

Neurology and enzyme therapy in MODY8

Submission date 21/11/2007	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
Registration date 04/12/2007	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 10/06/2021	Condition category Nutritional, Metabolic, Endocrine	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Study information

Scientific Title
Neurology and enzyme therapy in MODY8

Study objectives
The MODY8 syndrome is a monogenically inherited syndrome of diabetes and pancreatic exocrine dysfunction due to single-base deletion mutations in the Carboxyl-Ester Lipase (CEL) gene Variable Number of Tandem Repeats (VNTR), registered in OMIM as MODY8 or DPED.

Hypotheses:

That pancreatic enzyme substitution therapy will:

1. Ameliorate exocrine function as reflected by fecal fat excretion and fat-soluble vitamin status
2. Improve glycemic control as measured by HbA1c
3. Improve neuropathology in patients with the MODY8 syndrome

Ethics approval required

Old ethics approval format

Ethics approval(s)

The Regional Committee for Research Ethics of Western Norway (REK Vest), approved on 11 November 2004, (ref: REK Vest 209.04)

Study design

Open, non-randomized, single-center, interventional study.

Primary study design

Interventional

Study type(s)

Treatment

Health condition(s) or problem(s) studied

MODY8 syndrome

Interventions

All participants initially received one Creon enterocapsule (Solvay Pharmaceuticals, Germany) containing 10,000 units lipase, 18,000 units amylase, 600 units protease three times daily, orally at meals. If the clinical effect was unsatisfactory based on patient symptoms, the dose was first increased to 1-2 capsules 3-4 times daily, and if the clinical effect was still unsatisfactory, the medication was changed to Creon Forte, taken orally with meals, 1-2 capsules per meal.

Intervention Type

Drug

Phase

Not Specified

Drug/device/biological/vaccine name(s)

Creon enterocapsule and Creon Forte, Creon enterocapsule and Creon Forte

Primary outcome(s)

1. Fecal elastase-1, assessed at 0, 6, 12 and 30 months
2. Fecal fat excretion, assessed at 0, 12 and 30 months
3. HbA1c (blood test), assessed at 0, 6, 12 and 30 months
4. Vitamins A, D and E (blood test), assessed at 0, 6, 12 and 30 months
5. Creatinine (blood test), assessed at 0, 6, 12 and 30 months
6. Total calcium (blood test), assessed at 0, 6, 12 and 30 months
7. Total High Density Lipoprotein (HDL) and Low Density Lipoprotein (LDL) cholesterol (blood test), assessed at 0, 6, 12 and 30 months
8. Triglycerides (blood test), assessed at 0, 6, 12 and 30 months

9. C-peptide (blood test), assessed at 0, 6, 12 and 30 months
10. Bone mass density (age-matched Z-scores), assessed at 0, 12 and 30 months
11. Visual evoked potential, assessed at 0 and 18 months
12. Sensory evoked potential, assessed at 0 and 18 months
13. Nerve conduction velocity, assessed at 0 and 18 months

Key secondary outcome(s)

No secondary outcome measures

Completion date

30/06/2007

Eligibility

Key inclusion criteria

Participants should:

1. Be a carrier of a single-nucleotide deletion mutation in the CEL VNTR
2. Have diabetes by the World Health Organization criteria
3. Have exocrine dysfunction defined by fecal elastase <200 micrograms/ml in two consecutive tests
4. Patients of both sexes and all ages should be included

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Not Specified

Sex

All

Total final enrolment

9

Key exclusion criteria

1. Ongoing treatment with pancreatic enzyme supplements
2. Inability to attend clinical examinations and other necessary investigations for geographical reasons
3. Side effects of medication (strong stomach ache)

Date of first enrolment

01/09/2004

Date of final enrolment

30/06/2007

Locations

Countries of recruitment

Norway

Study participating centre

Section for Paediatrics

Bergen

Norway

5021

Sponsor information

Organisation

University of Bergen (Norway)

ROR

<https://ror.org/03zga2b32>

Funder(s)

Funder type

University/education

Funder Name

Haukeland University Hospital, Innovest, University of Bergen (Norway)

Funder Name

The Norwegian Research Council (FUGE Program)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration

Study outputs

Output type
[Results article](#)

Details

Date created
01/09/2008

Date added
10/06/2021

Peer reviewed?
Yes

Patient-facing?
No