Validation of MyCyFAPP as a portable system for self-management in children with CF

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To validate and assess the impact of the use of the MyCyFAPP system for self-management in children with CF, more specifically the effect on patients* quality of life, nutritional status, gastro-intestinal symptoms, general wellbeing and economic...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Chromosomal abnormalities, gene alterations and gene variants
Study type	Interventional

Summary

ID

NL-OMON46610

Source
ToetsingOnline

Brief title
MYCYFAPP, clinical trial

Condition

- Chromosomal abnormalities, gene alterations and gene variants
- Exocrine pancreas conditions

Synonym
cystic fibrosis, pancreas insufficieny

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: Europese unie;horizon 2020 project

Intervention

Keyword: CF Enzymes, education, therapy

Outcome measures

Primary outcome

The primary outcome will be change of modified PedsQL GI questionnaire after 3 months (M3) of APP use. PedsQL GI is the Pediatric Quality of Life score, Gastrointestinal domain Symptoms Module. The questionnaire was validated in childeren with CF in the first phase of the study. Age appropriate questionnaires will be used.

Secondary outcome

A. Impact assessment of APP on health parameters:

* Change in modified PedsQL GI score from baseline (M0) to M6 visit

* Change in health-related quality of life as measured by the CFQ-R (age appropriate version) at M3 and M6 compared to baseline (M0)

* Change in health-related quality of life as measured by the VAS at M3 and M6 compared to baseline (M0)

* Change in FEV1, FVC, FEV1/FVC and FEF25-75 z-scores (lung function) at M3 and M6 compared to baseline.

* Subgroup analysis in patients with low baseline BMI z-scores (<-0.5): change in H, W, BMI z-score at M3 and M6 compared to baseline

* Change in Lipase units used per day and per meal at M3 and M6 compared to M0

* Extra health care visits compared to baseline as a surrogate for economic savings

* Change in food pattern (% of energy of proteins/carbohydrates/fat, % intake

of fruit and vegetables) at M6 compared to M0. Data will be

available from the food record in the APP

* Change in malabsorption of fat between M0 and M1, based on results of stool

collections in a subgroup of patients (substudy).

Change in abdominal symptoms as measured by the CF Abd-Score at M3 and

M6 compared to baseline (M0)

B. Exploratory outcomes considering the user acceptance of the app and the game

APP.

Study description

Background summary

Nutritional status has an enormous impact on the progression of the pulmonary disease in CF, and particular attention has to be devoted to the occurrence of potential imbalance between energy losses (due to pancreatic insufficiency), increased energy needs (due to pulmonary disease) and actual energy intake. Malnutrition and stunting can only be avoided by accurate pancreatic enzyme replacement therapy (PERT) and close nutritional follow up and support. Despite the use of high doses of PERT, many children still have abdominal pain, diarrhea and other gastrointestinal problems and growth may be suboptimal. The developed APP contains a prediction model for optimal PERT dosing for each meal, taking into account the individual needs of the patient. This formula is based on the results of the previous laboratory studies in which the optimal dose of PERT was assessed per food item.

Study objective

To validate and assess the impact of the use of the MyCyFAPP system for self-management in children with CF, more specifically the effect on patients* quality of life, nutritional status, gastro-intestinal symptoms, general wellbeing and economic save. The validation is a crucial stage before implementing the APP in clinical practice.

Objective substudy: evaluation of the developed prediction model for PERT dosing in clinical practice with use of free diet.

Study design

An open label prospective European multicentre interventional study among paediatric CF patients in six reference paediatric CF units of the European Union: Valencia (Hospital Universitari i Politècnic La Fe Valencia* Spain), Madrid (Hospital Universitario Ramón y Cajal Madrid* Spain), Rotterdam (Erasmus University Medical Center * The Netherlands), Milan (Università degli Studi di Milano, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico * Italy), Lisbon (Associação para a Investigação e Desenvolvimento da Faculdade de Medicina Lisboa * Portugal) and Leuven (University Hospital Leuven * Belgium). After a screening visit and obtaining informed consent, 3 study visits will take place (M0, M3 en M6) in a period of 6 months (mostly coinciding with regular outpatient visits) during which the modified 4 questionanires will be obtained and also optional questionnaires about user acceptance of the APP. Height, weight, BMI and lung function will be obtained during the study visits and the nutritional intake will be evaluated (via APP). The intervention (use of the APP on smartphone and/or tablet) will last 6 months. The link of the self-management APP and the professional webtool will allow for online interaction between clinical teams and patients.
Substudy: when participating in the substudy, fat absorption will be determined at M0 and M1 by assessing amount of fat in collected stools in comparison with intake (via nutritional diary in APP).

Intervention

The study intervention consists of use of the MyCyFAPP with its different features during 6 months.

Study burden and risks

The burden of the study for participating patients consists of the use of the app by a smartphone or tablet during 6 months with 3 visits, which will co inside with he regular poli clinic visits. Extra time needed for the study visit will be 40 minutes for filling in 4 questionnaires (PedsQL-GI, CFQr, CF Abd Score and VAS) and optional, 3x a questionnaire about user acceptance of the app. Patients/parents need to follow a training on the use of the app and tablet. During the 6 months patients will be asked to use the app and the suggested amount of creon daily for one month, the other 5 months at least for 2x a week and fill in a food diary 2x during 3 days at the beginning and the end of the study.If patient takes part of the sub study (which is optional) they will be asked to collect feces 2x during 48hrs.
Participating in this study can increase knowledge on the optimal use of creon which will lead to less abdominal pain or diarrhea and in the long term a

positive effect on growth and quality of life. It will also increase knowledge on CF and healthy food. disadvantage of participating is the time it will take and the advised amount of creon could lead to more abdominal pain.

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

1. Diagnosis of CF as evidenced by one or more clinical feature consistent with the CF phenotype or positive CF newborn screen AND one or more of the following criteria:
a) A documented sweat chloride * 60 mEq/L by quantitative pilocarpine iontophoresis (QPIT)
b) A documented genotype with two disease-causing mutations in the CFTR gene
2. Having pancreatic insufficiency (stool elastase < 200 mcg/g stool) and using PERT
3. Age * 24 months and < 18 years at Screening visit

4. Informed consent by parent or legal guardian; assent for children from age 12 years on
5. Inclusion visit coincides with scheduled routine clinic visit
6. Ability and willingness to comply with APP use and evaluations at time of routine clinic visits as judged by the site investigator
7. Availability of wifi connection at home so that connection to the internet is feasible at home at least weekly.

Exclusion criteria

1. Acute infection associated with decreased appetite or fever at time of run-in visit
2. Acute abdominal pain necessitating an intervention at time of run-in visit
3. Physical findings that would compromise the safety of the participant or the quality of the study data as determined by site investigator
4. Investigational drug use within 30 days prior to run-in visit
5. Started with CFTR modulator treatment less than 3 months before start of run-in visit
6. Inability to use APP due to patient specific factors such as language or educational issues

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):	05-02-2018
Enrollment:	30
Type:	Actual

Ethics review

Approved WMO

Date:	03-01-2018

Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	14-03-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	11-09-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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
Other	clinical trials.gov, nummer volgt
CCMO	NL63421.078.17