Interventional, open-label, single-group, multiple-dose study investigating the pharmacokinetic properties of arimoclomol (BRX-345) and its metabolites following oral administration to healthy young men

Published: 05-09-2018
Last updated: 10-01-2025

Primary objective: To investigate the pharmacokinetics (PK) of multiple doses arimoclomol in plasma en urine.To investigate and quantify metabolites of arimoclomol in plasma and urine (reported seperately)Secondary objective: To evaluate the safety...

Ethical review	Approved WMO
Status	Completed
Health condition type	Other condition
Study type	Interventional

Summary

ID

NL-OMON45787

Source
ToetsingOnline

Brief title
CS0305 (OR-ARI-MET-01)

Condition

- Other condition

Synonym
ALS, Gaucher's disease

Health condition

diseases regarding conditions that involve misfolded proteins or protein aggregation disorders

Research involving
Human

Sponsors and support

Primary sponsor: Orphazyme A/S
Source(s) of monetary or material Support: Orphazyme A/S

Intervention

Keyword: Arimoclomol, BRX-345, Pharmacokinetic

Outcome measures

Primary outcome

Pharmacokinetic parameters of arimoclomol including Cmax, tmax, AUC0-t, AUC0-8, AUC0-inf, CL/F, Vz/F, and t*, Cavg, AR, Ae, fe and CLR after multiple dose administration in healthy young male subjects.

Secondary outcome

Safety parameters include: adverse events (AEs), clinical laboratory assessments, vital signs, weight, ECG, physical and neurological examination and C-SSRS scores after multiple dose administration in healthy young male subjects.

Study description

Background summary

The heat shock response is a natural defense mechanism in all cells. It protects the cells from accumulation of misfolded proteins or other waste products, which would otherwise lead to toxicity and disease. Arimoclomol is an orally available small molecule that readily crosses the blood brain barrier to stimulate an increased production of HSP, in particular HSP70, in stressed

cells. Arimoclomol may therefore be used to treat conditions that involve misfolded proteins, including lysosomal storage disease (LSD) (for example Niemann Pick disease, type C (NPC) and Gaucher's disease (GD)) and protein aggregation disorders (for example amyotrophic lateral sclerosis (ALS) and sporadic inclusion body myositis (sIBM)).

Study objective

Primary objective:
To investigate the pharmacokinetics (PK) of multiple doses arimoclomol in plasma en urine.
To investigate and quantify metabolites of arimoclomol in plasma and urine (reported seperately)

Secondary objective:
To evaluate the safety and tolerability of multiple doses arimoclomol.

Study design

This study is an interventional, open-label, multiple-dose study in healthy young men.

Intervention

All subjects will receive multiple doses of 400 mg arimoclomol three times a day (TID) for 5 consecutive days and a single morning dose on Day 6.

Study burden and risks

Since this study is being executed in healthy volunteers. There are no anticipated benefits of the IMP. Please see the IMPD for further information.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

subject is a man
subject is older than or 18 and younger than or 45 years of age at Screening
For more inclusion criteria, please refer to the protocol

Exclusion criteria

The subject has a history of severe drug allergy or hypersensitivity.
The subject has taken any investigational medicinal product within 3 months prior to the first dose of IMP.
For more exclusion criteria, please refer to the protocol

Study design

Design

Study type: Interventional

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	18-09-2018
Enrollment:	6
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Arimoclomol
Generic name:	n.a.

Ethics review

Approved WMO Date:	05-09-2018
Application type:	First submission
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
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Application type:	First submission
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)

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	EUCTR2018-002517-34-NL
CCMO	NL66925.056.18

Study results

Date completed: 06-11-2018

Results posted: 12-11-2020

First publication
28-05-2020