A first-in-human, randomized, dose-Escalation, double-blind, placebo-controlled study to assess safety, tolerability and pharmacokinetics of APX001 administered by intravenous infusion to healthy subjects.

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Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Autoimmune disorders
Study type	Interventional

Summary

ID

NL-OMON46244

Source
ToetsingOnline

Brief title
APX001 First in human study.

Condition

- Autoimmune disorders

Synonym
Fungal diseases.

Research involving

Human

Sponsors and support

Primary sponsor: Amplyx Pharmaceuticals, Inc.
Source(s) of monetary or material Support: Farmaceutische industrie.

Intervention

Keyword: APX001, Fungal infections., GWT1

Outcome measures

Primary outcome

To evaluate the safety, tolerability, and pharmacokinetics of single and multiple doses of APX001 administered by intravenous infusion in healthy volunteers.

Secondary outcome

1. Determine the maximum tolerated dose.

2. Explore the APX001 safety profile in relation to the duration of infusion observed at APX001A target plasma exposures (AUC24) required for clinical efficacy.

3. Explore the APX001 dose and dose regimen required to attain APX001A target plasma exposures (AUC24) required for clinical efficacy against Candida, Aspergillus and the hard-to-treat rare Molds (Scedosporium, Fusarium and Mucorales) invasive fungal infections.

Study description

Background summary

APX001 is a new investigational compound that may eventually be used for the

treatment of fungal infections. APX001 binds to an enzyme specific for fungi (GWT1) and decreases the activity of this enzyme. As a result the cell walls of the fungus will lose integrity and the growth will slow or stop. This is the first time that this study compound is being given to humans.

Study objective

The study will be performed in 4 parts, Parts A, B, C and D. The purpose of the study is to investigate to what extent APX001 is tolerated.
It will also be investigated how quickly and to what extent APX001 is absorbed and eliminated from the body (this is called pharmacokinetics).
This study will be performed in 120 healthy male and female volunteers, divided over 12 groups.

Study design

The actual study will consist of 1 period during which the volunteers will stay in the clinical research center in Groningen (location UMCG for Cohort 1 to 3 and location Martini for Cohort 4 to 6) for 4 days (3 nights). This stay will be followed by short visits to the clinical research center on Days 4 to 8 and on Day 11, 12, or 13. During the visit on Day 8 the post-study screening will be performed.
The volunteers are expected at the clinical research center at 14:00 h in the afternoon prior to the day of administration of the study compound. They will be required not to have consumed any food or drinks during the 4 hours prior to arrival in the clinical research center (with the exception of water). They will leave the clinical research center on Day 3 (Day 1 is the day of administration of the study compound).
The post-study screening will take place on Day 7 - 9 . The appointment for the post-study screening will be made during the study.
The participation to the entire study, from the pre-study screening until the post study screening, will be a maximum of 36 days.

The actual study for cohorts 7-10 will consist of 1 period during which subjects will stay in the clinical research center in MZH in Groningen for 17 days (16 nights). This stay will be followed by a short visit to the clinical research center on Day 18 and for the post-study screening on Day 21.
The participation for to the entire study, from the pre-study screening until the post-study screening, will be a maximum of 49 days.

Part C:
The actual study will consist of 1 period during which the volunteer will stay in the clinical research center in Groningen (location Martini) for 4 days (3 nights). This stay will be followed by short visits to the clinical research center on Days 4 to 8 (Day 1 is the day of administration of the study compound) and on Day 11, 12, or 13. During the visit on Day 8 the post study

screening will be performed.
The volunteers are expected at the clinical research center at 14:00 h in the afternoon prior to the day of administration of the study compound. They will be required not to have consumed any food or drinks during the 4 hours prior to arrival in the clinical research center (with the exception of water). They will leave the clinical research center on Day 3.
The post-study screening will take place on Day 8.
The participation to the entire study, from the pre-study screening until the post study screening, will be a maximum of 41 days.

Part D:
The actual study will consist of 1 period during which the volunteers will stay in the clinical research center in Groningen for 10 days (9 nights). This stay will be followed by short visits to the clinical research center on Day 11 and Day 14 (Day 1 is the first day of administration of the study compound). They are expected at the clinical research center at 14:00 h in the afternoon prior to the day of first administration of the study compound. They will be required not to have consumed any food or drinks during the 4 hours prior to arrival in the clinical research center (with the exception of water). They will leave the clinical research center on Day 9. The post-study screening will take place 7 days after the last administration of study compound on Day 14. The participation to the entire study, from the pre-study screening until the post-study screening, will be a maximum of 42 days.

Intervention

SAD:
Cohort 1: 1 x 10 mg
Cohort 2: 1 x 30 mg
Cohort 3: 1 x 100 mg
Cohort 4: 1 x 300 mg
Cohort 5: 1 x 525 mg
Cohort 6: 1 x 800 mg

MAD:
Cohort 7: 1 x 50 mg
Cohort 8: tbd x tbc mg
Cohort 9: tbc x tbc mg
Cohort 10: tbc x tbc mg
Cohort 11: tbd
Cohort 12: tbd

Study burden and risks

Pain, minor bleeding, bruising, possible an infection due to blood sampling.

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

Healthy male or female volunteers
18 - 55 years, inclusive
BMI 18.0 - 30.0 kilogram/meter2
non smoking

Exclusion criteria

Suffering from hepatitis B, hepatitis C, cancer or HIV/AIDS. In case of participation in another drug study within 90 days before the start of this study or being a blood donor within 60 days from the start of the study. In case of donating more than 1.5 liters of blood in the 10 months prior the start of this study.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	19-05-2016
Enrollment:	120
Type:	Actual

Ethics review

Approved WMO Date:	26-04-2016
Application type:	First submission
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	12-05-2016
Application type:	First submission
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	29-06-2016
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)

Approved WMO

Date:	06-07-2016
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	24-08-2016
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	06-09-2016
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	03-11-2016
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	07-03-2017
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	15-03-2017
Application type:	Amendment
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)
Approved WMO Date:	08-06-2017
Application type:	Amendment
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	EUCTR2016-000974-39-NL
CCMO	NL57571.056.16