

# Neurology and enzyme therapy in MODY8

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<b>Registration date</b> 04/12/2007	<b>Overall study status</b> Completed	<input type="checkbox"/> Protocol
<b>Last Edited</b> 10/06/2021	<b>Condition category</b> 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

### Contact name

Prof Pal Njolstad

### Contact details

Section for Paediatrics  
Department of Clinical Medicine  
Haukeland University Hospital  
Bergen  
Norway  
5021  
+47 55 97 51 53  
pal.njolstad@pedi.uib.no

## Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers

N/A

## 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

**Secondary study design**

Non randomised controlled trial

**Study setting(s)**

Not specified

**Study type(s)**

Treatment

**Participant information sheet****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 measure**

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

**Secondary outcome measures**

No secondary outcome measures

**Overall study start date**

01/09/2004

**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

**Age group**

Not Specified

**Sex**

Both

**Target number of participants**

16

**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)

**Sponsor details**

Faculty of Medicine

Post Box 7804

Bergen

Norway

5020

+47 55 58 20 86

post@medfa.uib.no

**Sponsor type**

University/education

**Website**

<http://www.uib.no/info/english/>

**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

**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?
<a href="#">Results article</a>		01/09/2008	10/06/2021	Yes	No