VALIDATION OF THE INTERNATIONAL PAEDIATRIC MITOCHONDRIAL DISEASE SCORE

Published: 27-08-2013
Last updated: 22-04-2024

To test the reliability and validity of the IPMDS in children with mitochondrial disorders.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Metabolic and nutritional disorders congenital
Study type	Observational non invasive

Summary

ID

NL-OMON38747

Source
ToetsingOnline

Brief title
INTERNATIONAL PAEDIATRIC MITOCHONDRIAL DISEASE SCORE

Condition

- Metabolic and nutritional disorders congenital

Synonym
disturbed cellular energy metabolism, Mitochondrial disease

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Sint Radboud
Source(s) of monetary or material Support: Zonmw

Intervention

Keyword: Children, Followup, Mitochondrial disease, Scoring systemen

Outcome measures

Primary outcome

The main study endpoints will be the (inter- and intrarater) reliability and

(construct and external) validity of the IPMDS.

Secondary outcome

na

Study description

Background summary

The currently used Newcastle Paediatric Mitochondrial Disease Scale (NPMDS) is designed to follow up patients at the outpatient clinic. It is quick to administer, but thereby lacks detail and is in our opinion therefore not suitable for clinical trials. We designed a new, more detailed scoring system (the International Paediatric Mitochondrial Disease Score (IPMDS)) for follow-up of children with a mitochondrial disorder in clinical studies in which more detail is required.

Study objective

To test the reliability and validity of the IPMDS in children with mitochondrial disorders.

Study design

The study will take place at the outpatient clinic, where four independent physicians involved in the care of patients with mitochondrial disorders will perform the test (interrater reliability). Two physicians will be videotaped and will score the video again after four weeks (intrarater reliability). The construct validity will be calculated by correlating with the previous NPMDS scale, with the Paediatric Evaluation of Disability Inventory (PEDI) score and with the mean severity score (Likert scale 0-10, scored by every physician). External validity and interrater reliability will be tested in several clinics specialized in mitochondrial disorders around the world. Test-retest

reliability will be assessed for the subjective domain only.

Study burden and risks

Patients are asked to visit the outpatient clinic to participate in this study. They will see four physicians, asking approximately the same questions and performing the same physical examination. Since patients with mitochondrial disorder tire easily, this study will be burdensome to them. We will limit the tiredness by pausing regularly. The burden for parents will also be quite high, since they are asked the same questions four to six times that day. We will explain this in our recruitment letter so patients and their parents can decide whether they want to participate.
The risks associated with this study are negligible because no invasive procedures or interventions are involved. Since this study aims to validate a scoring system specific to this group, the study cannot be performed in adults or healthy children. The only benefit from this study is a more complete overview of the patient*s health status than can be obtained at a regular outpatient clinic visit. Therefore, a summary letter of all findings will be written to all physicians caring for the patient.

Contacts

Public
Universitair Medisch Centrum Sint Radboud

Geert Grooteplein 10 route 804
Nijmegen 6500HB
NL
Scientific
Universitair Medisch Centrum Sint Radboud

Geert Grooteplein 10 route 804
Nijmegen 6500HB
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

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

Inclusion criteria

1. 0 to 18 years of age
2. Mitochondrial disorder established by a confirmed mutation in mtDNA or nuclear DNA

Exclusion criteria

- it is estimated that the study will be too burdensome to the patient
- insufficient knowledge of the Dutch language

Study design

Design

Study type: Observational non invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	22-11-2013
Enrollment:	8
Type:	Actual

Ethics review

Approved WMO Date:	27-08-2013
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	16-10-2014
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	NL44833.091.13