Clinical trial results:
Anakinra (Kineret®) for a hereditary autoinflammatory disease with MEFV mutation and inflammasome activation.

Summary

EudraCT number	2015-004292-69
Trial protocol	BE
Global end of trial date	30 August 2022

Results information

Result version number	v1 (current)
This version publication date	28 July 2023
First version publication date	28 July 2023
Summary attachment (see zip file)	Anakinra in PAAND (Van Nieuwenhove et al.pdf)

Trial information

Trial identification

Sponsor protocol code	MEFV01

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	UZ Leuven
Sponsor organisation address	Herestraat 49, Leuven, Belgium, 3000
Public contact	Wouters Carine, UZ Leuven, 32 1634 39 74, carine.wouters@uzleuven.be
Scientific contact	Wouters Carine, UZ Leuven, 32 1634 39 74, carine.wouters@uzleuven.be

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	30 August 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	30 August 2022
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Primary:
1. Control of inflammatory symptoms (cutaneous, articular, muscular) and of systemic inflammation (anemia, acute phase reactants).
2. Proof of concept that the novel MEFV mutation identified in this family, causes inflammasome and caspase-1activation with increased release of IL-1.

Protection of trial subjects:

Alll patients fulfilled inclusion criteria, gave informed consent for enrollement and treatment with Anakinra.
The trial protocol lwas approved by the ethical comittee of UZ Leuven.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 December 2015
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	Belgium: 3
Worldwide total number of subjects	3
EEA total number of subjects	3

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)	2
From 65 to 84 years	1

85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Mutation in MEFV gene confirmed

Period 1

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

Arms

Arm title	Anakinra treatment

Arm description: -

Arm type	active treatment
Investigational medicinal product name	Anakinra
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection in pre-filled syringe
Routes of administration	Subcutaneous use

Dosage and administration details:

100 mg daily via sc injection

Number of subjects in period 1	Anakinra treatment
Started	3
Completed	3

Baseline characteristics

Reporting groups	
Reporting group title	whole study

Reporting group description: -

Reporting group values	whole study	Total	
Number of subjects	3	3	
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)	2	2	
From 65-84 years	1	1	
85 years and over	0	0	
Age continuous Units: years			
arithmetic mean	61.3		
standard deviation	± 12.7	-	
Gender categorical Units: Subjects			
Female	0	0	
Male	3	3	
disease duration Units: years			
arithmetic mean	34.0		
standard deviation	± 20.8	-	

End points

End points reporting groups

Reporting group title	Anakinra treatment

Reporting group description: -

Primary: remission of systemic inflammatory markers (CRP)

End point title	remission of systemic inflammatory markers (CRP)[1]

End point description:

End point type	Primary

End point timeframe:

at week 12

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: this is a pilot open label study to evaluate the efficacy and safety of anakinra in PAAND patients. Only evaluation of 3 subjects to report, only descriptive statistics possible.

End point values	Anakinra treatment			
Subject group type	Reporting group			
Number of subjects analysed	3			
Units: ≤ 5.0 mg/L				
yes	2			
no	1			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 01-12-2015 until 30-08-2022.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	2.1

Reporting groups	
Reporting group title	whole study

Reporting group description: -

Serious adverse events	whole study		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 3 (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	whole study		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	2 / 3 (66.67%)		
Skin and subcutaneous tissue disorders			
local site reaction			
subjects affected / exposed	2 / 3 (66.67%)		
occurrences (all)	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

None reported