to deal with uncertainty about the nature and course of the disease. Currently no cure or substantially alleviating therapy is available for these disorders and care is focused on alleviating the broad range of symptoms of the disease. Fatigue is reported as the most burdensome complaint, also affecting quality of life. It results in more school absence which influences their social participation. There are no disease-related interventions to support these patients.
Because of the heterogeneity in genotype and fenotype there are not many systematic studies to investigate the impact of the disease on quality of life. Research is currently ongoing to develop medication that might cure the disease or positively influence the course of the disease. However, developing medication like this takes time. For now, children and parents will still be confronted with the complaints of this disease and no sufficient treatment.

Study objective

The PowerMe study will analyse fatigue characteristics and severity in children and adolescents with mitochondrial myopathy and their families. Then a blended personalized cognitive behaviour therapy program for fatigue will be designed and tested, encompassing several treatment modules. This will result in an

individualized self-management intervention that is tailored to their personal goals and their personal fatigue and quality of life profile as measured in questionnaires. Parents will go through a parallel program, to support their children during the intervention.

Study design

Ten patients will be enrolled in ten single-case experiments, participating in an intervention period preceded by a random time frame waiting period, and as such serving as their own control.
A single-case experiment (SCE) is a rigorous, scientific methodology used to define basic principles of behaviour. Because SCEs documents experimental control within one case, it is an approach, like randomized-control group designs, that can be used to establish evidence-based practices. SCEs are experimental rather than correlational or descriptive, and its purpose is to document causal relationships between independent and dependent variables within one case. It employs within- and between-subject comparisons to control for treats to internal validity and requires replication to enhance external validity. Single-case designs are particularly useful in children with rare diseases when studying rare conditions and large samples are difficult to obtain; results also have direct applicability to healthcare professionals. In addition, interventions could be more optimally adjusted to individual patients.

Intervention

Participants will receive blended cognitive behavioral therapy (CBT) for fatigue in addition to care as usual. The PowerMe intervention covers a period of 4 months in which the patient will have 8 moments of contact with a therapist (three face-to-face sessions and five e-consultations via face-time of 1 hour) with simultaneous use of online treatment modules of the interactive self-management website. Children from 8 to 12 will follow the program with their parents, children from age 12 will follow the program individually with parents following a parallel program. The CBT will be given by trained and experienced therapists working at the department of Medical Psychology, section children and youth, Radboud university medical center (RadboudUMC). They are specialized in working with children, adolescents and their parents.

The PowerMe program is a personalized cognitive behavior therapy program for fatigue encompassing several treatment modules. All participants will start with setting their personal treatment goals. Then, they will work on the fatigue-perpetuating factors that are applicable to them, for example coping with disease aspects, dealing with emotional complaints, and learning adaptive social skills. Each of these fatigue-perpetuating factors will coincide with specific treatment modules. At baseline assessment, it is decided which modules are relevant for each participant and the intervention will be tailored to the

participants* specific needs. Finally, all participants complete the therapy by realizing their treatment goals.

All parents will follow a parallel program in which they can access their own portal on the website where they can access the module*s content of the child modules, psycho-education, and an e-consult application. Parents will have e-mail contact with the therapist wherein results so far will be discussed and assignments can be given. Patients and parents will have separate accounts with unique usernames and passwords, ensuring confidentiality in communication with the therapist. The parents of patients in the age group 8 to 12 years are working together with their child, whereas parents of older patients will be asked to encourage their children to take responsibility for their treatment. Tips and tricks will be given on how to support their child. They also have access to a library with relevant information for parents of a child with mitochondrial disease.

Study burden and risks

There are no or only minimal risks involved in participating in the CBT intervention. The burden is limited and consists of a blended CBT program of 4 months, including extra travelling for 3 sessions, and an online intervention with home-work assignments. During the complete study period patients will complete a short weekly questionnaire. Several other questionnaires are filled out at baseline, post and follow-up measurement. Completing the questionnaires is without risks and the burden is limited. There are substantial potential benefits for participants, as CBT already proved to be a highly effective intervention in reducing fatigue and disabilities for several patient groups and it is likely that patients with mitochondrial disease will also profit and become better in dealing with fatigued. The research cannot be done without participation of this patient group.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Children (2-11 years)

Inclusion criteria

- Age between 8 and 18 years
- Able to speak, write, and read Dutch
- Diagnosed with genetically confirmed mitochondrial disease
- Being severely fatigued (CIS fatigue >= 35)
- Access to a computer with internet connection
- Basic computer skills
- Able to travel to the Radboudumc for the CBT intervention (3 visits)

Exclusion criteria

- Intellectual disability (developmental age younger than 8 years).
- Primary depression (CDI >=16) or anxiety disorder (SCARED-C >= 25)
- No current psychological treatment for fatigue

Study design

Design

Study type: Interventional
Masking: Open (masking not used)

Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	21-04-2019
Enrollment:	10
Type:	Actual

Ethics review

Approved WMO Date:	28-08-2018
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	30-12-2019
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL63537.091.18