An open-label study to evaluate the long-term safety and tolerability of AFQ056 in adolescent patients with Fragile X Syndrome

Published: 08-05-2013
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PrimaryTo evaluate the long-term safety and tolerability of AFQ056 in adolescent patients with FXS as assessed by:* Incidence and severity of adverse events and serious adverse events* Changes in vital signs, laboratory assessments, and ECGs*...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Chromosomal abnormalities, gene alterations and gene variants
Study type	Interventional

Summary

ID

NL-OMON38786

Source
ToetsingOnline

Brief title
Long term safety and efficacy of AFQ056 in Fragile X Syndrome

Condition

- Chromosomal abnormalities, gene alterations and gene variants
- Mental impairment disorders

Synonym
Fragile X Syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Novartis
Source(s) of monetary or material Support: Novartis Pharma B.V.

Intervention

Keyword: Fragile X Syndrome, Intervention study, mGluR5 antagonist

Outcome measures

Primary outcome

* Incidence and severity of adverse events and serious adverse events

* Changes in vital signs, laboratory assessments, and ECGs

* Monitoring the hypothalamic-pituitary-adrenal/thyroid axis function and

childhood developmental milestones

Secondary outcome

* Change from baseline in the Aberrant Behavior Checklist * Community edition

(ABC-C) total score and subscale scores (using the FXS specific algorithm *

ABC-CFX)

Study description

Background summary

The purpose of this study is to generate long-term safety, tolerability and efficacy data for AFQ056 in eligible adolescent patients with FXS who have participated in the CAFQ056B2214 study, the PK study CAFQ056B2131, or another study of AFQ056 which included FXS patients below 18 years of age provided the patient is at least 12 years of age at the time of entry into the current study. Data from this study will be used to support long-term safety data in labeling should the drug receive market authorization approval by health authorities.

Study objective

Primary
To evaluate the long-term safety and tolerability of AFQ056 in adolescent patients with FXS as assessed by:
* Incidence and severity of adverse events and serious adverse events
* Changes in vital signs, laboratory assessments, and ECGs
* Monitoring the hypothalamic-pituitary-adrenal/thyroid axis function and childhood developmental milestones

Secondary
To evaluate the long-term efficacy of AFQ056 treatment in both FM (Fully Methylated) and PM (Partially Methylated) patients with FXS as assessed by:
* Change from baseline in the Aberrant Behavior Checklist * Community edition (ABC-C) total score and subscale scores (using the FXS specific algorithm - ABC-CFX)

Study design

This is a multi-center, open-label, flexible-dose study to evaluate the long-term safety and tolerability of AFQ056 in adolescent patients with Fragile X Syndrome.
Patients who meet all inclusion and none of the exclusion criteria at the respective baseline visit will enter the open-label study and will be titrated from a starting dose of 25 mg b.i.d. at one week intervals up to a maximum of 100 mg b.i.d. or the highest tolerated dose if the maximum dose of 100 mg b.i.d. cannot be reached. Dose adjustments (up- and down-titrations) will be allowed as needed to handle any tolerability issues during the study and to ensure that patients reach their best welltolerated dose.
Initial visits (during titration) occur every two weeks with telephone contacts between scheduled visits. Thereafter, the visit frequency will decrease to 1 month and then 3 month intervals until 24 months of treatment have been reached (or until the study drug becomes available on the market (whichever occurs later)).
One week after the conclusion of the open-label treatment period, patients will undergo a safety follow-up visit.

Intervention

100mg AFQ056 bid (or 25mg, 50mg or 75mg bid if 100mg bid cannot be tolerated)

Study burden and risks

Risks are possible side effects of study medicine or another medicine, and those of taking the blood pressure or taking
blood.
The tests done during the study are standard medical tests. The most unpleasant is often having blood samples taken.

The risks of taking blood may include fainting, pain and/or bruising.
Rarely, these may be a small blood clot or infection at the site of the needle puncture.
Information from this study may help the patient and/or other people with Fragile X Syndrome.

Contacts

Public
Novartis

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Scientific
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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)

Inclusion criteria

Group 1 patients:
* Must have completed the CAFQ056B2214 or another study of AFQ056 which included FXS patients below 18 years of age within one week of enrollment into the open-label study;Group 2 patients:
* Must meet one of the following conditions:

o completed Study CAFQ056B2214 or another study of AFQ056 which included FXS patients below 18 years of age but enrollment into the current study was delayed for more than a week
o discontinued prematurely from Study CAFQ056B2214 or another study of AFQ056 which included FXS patients below 18 years of age due to intolerability of the dosage in the patient*s assigned treatment group

Exclusion criteria

* Discontinuation from CAFQ056B2214 or another study of AFQ056 which included FXS patients below 18 years of age due to safety reasons
* History of severe self-injurious behavior
* History of clinically significant allergies requiring hospitalization or non-inhaled corticosteroid therapy (asthma, anaphylaxis, etc.)
* History of malignancy of any organ system (other than localized basal cell carcinoma of the skin), treated or untreated, within the past 5 years, regardless of whether or not there is evidence of local recurrence or metastases

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	10-12-2013
Enrollment:	1
Type:	Actual

Medical products/devices used

Product type:	Medicine

Brand name:	Nog niet bekend
Generic name:	Mavoglurant

Ethics review

Approved WMO Date:	08-05-2013
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	08-07-2013
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	05-12-2013
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	24-03-2014
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	01-05-2014
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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	EUCTR2011-002379-40-NL
ClinicalTrials.gov	NCT01433354
CCMO	NL44082.078.13