



Clinical trial results:

An Open-Label, Single Arm Study to Provide Access to Fingolimod to MS Patients Who Completed Fingolimod Phase IIIb Studies and Who Benefited from Treatment with Fingolimod or do not Have Suitable Alternative Treatment Options, but do not Have Access to the Reimbursed Drug

Summary

EudraCT number	2012-005507-40
Trial protocol	IT
Global end of trial date	13 February 2014

Results information

Result version number	v1 (current)
This version publication date	05 November 2020
First version publication date	05 November 2020

Trial information

Trial identification

Sponsor protocol code	CFTY720DIT07
-----------------------	--------------

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	-
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Novartis Pharma AG
Sponsor organisation address	CH-4002, Basel, Switzerland,
Public contact	Novartis Pharma AG, Clinical Disclosure Office, +41 39 02 96541,
Scientific contact	Novartis Pharma AG, Clinical Disclosure Office, +41 39 02 96541,

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	13 February 2014
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	13 February 2014
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

To provide early access to fingolimod to subjects who completed local or global fingolimod phase IIIb studies in MS who benefited from treatment with fingolimod or do not have suitable alternative treatment options but do not have access to the reimbursed drug; and to generate long-term safety and tolerability data in a population different from that of the EMA approved label and routine medical care.

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and the International Conference on Harmonization (ICH) Good Clinical Practice (GCP) guidelines. All the local regulatory requirements pertinent to safety of trial subjects were also followed during the conduct of the trial.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	26 July 2013
Long term follow-up planned	Yes
Long term follow-up rationale	Safety
Long term follow-up duration	1 Years
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Italy: 25
Worldwide total number of subjects	25
EEA total number of subjects	25

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	0
Adolescents (12-17 years)	0

Adults (18-64 years)	25
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study was conducted in Italy.

Pre-assignment

Screening details:

A total of 25 subjects were enrolled in the study. The first subject was screened on 26-July-2013. The last study visit occurred on 13-February-2014.

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	All subjects
------------------	--------------

Arm description:

Subjects received 0.5 milligrams (mg) fingolimod capsules, orally, once daily up to study completion or early termination (Up to approximately 6.5 months).

Arm type	Experimental
Investigational medicinal product name	Fingolimod
Investigational medicinal product code	FTY720
Other name	Gilenya®, fingolimod hydrochloride
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

0.5 mg Fingolimod, capsules, orally, once daily

Number of subjects in period 1	All subjects
Started	25
Completed	25

Baseline characteristics

Reporting groups

Reporting group title	Overall Study
-----------------------	---------------

Reporting group description: -

Reporting group values	Overall Study	Total	
Number of subjects	25	25	
Age categorical			
Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	25	25	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical			
Units: Subjects			
Female	11	11	
Male	14	14	

End points

End points reporting groups

Reporting group title	All subjects
Reporting group description: Subjects received 0.5 milligrams (mg) fingolimod capsules, orally, once daily up to study completion or early termination (Up to approximately 6.5 months).	

Primary: Number of Subjects Experiencing any Serious Adverse Events (SAEs)

End point title	Number of Subjects Experiencing any Serious Adverse Events (SAEs) ^[1]
-----------------	--

End point description:

Safety population included all subjects who received at least one dose of fingolimod and had at least one post-baseline safety assessment.

End point type	Primary
----------------	---------

End point timeframe:

From study start visit up to study completion or early termination (Up to approximately 6.5 months).

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: No statistical analysis was planned or performed.

End point values	All subjects			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: Subjects				
SAEs	0			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From study start visit up to early termination (Up to approximately 7 months)

Adverse event reporting additional description:

Safety population included all subjects who received at least one dose of fingolimod and had at least one post-baseline safety assessment.

Assessment type	Systematic
-----------------	------------

Dictionary used

Dictionary name	MedDRA
-----------------	--------

Dictionary version	15.1
--------------------	------

Reporting groups

Reporting group title	All Subjects
-----------------------	--------------

Reporting group description:

Subjects received 0.5 mg fingolimod capsules, orally, once daily, up to study completion or early termination (Up to approximately 6.5 months).

Serious adverse events	All Subjects		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 25 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	All Subjects		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 25 (12.00%)		
Investigations			
WBC decrease			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Lymphopenia			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences (all)	1		
General disorders and administration site conditions			

Influenza like illness subjects affected / exposed occurrences (all)	1 / 25 (4.00%) 1		
Infections and infestations Nasopharyngitis subjects affected / exposed occurrences (all)	1 / 25 (4.00%) 1		
Oral herpes subjects affected / exposed occurrences (all)	1 / 25 (4.00%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date
13 February 2014	Due to the low number of enrolled subjects (25 vs. 200 planned), the program was terminated prematurely and therapeutic continuity was guaranteed through a compassionate use as per Ministerial Decree May 8, 2003, on an individual basis.	-

Notes:

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Due to the low number of enrolment, program was terminated prematurely.

Notes: