



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

| | | | |
|------------------------------------|----------------|----------------|--|
| Influenza | | | |
| subjects affected / exposed | 1 / 23 (4.35%) | 0 / 9 (0.00%) | |
| occurrences (all) | 1 | 0 | |
| Nasopharyngitis | | | |
| 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