Phase 1 open-label, crossover study to evaluate the effect of food on the pharmacokinetics and safety of a single oral dose of PTC518 in healthy volunteers.

Published: 17-01-2022
Last updated: 27-12-2024

In this study we will investigate what the effect of food is on how the study compound PTC518 is absorbed, transported, and eliminated from the body.We will also investigate how safe the new compound PTC518 is and how well it is tolerated when it is...

Ethical review	Approved WMO
Status	Completed
Health condition type	Neurological disorders NEC
Study type	Interventional

Summary

ID

NL-OMON51584

Source
ToetsingOnline

Brief title
PTC518 food effect study.

Condition

- Neurological disorders NEC

Synonym
brain disease, Huntington's disease

Research involving
Human

Sponsors and support

Primary sponsor: PTC Theraputics, Inc.
Source(s) of monetary or material Support: Pharmaceutical industry

Intervention

Keyword: Food, Pharmacokinetics, PTC518, Safety

Outcome measures

Primary outcome

Characterize the effect of food on the PK of PTC518 administered to healthy

subjects under fed and fasted conditions.

Secondary outcome

Characterize the safety and tolerability of a single dose of PTC518

administered to healthy subjects under fed and fasted conditions.

Study description

Background summary

PTC518 is a new compound that may potentially be used for the treatment of Huntington*s disease. Huntington*s disease is an inherited disease in the brain where nerve cells are broken down. This causes problems with movement and thinking. Huntington*s disease is caused by an error in the genetic information (DNA) that results in a harmful version of a protein called huntingtin. This version of the huntingtin protein damages brain cells. Reducing the amount of huntingtin is a potential way to treat Huntington*s disease. The study compound PTC518 aims to reduce the production of the huntingtin protein.

Study objective

In this study we will investigate what the effect of food is on how the study compound PTC518 is absorbed, transported, and eliminated from the body.

We will also investigate how safe the new compound PTC518 is and how well it is tolerated when it is used by healthy participants.

PTC518 has been used by humans before. In addition, it has been extensively tested in the laboratory and on animals.

Study design

For the study it is necessary that the volunteer stays in the research center for 2 periods of 5 days (4 nights). The volunteer will also come back to the research center for a visit after leaving each period (Day 11 [±3 days] and Day 32 [±3 days]).

Day 1 and Day 22 are the days that the volunteer will receive the study compound. The volunteer will be expected at the research center the day before the day of intake of the study compound. The volunteer will leave the research center after 4 nights in the research center (Day 4 and Day 25).

There will be 3 weeks between dosings.

Below is an overview of the days the volunteer stays at the research center, or when they visit the research center.

Screening:
Day -28 up to Day -2

Period 1:
Arrival on Day -1
In house stay from Day -1 up to Day 4
Departure on Day 4
Visit on Day 11 (±3 days)

Period 2:
Arrival on Day 21
In house stay from Day 21 up to Day 25
Departure on Day 25
Visit on Day 32 (±3 days)

Follow-up phone call:
Day 35

The volunteer will be given PTC518 as an oral tablet with 240 milliliters (mL) of (tap) water.

Prior to dosing the volunteer has to fast for at least 10 hours. During fasting, water is allowed, except 1 hour prior to until 1 hour after each intake of the study compound. Depending on what group the volunteer is in, they will either get a high-fat breakfast, low-fat breakfast, or no breakfast prior to dosing in each period. The volunteers will get 2 of these 3 possibilities.

There are 6 treatment sequences possible. Which sequence you will follow will be determined by drawing lots:

Sequence 1 = during period 1 a low-fat breakfast and during period 2 a high-fat breakfast
Sequence 2 = during period 1 a high-fat breakfast and during period 2 a low-fat breakfast
Sequence 3 = during period 1 a low-fat breakfast and during period 2 fasting
Sequence 4 = during period 1 fasting and during period 2 a low-fat breakfast
Sequence 5 = during period 1 a high-fat breakfast and during period 2 fasting
Sequence 6 = during period 1 fasting and during period 2 a high-fat breakfast

If dosing is done after breakfast, the breakfast must be started exactly on time and must be finished within 20 minutes. The entire breakfast must be consumed.

Hands and mouth of the volunteers will be inspected after the study compound intake. This it to check if they have taken the study compound.

Intervention

The volunteer will receive 20 milligram of PTC518 once per period, so in total 2 times.

Study burden and risks

Blood draw:
Drawing blood may be painful or cause some bruising. The use of the indwelling cannula can sometimes lead to inflammation, swelling, hardening of the vein, blood clotting, and bleeding in the environment of the puncture site. In some individuals, a blood draw can sometimes cause pallor, nausea, sweating, low heart rate, or drop in blood pressure with dizziness or fainting.
In total, we will take about 109 mL of blood from screening to follow-up. This amount does not cause any problems in adults. To compare: a blood donation involves 500 mL of blood being taken at once each time. If the investigator thinks it is necessary for the safety of a participant, extra samples might be taken for possible additional testing. If this happens, the total amount of blood drawn may be more than the amount indicated above.

Heart tracing:
To make a heart tracing, electrodes will be placed on arms, chest and legs. Prolonged use of these electrodes can cause skin irritation.

Fasting:
If someone has to fast for a prolonged time during the study, this may lead to symptoms such as dizziness, headache, stomach upset, or fainting.

Coronavirus test:
Samples for the coronavirus test will be taken from the back of the nose and throat using swabs. Taking the samples only takes a few seconds, but can cause discomfort and can give an unpleasant feeling. Taking a sample from the back of the throat may cause gagging. When the sample is taken from the back of the nose, The volunteer may experience a stinging sensation and eyes may become watery.

Contacts

Public
PTC Theraputics, Inc.

Corporate Court 100
South Plainfield 07080 NJ
US
Scientific
PTC Theraputics, Inc.

Corporate Court 100
South Plainfield 07080 NJ
US

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)

Inclusion criteria

1. Subjects must understand the nature of the study and must provide signed and dated written informed consent before the conduct of any study-related procedures and comply with all protocol requirements.
2. Sex: Male or female

3. Age: 18 to 65 years, inclusive, at Screening.
4. Body mass index (BMI): >=18.0 to 30.0 kg/m2 with a body weight >=50.0 kg for male subjects and a body weight >=45.0 kg for female subjects at Screening.
5. Healthy as determined by the investigator or designee, based upon a medical evaluation including medical history, physical examination, laboratory test results, ECG recording (eg, a corrected QT interval using Fridericia*s formula [QTcF] <=450 msec for males and QTcF <=470 msec for females), and vital signs at Screening. Out-of-range values can be repeated once.

Further criteria apply, see protocol.

Exclusion criteria

1. Employee of PRA Health Sciences (PRA) or PTC.
2. History of hypersensitivity reactions to any excipients in the study drug and/or food allergies.
3. History of malignancy within 5 years, unless one of the following, treated and considered cured: basal cell carcinoma, in situ cervical cancer, or breast ductal carcinoma in situ.
4. Previously randomized in this clinical trial.
5. Veins unsuitable for repeated venipuncture or for cannulation.

Further criteria apply, see protocol.

Study design

Design

Study type:	Interventional
Intervention model:	Crossover
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	26-04-2022
Enrollment:	24

Type:	Actual

Ethics review

Approved WMO Date:	17-01-2022
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	25-02-2022
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)

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
EudraCT	EUCTR2021-006463-23-NL
CCMO	NL80261.000.22

Study results

Date completed:	01-07-2022
Results posted:	25-04-2023

First publication
27-02-2023