Clinical trial results:
An extension study of CACZ885N2301 (NCT02059291), multi-center, open label study of canakinumab in Japanese patients with Periodic Fever Syndromes (Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS), Hyper Immunoglobulin D Syndrome ((also known as mevalonate kinase deficiency) (HIDS/MKD), or colchicine resistant/intolerant Familial Mediterranean Fever (crFMF))

Due to EudraCT system limitations, which EMA is aware of, data using 999 as data points in this record are not an accurate representation of the clinical trial results. Please use https://www.novctrd.com/CtrdWeb/home.novfor complete trial results.

Summary

EudraCT number	2017-001678-40
Trial protocol	Outside EU/EEA
Global end of trial date	27 January 2017

Results information

Result version number	v1 (current)
This version publication date	11 July 2018
First version publication date	11 July 2018

Trial information

Trial identification

Sponsor protocol code	CACZ885N2301E2

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02911857
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Novartis Pharma AG
Sponsor organisation address	CH-4002, Basel, Switzerland,
Public contact	Clinical Disclosure Office, Novartis Pharma AG, 41 613241111,
Scientific contact	Clinical Disclosure Office, Novartis Pharma AG, 41 613241111,

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?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	27 January 2017
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	27 January 2017
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study was to evaluate safety and tolerability of canakinumab in this extension study.

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	03 October 2016
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Japan: 4
Worldwide total number of subjects	4
EEA total number of subjects	0

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)	1

Adults (18-64 years)	3
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Participants with Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS), Hyper Immunoglobulin D Syndrome (also known as mevalonate kinase deficiency (HIDS/MKD), or colchicine resistant/intolerant Familial Mediterranean Fever (crFMF) received study drug based on the final dose regimen received in CACZ885N2301 (NCT02059291).

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	TRAPS

Arm description:

Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Arm type	Experimental
Investigational medicinal product name	Canakinumab
Investigational medicinal product code	CACZ885
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Arm title	HIDS/MKD

Arm description:

Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Arm type	Experimental
Investigational medicinal product name	Canakinumab
Investigational medicinal product code	CACZ885
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Participant continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Arm title	cfFMF

Arm description:

Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Arm type	Experimental
Investigational medicinal product name	Canakinumab
Investigational medicinal product code	CACZ885
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Participant continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.

Number of subjects in period 1	TRAPS	HIDS/MKD	cfFMF
Started	2	1	1
Completed	2	1	1

Baseline characteristics

Reporting groups	
Reporting group title	TRAPS
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	
Reporting group title	HIDS/MKD
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	
Reporting group title	cfFMF
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	

Reporting group values	TRAPS	HIDS/MKD	cfFMF
Number of subjects	2	1	1
Age categorical Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	0	0	0
Adolescents (12-17 years)	0	1	0
Adults (18-64 years)	2	0	1
From 65-84 years	0	0	0
85 years and over	0	0	0
Age Continuous Units: Years			
arithmetic mean	20	14	20
standard deviation	± 2.83	± 9999	± 9999
Gender, Male/Female Units: Subjects			
Female	0	1	0
Male	2	0	1

Reporting group values	Total		
Number of subjects	4		
Age categorical Units: Subjects			
In utero	0		
Preterm newborn infants (gestational age < 37 wks)	0		
Newborns (0-27 days)	0		

Infants and toddlers (28 days-23 months)	0		
Children (2-11 years)	0		
Adolescents (12-17 years)	1		
Adults (18-64 years)	3		
From 65-84 years	0		
85 years and over	0		
Age Continuous			
Units: Years			
arithmetic mean			
standard deviation	-		
Gender, Male/Female			
Units: Subjects			
Female	1		
Male	3		

End points

End points reporting groups

Reporting group title	TRAPS
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	
Reporting group title	HIDS/MKD
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	
Reporting group title	cfFMF
Reporting group description:	
Participants continued the study drug based on the final dose and regimen administered at the end of the CACZ885N2301 study. All participants received 1 or 2 ACZ885 subcutaneous injections every 4 or 8 weeks.	

Primary: Number of participants with non-serious adverse events, serious adverse events and deaths

End point title	Number of participants with non-serious adverse events, serious adverse events and deaths[1]
End point description:	
Participants were monitored for safety throughout the study.	
End point type	Primary
End point timeframe:	
Participants were followed for the duration until approval, an expected average of 3 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: Statistical analysis does not apply to this end point.

End point values	TRAPS	HIDS/MKD	cfFMF	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	2	1	1	
Units: Participants				
Non-serious adverse events	1	1	0	
Serious adverse events	0	0	0	
Deaths	0	0	0	

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Adverse events are collected from First Patient First Visit (FPFV) until Last Patient Last Visit (LPLV). All adverse events reported in this record are from date of First Patient First Treatment until Last Patient Last Visit.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	19.1

Reporting groups

Reporting group title	HIDS
Reporting group description:	
HIDS	
Reporting group title	TRAPS
Reporting group description:	
TRAPS	

Serious adverse events	HIDS	TRAPS	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 1 (0.00%)	0 / 2 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

Frequency threshold for reporting non-serious adverse events: 1 %

Non-serious adverse events	HIDS	TRAPS	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 1 (100.00%)	1 / 2 (50.00%)	
Infections and infestations			
NASOPHARYNGITIS			
subjects affected / exposed	1 / 1 (100.00%)	1 / 2 (50.00%)	
occurrences (all)	1	2	

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? No

Limitations and caveats

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

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