Clinical trial results:
Effects of the activation of peroxisome proliferator-activated receptors in patients with primary biliary cirrhosis

Summary

EudraCT number	2011-004681-15
Trial protocol	AT
Global end of trial date	14 October 2013

Results information

Result version number	v1 (current)
This version publication date	23 June 2021
First version publication date	23 June 2021

Trial information

Trial identification

Sponsor protocol code	KIMCL_TS_2011-09

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Medical University of Graz
Sponsor organisation address	Auenbruggerplatz 15, Graz, Austria, 8036
Public contact	MUG, Medical University of Graz, +43 316385 80442, tatjana.stojakovic@medunigraz.at
Scientific contact	MUG, Medical University of Graz, +43 316385 80442, tatjana.stojakovic@medunigraz.at

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	27 January 2014
Is this the analysis of the primary completion data?	Yes
Primary completion date	14 October 2013
Global end of trial reached?	Yes
Global end of trial date	14 October 2013
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Our major hypothesis is that the treatment with bezafibrate will improve levels of AP in early-stage PBC patients with an incomplete biochemical response to UCDA through a combination of metabolic and anti-inflammatory effects.

Protection of trial subjects:

close follow-up at 3 study visits (baseline, week 4, week 8)

Background therapy:

ursodeoxycholic acid (UDCA)

Evidence for comparator: -

Actual start date of recruitment	09 January 2012
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	Austria: 13
Worldwide total number of subjects	13
EEA total number of subjects	13

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)	12
From 65 to 84 years	1
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

January 2012 - June 2013

Pre-assignment

Screening details:

Patients with PBC and incomplete response to UDCA

Period 1

Period 1 title	baseline
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	bezafibrate

Arm description: -

Arm type	Experimental
Investigational medicinal product name	bezafibrate
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

400 mg once daily

Number of subjects in period 1	bezafibrate
Started	13
Completed	13

Period 2

Period 2 title	week 8
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	bezafibrate
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	bezafibrate
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

400 mg once daily

Number of subjects in period 2	bezafibrate
Started	13
Completed	12
Not completed	1
Adverse event, non-fatal	1

Baseline characteristics

Reporting groups	
Reporting group title	baseline

Reporting group description: -

Reporting group values	baseline	Total	
Number of subjects	13	13	
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)		0	
Adults (18-64 years)		0	
From 65-84 years		0	
85 years and over		0	
Age continuous Units: years			
arithmetic mean	54		
standard deviation	± 8	-	
Gender categorical Units: Subjects			
Female	12	12	
Male	1	1	

End points

End points reporting groups	
Reporting group title	bezafibrate
Reporting group description: -	
Reporting group title	bezafibrate
Reporting group description: -	

Primary: alkaline phosphatase (AP)	
End point title	alkaline phosphatase (AP)
End point description:	
End point type	Primary
End point timeframe: baseline	

End point values	bezafibrate	bezafibrate		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	13	12		
Units: U/l				
arithmetic mean (standard deviation)	217 (± 75)	105 (± 26)		

Attachments (see zip file)	Tables/Tables_PBC_beza.docx

Statistical analyses

Statistical analysis title	paired t-test
Statistical analysis description: week 8 vs. baseline	
Comparison groups	bezafibrate v bezafibrate
Number of subjects included in analysis	25
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.001
Method	t-test, 2-sided

Adverse events

Adverse events information	
Timeframe for reporting adverse events: January 2012 - October 2013	
Assessment type	Non-systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	24.0
Reporting groups	
Reporting group title	bezafibrate
Reporting group description: oral administration of bezafibrate for 8 weeks	

Serious adverse events	bezafibrate		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 13 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	bezafibrate		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 13 (7.69%)		
Musculoskeletal and connective tissue disorders			
Myalgia			
subjects affected / exposed	1 / 13 (7.69%)		
occurrences (all)	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? No

Limitations and caveats

None reported