Using D-Galactose as a food supplement in Congenital Disorders of Glycosylation (CDG)

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The goal of this study is to better characterize the metabolic alterations and sugar structure alterations (glycosylation abnormalities) in patients diagnosed with Congenital Disorders of Glycosylation.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Inborn errors of metabolism
Study type	Interventional

Summary

ID

NL-OMON44612

Source
ToetsingOnline

Brief title
Using Galactose in CDG

Condition

- Inborn errors of metabolism

Synonym
CDG, Congential Disorder of Glycosylation

Research involving
Human

Sponsors and support

Primary sponsor: UZ Leuven
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: Congenital Disorders of Glycosylation, Galactose, Prospective study

Outcome measures

Primary outcome

Efficacy: Change of glycosylation in blood (sialotransferrin isoforms)

Secondary outcome

Compliance intake of galactose

Study description

Background summary

Congenital Disorders of Glycosylation (CDG) is a recently discovered metabolic disorder that changes the way sugars are broken down and disrupts how these sugars are built in a person*s body1. Congenital Disorders of Glycosylation (CDG) leads to severe metabolic alterations in patients. These include hypoglycemia (low blood sugar levels), abnormal liver function, abnormal hormone levels, and muscle symptoms2,3,4. The symptoms occur due to changes in the normal structure and attachment of the sugar chains that are built on the surface of body proteins and will stay attached to the proteins in a healthy individuals body5,6,7. The development of effective dietary interventions for Congenital Disorders of Glycosylation is limited because of our poor understanding of the disease. Galactose, the simple milk sugar has been applied as a dietary supplement, and in high dosage (50g/dose) has been used in clinical loading tests for more then 50 years, without side effects. Increased intake of galactose, has previously shown beneficial effects on the sugar chain structure on body proteins (this is called glycosylation) initially in 6 patients with PGM1 deficiency8. The positive effect of galactose was proven in cell culture studies of patients as well8. Since then patients with other types of CDGs have been trialed on galactose supplements with succes

Study objective

The goal of this study is to better characterize the metabolic alterations and sugar structure alterations (glycosylation abnormalities) in patients diagnosed with Congenital Disorders of Glycosylation.

Study design

Over a two-year period, we will enroll patients diagnosed with Congenital Disorders of Glycosylation. We propose to administer oral galactose supplementation for a period of 18 weeks in increasing dose to assess its effectiveness at normalizing glycosylation. Galactose will be given in a series of doses within the range of normal dietary intake of galactose over fixed time points. To assess the effects of oral galactose supplementation for each participant, changes in participant growth, as well as blood sugar levels, coagulation parameters and liver function (the primary clinical features of Congenital Disorders of Glycosylation) will be correlated with biomarkers derived from participant blood and urine samples obtained at key time points and then compared to standard normative ranges of data for each measure.

Intervention

1The participant will remain on his/her regular diet and will be asked to ingest an oral galactose (simple milk sugar) supplement. The amount of galactose in the supplement will be not more than what is found in a recommended healthy diet. The maximum galactose dose used is 50g/day. This dosage has been shown to be safe when used in healthy individuals in oral or venous administration, and when taken by mouth for several weeks9,10,13,14.

This oral supplement, *D-Galactose*, is a simple tasteless powder measured by spoon (Necaseo) and will be taken by mouth. The galactose dosage will be increased in three increments as follows throughout the 18 weeks of the study period: 0.5 g galactose per kg (first 6 weeks), 1.0 g per kg (weeks 7-12), and 1.5 g per kg (weeks 13-18) (maximum daily dose : 50g).

Study burden and risks

At this moment there is no treatment for patients with CDG. All patients with CDG have a (severe) cognitive imairment. Burden and risks are acceptable in our opinion in relation to the progeressive charcater of the CDG. A recently published pilot study shows that galactose is safe and tolerated in CDG1A patients. (Marova et al., 2017)

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Children (2-11 years)

Inclusion criteria

Patient is younger than 21 years old
Patient has a biochemically and genetically proven Congenital Disorders of Glycosylation.
No galactose intake last 6 weeks

Exclusion criteria

Patient has any of the following conditions:
Aldolase B deficiency
Galactosemia
Hemolytic uremic syndrome
Severe anemia
Diagnosis of intellectual disability or developmental delay
Galactose Intolerance

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	13-12-2017
Enrollment:	1
Type:	Actual

Ethics review

Approved WMO

Date:	07-12-2017
Application type:	First submission
Review commission:	METC Amsterdam UMC

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	NL61943.018.17