GBA-PD Patient Imaging study of LTI-291 versus placebo.

No registrations found.

Ethical review	Positive opinion
Status	Recruitment stopped
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON24854

Source
Nationaal Trial Register

Health condition

Parkinson's disease

Sponsors and support

Primary sponsor: Lysosomal Therapeutics Inc.
Source(s) of monetary or material Support: Lysosomal Therapeutics Inc.

Intervention

Outcome measures

Primary outcome

To evaluate the safety and tolerability of two oral dose levels of LTI-291 (10 and 60 mg) following 28 days of treatment in patients with GBA-PD.

Secondary outcome

To characterize the plasma PK of LTO-291 (10 and 60 mg) in patients with GBA-PD.

Study description

Background summary

Background of the study: LTI-291 is a new compound that may eventually be used for the treatment of patients with Parkinson’s disease who have a GBA1 mutation. LTI-291 is an activator of an enzyme named glucocerebrosidase (GCase). Everyone has this enzyme, but

studies have shown that in a subgroup of patients with Parkinson’s disease who have a mutation in a specific gene called

glucocerebrosidase 1 (GBA1), there is a deficiency of GCase which results in build-up of certain substrates of this enzyme

in the cells. LTI-291 acts by increasing the activity of the enzyme GCase to a normal level and thus leads to a decrease in

the build-up of substrates.

Objective of the study:

This study will be performed in patients with Parkinson’s disease who have a GBA1 mutation.

The purpose of this study is to investigate how safe the new compound LTI-291 is and how well it is tolerated when it is

administered to patients with Parkinson’s disease. It will also be investigated how quickly and to what extent LTI-291 is

absorbed and eliminated from the body (this is called pharmacokinetics). In addition, the effect of LTI-291 on the body (on

the brain and on certain biomarkers in the blood) will be investigated (this is called pharmacodynamics). LTI-291 will be compared with a placebo.

Study design

28 days.

Intervention

Multiple doses of LTI-291 10 or 60 mg once daily for 28 days.

Contacts

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Eligibility criteria

Inclusion criteria

Mannelijke of vrouwelijke vrijwilliger; ziekte van Parkinson met een GBA1 mutatie; minimaal 18 jaar; BMI 18.0 - 35.0 (kilogram/meter2); gewicht minstens 45 kilogram.

Exclusion criteria

Lijdend aan hepatitis B, diabetes, hepatitis C, kanker of HIV/AIDS. Indien gedurende de 90 dagen voorafgaand aan de start van dit onderzoek aan een ander geneesmiddelenonderzoek is deelgenomen. Indien gedurende de 60 dagen voor start van dit onderzoek bloed is gegeven.

Study design

Design

Study type:	Interventional
Intervention model:	Other
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	05-06-2018
Enrollment:	15
Type:	Actual

Ethics review

Positive opinion

Date:	30-05-2018
Application type:	First submission

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
NTR-new	NL7061
NTR-old	NTR7299
Other	LSO882EC-178821 : LTI-291-004

Study results