Clinical trial results:
A Phase IIa study to evaluate the safety and efficacy of ABX464 50 mg once daily versus Placebo in subjects with Moderate to Severe Active Ulcerative Colitis who have failed or are intolerant to immunomodulators, Anti-TNF, vedolizumab and/or corticosteroids.

Summary

EudraCT number	2017-000937-30
Trial protocol	BE HU FR DE PL ES AT
Global end of trial date	04 February 2019

Results information

Result version number	v1 (current)
This version publication date	28 October 2021
First version publication date	28 October 2021

Trial information

Trial identification

Sponsor protocol code	ABX464-101

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03093259
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	ABIVAX
Sponsor organisation address	5 rue de la Baume, Paris, France, 75008
Public contact	Head of Clinical Operations , Abivax , +33 153830961, paul.gineste@abivax.com
Scientific contact	Chief Medical Officer, Abivax , +33 153830961, sophie.biguenet@abivax.com

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	04 February 2019
Is this the analysis of the primary completion data?	Yes
Primary completion date	25 July 2018
Global end of trial reached?	Yes
Global end of trial date	04 February 2019
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the study is to evaluate safety of ABX464 given at 50 mg once daily versus Placebo in subjects with Moderate to Severe Active Ulcerative Colitis who have failed or are intolerant to immunomodulators, Anti-TNFα, vedolizumab and/or corticosteroids.

Protection of trial subjects:

All study participants were required to read and sign an Informed Consent Form (ICF).

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	16 November 2017
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Poland: 11
Country: Number of subjects enrolled	Austria: 1
Country: Number of subjects enrolled	France: 6
Country: Number of subjects enrolled	Germany: 1
Country: Number of subjects enrolled	Hungary: 10
Country: Number of subjects enrolled	Belgium: 3
Worldwide total number of subjects	32
EEA total number of subjects	32

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)	28
From 65 to 84 years	4
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

a total of 32 patients were enrolled
a total of 30 patients completed the study
a total of 17 patients achieved clinical response at week 8

Period 1

Period 1 title	Overall trial (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	ABX464 50mg

Arm description:

ABX464 50mg

Arm type	Experimental
Investigational medicinal product name	ABX464
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule, hard
Routes of administration	Oral use

Dosage and administration details:

Upon confirmation of eligibility, each subject was randomized using the electronic CRF (eCRF) in a 2:1 ratio to once daily oral 50 mg ABX464 added to background therapy for 8 weeks.

Arm title	Placebo

Arm description:

Placebo

Arm type	Placebo
Investigational medicinal product name	placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule, hard
Routes of administration	Oral use

Dosage and administration details:

Upon confirmation of eligibility, each subject was randomized using the electronic CRF (eCRF) in a 2:1 ratio to once daily oral 50 mg placebo added to background therapy for 8 weeks.

Number of subjects in period 1	ABX464 50mg	Placebo
Started	23	9
Completed	21	9
Not completed	2	0
Consent withdrawn by subject	1	-
Adverse event, non-fatal	1	-

Baseline characteristics

Reporting groups	
Reporting group title	Overall trial

Reporting group description: -

Reporting group values	Overall trial	Total	
Number of subjects	32	32	
Age categorical Units: Subjects			
Adults (18-64 years)	28	28	
From 65-84 years	4	4	
Gender categorical Units: Subjects			
Female	12	12	
Male	20	20	

End points

End points reporting groups

Reporting group title	ABX464 50mg
Reporting group description: ABX464 50mg	
Reporting group title	Placebo
Reporting group description: Placebo	

Primary: The number of incidences of treatment-emergent adverse events (TEAEs) in the ABX464-treated subjects compared to placebo

End point title	The number of incidences of treatment-emergent adverse events (TEAEs) in the ABX464-treated subjects compared to placebo
End point description: The primary endpoint of this study is defined as the number of incidences of treatment-emergent adverse events in the ABX464 treated subjects compared to placebo.	
End point type	Primary
End point timeframe: week 8	

End point values	ABX464 50mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	23	9		
Units: 32	18	5		

Statistical analyses

Statistical analysis title	primary endpoint statistical analysis
Comparison groups	ABX464 50mg v Placebo
Number of subjects included in analysis	32
Analysis specification	Pre-specified
Analysis type	other[1]
P-value	= 0.2096
Method	Chi-squared
Confidence interval	
level	90 %
sides	1-sided

Notes:

[1] - safety study

Secondary: The proportion of subjects receiving ABX464 with clinical remission

according to the Total Mayo Score at Week 8 compared to placebo (primary efficacy endpoint)

End point title	The proportion of subjects receiving ABX464 with clinical remission according to the Total Mayo Score at Week 8 compared to placebo (primary efficacy endpoint)

End point description:

End point type	Secondary

End point timeframe:

8 weeks

End point values	ABX464 50mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	20	9		
Units: 32	7	1		

Statistical analyses

Statistical analysis title	statistical analysis secondary endpoint
Comparison groups	ABX464 50mg v Placebo
Number of subjects included in analysis	29
Analysis specification	Post-hoc
Analysis type	other
P-value	= 0.1588
Method	Chi-squared
Confidence interval	
level	90 %
sides	1-sided

Secondary: The change from baseline in fecal calprotectin levels at Week 4 and Week 8 compared to placebo

End point title	The change from baseline in fecal calprotectin levels at Week 4 and Week 8 compared to placebo

End point description:

End point type	Secondary

End point timeframe:

week 8

End point values	ABX464 50mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	20	9		
Units: 32	15	8		

Statistical analyses

Statistical analysis title	statistical analysis secondary endpoint
Comparison groups	ABX464 50mg v Placebo
Number of subjects included in analysis	29
Analysis specification	Post-hoc
Analysis type	other
P-value	= 0.483
Method	ANCOVA

Secondary: The change from screening in Total Mayo Score in subjects receiving ABX464 at Week 8 compared to placebo

End point title	The change from screening in Total Mayo Score in subjects receiving ABX464 at Week 8 compared to placebo

End point description:

End point type	Secondary

End point timeframe:

week 8

End point values	ABX464 50mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	20	9		
Units: Change in Total Mayo Score				
arithmetic mean (standard deviation)	-4.6 (± 2.8)	-2.1 (± 2.5)		

Statistical analyses

Statistical analysis title	statistical analysis secondary endpoint
Comparison groups	ABX464 50mg v Placebo

Number of subjects included in analysis	29
Analysis specification	Post-hoc
Analysis type	other
P-value	= 0.0742
Method	ANCOVA

Secondary: The change from baseline in Partial Mayo Score in subjects receiving ABX464 at Week 4 and Week 8 compared to placebo

End point title	The change from baseline in Partial Mayo Score in subjects receiving ABX464 at Week 4 and Week 8 compared to placebo

End point description:

End point type	Secondary

End point timeframe:

week 8

End point values	ABX464 50mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	20	9		
Units: Change in Partial Mayo Score				
arithmetic mean (standard deviation)	-3.9 (± 2.2)	-1.8 (± 2.0)		

Statistical analyses

Statistical analysis title	statistical analysis secondary endpoint
Comparison groups	ABX464 50mg v Placebo
Number of subjects included in analysis	29
Analysis specification	Post-hoc
Analysis type	other
P-value	= 0.0462
Method	ANCOVA

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 16 November 2017- 30 July 2018	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	20.0
Reporting groups	
Reporting group title	ABX464 50mg
Reporting group description: -	
Reporting group title	Placebo
Reporting group description: -	

Serious adverse events	ABX464 50mg	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 23 (4.35%)	1 / 9 (11.11%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Renal and urinary disorders			
Nephrolithiasis			
subjects affected / exposed	0 / 23 (0.00%)	1 / 9 (11.11%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

Frequency threshold for reporting non-serious adverse events: 4 %

Non-serious adverse events	ABX464 50mg	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	18 / 23 (78.26%)	5 / 9 (55.56%)	
Investigations			
AST/ALT ratio			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Injury, poisoning and procedural complications			
Ligament sprain			

subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Vascular disorders			
Haematoma			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Peripheral vascular disorder			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Nervous system disorders			
Headache			
subjects affected / exposed	4 / 23 (17.39%)	0 / 9 (0.00%)	
occurrences (all)	5	0	
Poor quality sleep			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
General disorders and administration site conditions			
Chest pain			
subjects affected / exposed	2 / 23 (8.70%)	0 / 9 (0.00%)	
occurrences (all)	2	0	
Influenza like illness			
subjects affected / exposed	2 / 23 (8.70%)	0 / 9 (0.00%)	
occurrences (all)	2	0	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	4 / 23 (17.39%)	1 / 9 (11.11%)	
occurrences (all)	4	1	
Abdominal pain upper			
subjects affected / exposed	3 / 23 (13.04%)	0 / 9 (0.00%)	
occurrences (all)	3	0	
Anal fissure			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Anorectal discomfort			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	

Diarrhoea			
subjects affected / exposed	0 / 23 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Dyspepsia			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Nauseae			
subjects affected / exposed	2 / 23 (8.70%)	0 / 9 (0.00%)	
occurrences (all)	2	0	
Respiratory, thoracic and mediastinal disorders			
Dyspnoea			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Oropharyngeal pain			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Skin discolouration			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Renal and urinary disorders			
Nephrolithiasis			
subjects affected / exposed	0 / 23 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Renal colic			
subjects affected / exposed	0 / 23 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Infections and infestations			

Influenza			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Nasopharyngitistis			
subjects affected / exposed	1 / 23 (4.35%)	1 / 9 (11.11%)	
occurrences (all)	1	1	
Oral herpes			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Rhinitis			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Sinusitis			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Upper respiratory tract infection			
subjects affected / exposed	1 / 23 (4.35%)	0 / 9 (0.00%)	
occurrences (all)	1	0	
Metabolism and nutrition disorders			
Hypophosphataemia			
subjects affected / exposed	1 / 23 (4.35%)	2 / 9 (22.22%)	
occurrences (all)	2	2	
Iron deficiency			
subjects affected / exposed	1 / 23 (4.35%)	1 / 9 (11.11%)	
occurrences (all)	1	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
13 March 2018	Administrative changes and clarifications

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported