ISRCTN57423639 https://doi.org/10.1186/ISRCTN57423639

Pancreatic exocrine replacement therapy in severely malnourished children

Submission date	Recruitment status	[_] Prospectively registered
23/01/2014	No longer recruiting	[_] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
14/04/2014	Completed	[X] Results
Last Edited	**Condition category**	[_] Individual participant data
18/09/2017	Nutritional, Metabolic, Endocrine	

Plain English summary of protocol

Background and study aims
Every day, 24000 children under five die of which more than one third of deaths are associated with malnutrition. Malnourished children have an inability to properly digest food because they lack digestive enzymes made by the pancreas, an abdominal digestive organ. These enzymes are used to break down fats, proteins and carbohydrates into units that can be digested. Loss of digestive enzymes leads to incomplete digestion and insufficient absorption of nutrients. In other diseases with these characteristics, like Cystic Fibrosis, improving this maldigestion with pancreatic enzyme replacement therapy (PERT) is very common. The goal of PERT is to restore normal digestion, achieve adequate nutritional status and to reach a normal pattern of growth. However, it is not known whether exocrine pancreatic dysfunction in childhood severe malnutrition can be corrected by PERT and will lead to additional weight gain. The aim of this study is to test whether PERT results in additional weight gain in severely malnourished children.

Who can participate?
Severely malnourished children, aged between 6 and 60 months, admitted to the ward dedicated to malnourished children: MOYO House or Moyo Nutritional Rehabilitation Unit. Both HIV positive and negative children can participate.

What does the study involve?
100 children are randomly allocated into two groups: the group getting the enzymes (PERT) and the control group. Both groups are treated according to the current departmental refeeding guidelines that are based on what the World Health Organization recommends. The group getting PERT receive this during a four-week period.

What are the possible benefits and risks of participating?
Several benefits are present for participants. A special study team will follow and take care of the children during the entire admission to MOYO. The study information provided to the parents creates an opportunity to learn more about the nutritional problem and the health of their child. No more than the maximum recommended daily dose per kilogram bodyweight, as is currently practiced in children with Cystic Fibrosis, will be prescribed. Mild discomfort and minimal bleeding is related to the venous blood drawing.

Where is the study run from?
The study takes place in MOYO Nutritional Rehabilitation Unit, Paediatric Department, Queen Elizabeth Central Hospital, Blantyre, Malawi.

When is the study starting and how long is it expected to run for?
February 2014 to August 2014

Who is funding the study?
Stichting Steun Emma Kinderziekenhuis (Foundation Support at the Emma Childrens Hospital in Amsterdam) (Netherlands)

Who is the main contact?
Dr Wieger P. Voskuijl

Contact information

Type(s)
Scientific

Contact name
Dr Wieger Voskuijl

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N/A

Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
P.11/12/1306

Study information

Scientific Title
The OPTIMISM trial: pancreatic exocrine replacement therapy in severely malnourished children: a pilot study

Acronym
OPTIMISM

Study objectives
Exocrine pancreatic dysfunction in childhood severe malnutrition can be corrected by pancreatic enzymatic replacement therapy and will lead to enhanced weight gain and ultimately to a reduced morbidity and mortality.

Ethics approval required
Old ethics approval format

Ethics approval(s)
College of Medicine Research Ethics Committee, University of Malawi, Blantyre, Malawi, 02/10 /2013, P.11/12/1306

Study design
Prospective randomised single-blinded study

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details to request a patient information sheet

Health condition(s) or problem(s) studied
Severe acute malnutrition in children

Interventions
Children are randomised to two groups: receiving pancreatic enzyme replacement therapy (PERT) or standard treatment. All children admitted to MOYO have a thick blood film examined for parasitaemia and a haematocrit measured. They are all offered an HIV antibody test with appropriate pre- and post counselling. After admission children will be treated according to the current departmental refeeding guidelines that are WHO based. The intervention will be given during a four-week period. PERT is prescribed as 3000 Units Lipase/kilogram bodyweight 3 times per day with an upper limit dose of 10,000 Units Lipase/kg bodyweight per day 35. If the child is discharged before this period, parents will be given transport money to return to the hospital after the four-week period has ended.

Intervention Type
Other

Phase
Not Applicable

Primary outcome measure
1. To compare the percentage of weight gain after 28 days of PERT with the current standard of care (calculated using the final weight at 28 days and the minimum weight during admission)
2. An additional 10% increase in weight will be considered clinically relevant

Secondary outcome measures
The differences after 28 days of PERT compared to the current standard of care with respect to:
1. Improvement in exocrine pancreatic function assessed by increased serum cationic and faecal elastase-1 levels
2. Decreased time on the ward in the PERT group
3. Overall mortality by 4 weeks in both groups

Overall study start date
10/02/2014

Completion date
01/08/2014

Eligibility

Key inclusion criteria
1. Severely malnourished children
2. Aged between 6 and 60 months
3. Admitted to MOYO House
4. Diagnosed with kwashiorkor, and/or marasmus (WHO criteria)
5. Both HIV positive and negative children

Participant type(s)
Patient

Age group
Child

Lower age limit
6 Months

Upper age limit
60 Months

Sex
Both

Target number of participants
100

Key exclusion criteria

1. Malaria (as proven by a positive blood smear)
2. Sepsis (i.e. circulatory failure)
3. Severe pneumonia, vomiting or severe diarrhoea
4. Those whose parent/guardian has not given consent

Date of first enrolment
10/02/2014

Date of final enrolment
01/08/2014

Locations

Countries of recruitment
Malawi

Study participating centre
College of Medicine
Blantyre
Malawi
N/A

Sponsor information

Organisation
Emma Children's Hospital Foundation Support (Stichting Steun Emma Kinderziekenhuis AMC) (Netherlands)

Sponsor details
Meibergdreef 9 (TKs0-253)
Amsterdam
Netherlands
1105 AZ

Sponsor type
Research organisation

Website
http://www.steunemma.nl/site/de-stichting/

ROR
https://ror.org/00bmv4102

Funder(s)

Funder type
Hospital/treatment centre

Funder Name
Emma Children's Hospital Foundation Support (Stichting Steun Emma Kinderziekenhuis) (Netherlands)

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	01/11/2017		Yes	No