Clinical trial results:
A multicenter, open-label extension study to evaluate the long-term safety, tolerability and efficacy of orally administered GLPG1690 in subjects with systemic sclerosis

Summary

EudraCT number	2019-001279-34
Trial protocol	GB ES DE BE IT
Global end of trial date	13 April 2021

Results information

Result version number	v1
This version publication date	08 March 2022
First version publication date	08 March 2022

Trial information

Trial identification

Sponsor protocol code	GLPG1690-CL-206

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03976648
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Galapagos NV
Sponsor organisation address	Generaal De Wittelaan L11 A3 , Mechelen, Belgium, 2800
Public contact	Medical Information, Galapagos NV, medicalinfo@glpg.com
Scientific contact	Medical Information, Galapagos NV, medicalinfo@glpg.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	13 April 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	13 April 2021
Global end of trial reached?	Yes
Global end of trial date	13 April 2021
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

To evaluate the long-term safety and tolerability of GLPG1690 in subjects with systemic sclerosis.

Protection of trial subjects:

The study was performed in accordance with the ethical principles that have their origin in the "Declaration of Helsinki" and its amendments in force at the time of the study (2013 version). It was also carried out in conformity with the protocol, the International Council for Harmonisation Guideline for Good Clinical Practice (ICH-GCP) E6 (R2), and local ethical and legal requirements. The investigator informed the subjects of the risks and benefits of the study. The subjects were informed that they could withdraw from the study at any time for any reason. Consent was obtained in writing prior to any study-related activities; the investigator retained a copy of the ICFs, which are available to the sponsor for inspection. The subjects were covered by the sponsor's insurance according to local legal requirements.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	18 July 2019
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	Spain: 3
Country: Number of subjects enrolled	United Kingdom: 6
Country: Number of subjects enrolled	Belgium: 12
Country: Number of subjects enrolled	Italy: 2
Country: Number of subjects enrolled	United States: 8
Worldwide total number of subjects	31
EEA total number of subjects	17

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)	26
From 65 to 84 years	5
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants were enrolled at study sites in Belgium, Italy, Spain, the United Kingdom, and the United States. The first participant was screened on 18 Jul 2019. The last study visit occurred on 13 Apr 2021. The treatment duration was planned for 104 weeks but the study was terminated at 91 weeks.

Pre-assignment

Screening details:

A total of 31 participants who completed 24-week double-blind treatment in the GLPG1690-CL-204 (2018-001817-33) study were rolled over and randomized in this study.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	GLPG1690 600 mg

Arm description:

Participants who received GLPG1690 600 milligrams (mg) in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.

Arm type	Experimental
Investigational medicinal product name	GLPG1690
Investigational medicinal product code	
Other name	ziritaxestat
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received GLPG1690 600 mg orally once daily.

Arm title	Placebo

Arm description:

Participants who received placebo matched to GLPG1690 in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.

Arm type	Experimental
Investigational medicinal product name	GLPG1690
Investigational medicinal product code	
Other name	ziritaxestat
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received GLPG1690 600 mg orally once daily.

Number of subjects in period 1	GLPG1690 600 mg	Placebo
Started	21	10
Completed	0	0
Not completed	21	10
Study terminated by sponsor	21	8
Adverse event	-	2

Baseline characteristics

Reporting groups	
Reporting group title	GLPG1690 600 mg
Reporting group description:	
Participants who received GLPG1690 600 milligrams (mg) in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	
Reporting group title	Placebo
Reporting group description:	
Participants who received placebo matched to GLPG1690 in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	

Reporting group values	GLPG1690 600 mg	Placebo	Total
Number of subjects	21	10	31
Age categorical Units: Subjects			
Less than or equal to 45 years	7	4	11
Greater than 45 years	14	6	20
Age continuous Units: years			
arithmetic mean	50.4	49.4	
standard deviation	± 13.58	± 18.57	-
Gender categorical Units: Subjects			
Female	15	6	21
Male	6	4	10
Race Units: Subjects			
White	21	9	30
Asian	0	1	1
Ethnicity Units: Subjects			
Hispanic or Latino	1	0	1
Non-hispanic or Latino	20	10	30

End points

End points reporting groups

Reporting group title	GLPG1690 600 mg
Reporting group description:	
Participants who received GLPG1690 600 milligrams (mg) in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	
Reporting group title	Placebo
Reporting group description:	
Participants who received placebo matched to GLPG1690 in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	

Primary: Number of Participants With Treatment Emergent Adverse Events (TEAEs) and Serious TEAEs

End point title	Number of Participants With Treatment Emergent Adverse Events (TEAEs) and Serious TEAEs[1]
End point description:	
An adverse event (AE) was any untoward medical occurrence in a participant-administered study drug and which did not necessarily have a causal relationship with the study drug. A treatment-emergent adverse event (TEAE) is any AE with an onset date on or after the start of stud drug intake and no later than 30 days after the last dose of study drug, or any worsening of any AE on or after the start of stud drug intake. A serious AE was defined as an AE that resulted in death, was life-threatening, required hospitalization or prolongation of existing hospitalization resulted in persistent or significant disability/incapacity, was a congenital anomaly/birth defect, or was medically significant. The OLE-FAS was defined as all participants who had at least one intake of investigational product.	
End point type	Primary
End point timeframe:	
Day 1 up to Day 91	
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: No statistical analysis was planned for this endpoint.	

End point values	GLPG1690 600 mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	21	10		
Units: participants				
TEAEs	21	10		
Serious TEAEs	6	3		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Day 1 up to Day 91	
Adverse event reporting additional description:	
Participants in the OLE-FAS were analyzed.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	23.0

Reporting groups	
Reporting group title	GLPG1690 600 mg
Reporting group description:	
Participants who received GLPG1690 600 milligrams (mg) in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	
Reporting group title	Placebo
Reporting group description:	
Participants who received placebo matched to GLPG1690 in the GLPG1690-CL-204 (2018-001817-33) study, received GLPG1690 600 mg orally once daily up to 91 weeks.	

Serious adverse events	GLPG1690 600 mg	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	6 / 21 (28.57%)	3 / 10 (30.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Cardiac disorders			
Atrial tachycardia			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Leukopenia			

subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Diverticulum intestinal			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Rectal prolapse			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Reproductive system and breast disorders			
Female genital tract fistula			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Interstitial lung disease			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumothorax			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Skin and subcutaneous tissue disorders			
Skin hypertrophy			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Endocrine disorders			
Thyroid mass			

subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Colonic abscess			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Device related infection			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pharyngitis			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Sepsis			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	0 / 3	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urinary tract infection			
subjects affected / exposed	1 / 21 (4.76%)	0 / 10 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	GLPG1690 600 mg	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	21 / 21 (100.00%)	10 / 10 (100.00%)	
Vascular disorders			
Hot flush			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Raynaud's phenomenon			

subjects affected / exposed	3 / 21 (14.29%)	0 / 10 (0.00%)	
occurrences (all)	5	0	
General disorders and administration site conditions			
Discomfort			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Fatigue			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	3	0	
Nodule			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Peripheral swelling			
subjects affected / exposed	4 / 21 (19.05%)	1 / 10 (10.00%)	
occurrences (all)	5	1	
Pyrexia			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	3	1	
Reproductive system and breast disorders			
Breast fibrosis			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Vaginal discharge			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Vaginal haemorrhage			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	7 / 21 (33.33%)	1 / 10 (10.00%)	
occurrences (all)	9	1	
Oropharyngeal pain			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	

Psychiatric disorders Disorientation subjects affected / exposed occurrences (all)	0 / 21 (0.00%) 0	1 / 10 (10.00%) 1	
Investigations Alanine aminotransferase increased subjects affected / exposed occurrences (all) Weight increased subjects affected / exposed occurrences (all)	2 / 21 (9.52%) 2 2 / 21 (9.52%) 3	0 / 10 (0.00%) 0 0 / 10 (0.00%) 0	
Injury, poisoning and procedural complications Procedural pain subjects affected / exposed occurrences (all)	2 / 21 (9.52%) 2	0 / 10 (0.00%) 0	
Congenital, familial and genetic disorders Hydrocele subjects affected / exposed occurrences (all)	0 / 21 (0.00%) 0	1 / 10 (10.00%) 1	
Cardiac disorders Atrial fibrillation subjects affected / exposed occurrences (all) Bundle branch block left subjects affected / exposed occurrences (all) Bundle branch block right subjects affected / exposed occurrences (all) Palpitations subjects affected / exposed occurrences (all) Pericarditis subjects affected / exposed occurrences (all) Ventricular extrasystoles	0 / 21 (0.00%) 0 1 / 21 (4.76%) 1 0 / 21 (0.00%) 0 4 / 21 (19.05%) 4 0 / 21 (0.00%) 0	1 / 10 (10.00%) 1 1 / 10 (10.00%) 1 1 / 10 (10.00%) 1 0 / 10 (0.00%) 0 1 / 10 (10.00%) 1	

subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	3	0	
Nervous system disorders			
Dizziness			
subjects affected / exposed	3 / 21 (14.29%)	2 / 10 (20.00%)	
occurrences (all)	3	3	
Headache			
subjects affected / exposed	6 / 21 (28.57%)	2 / 10 (20.00%)	
occurrences (all)	6	3	
Blood and lymphatic system disorders			
Leukopenia			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Normocytic anaemia			
subjects affected / exposed	0 / 21 (0.00%)	2 / 10 (20.00%)	
occurrences (all)	0	2	
Eye disorders			
Altered visual depth perception			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Blepharitis			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Gastrointestinal disorders			
Abdominal distension			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Abdominal pain			
subjects affected / exposed	3 / 21 (14.29%)	0 / 10 (0.00%)	
occurrences (all)	3	0	
Anal incontinence			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Aphthous ulcer			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Constipation			

subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Diarrhoea			
subjects affected / exposed	8 / 21 (38.10%)	4 / 10 (40.00%)	
occurrences (all)	13	7	
Dyspepsia			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Dysphagia			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Eructation			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Gastric antral vascular ectasia			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Gastrooesophageal reflux disease			
subjects affected / exposed	2 / 21 (9.52%)	1 / 10 (10.00%)	
occurrences (all)	3	1	
Nausea			
subjects affected / exposed	4 / 21 (19.05%)	1 / 10 (10.00%)	
occurrences (all)	6	1	
Oesophagitis			
subjects affected / exposed	2 / 21 (9.52%)	1 / 10 (10.00%)	
occurrences (all)	2	1	
Skin and subcutaneous tissue disorders			
Dermatitis psoriasiform			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Digital pitting scar			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Hair growth abnormal			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	

Hyperhidrosis			
subjects affected / exposed	2 / 21 (9.52%)	1 / 10 (10.00%)	
occurrences (all)	2	1	
Night sweats			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Perioral dermatitis			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Rash macular			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Scleroderma associated digital ulcer			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Skin lesion			
subjects affected / exposed	5 / 21 (23.81%)	1 / 10 (10.00%)	
occurrences (all)	6	1	
Skin ulcer			
subjects affected / exposed	6 / 21 (28.57%)	1 / 10 (10.00%)	
occurrences (all)	14	4	
Telangiectasia			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Urticaria			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	2	
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	4 / 21 (19.05%)	0 / 10 (0.00%)	
occurrences (all)	7	0	
Back pain			
subjects affected / exposed	3 / 21 (14.29%)	0 / 10 (0.00%)	
occurrences (all)	4	0	
Bursitis			

subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Muscle spasms			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Muscular weakness			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Musculoskeletal chest pain			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Musculoskeletal stiffness			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Myalgia			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	2	
Pain in extremity			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	2	
Rotator cuff syndrome			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Tendonitis			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	4	0	
Infections and infestations			
Bronchitis			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
COVID-19			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Cellulitis			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	

Conjunctivitis			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Infected skin ulcer			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Influenza			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Nasopharyngitis			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Oral herpes			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Otitis media			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Paronychia			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Pharyngitis			
subjects affected / exposed	2 / 21 (9.52%)	0 / 10 (0.00%)	
occurrences (all)	2	0	
Rhinitis			
subjects affected / exposed	2 / 21 (9.52%)	1 / 10 (10.00%)	
occurrences (all)	2	1	
Suspected COVID-19			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Upper respiratory tract infection			
subjects affected / exposed	5 / 21 (23.81%)	1 / 10 (10.00%)	
occurrences (all)	7	1	
Urinary tract infection			
subjects affected / exposed	6 / 21 (28.57%)	0 / 10 (0.00%)	
occurrences (all)	11	0	

Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 21 (4.76%)	1 / 10 (10.00%)	
occurrences (all)	1	1	
Dyslipidaemia			
subjects affected / exposed	2 / 21 (9.52%)	1 / 10 (10.00%)	
occurrences (all)	2	1	
Lactose intolerance			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	
Vitamin D deficiency			
subjects affected / exposed	0 / 21 (0.00%)	1 / 10 (10.00%)	
occurrences (all)	0	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 January 2020	- The protocol was updated to extend the duration of the treatment period from 52 weeks to 104 weeks. - The information on ziritaxestat was updated in line with the latest Investigator's Brochure. - Events meeting the following defined criteria were to be reported as an SAE and IP was to be discontinued: • Aspartate aminotransferase (AST) or alanine aminotransferase (ALT) ≥ 8x upper limit of normal (ULN) • AST or ALT ≥ 3x ULN with signs of severe liver damage (i.e., with the appearance of fatigue, nausea, vomiting, right upper quadrant pain or tenderness, fever, rash and/or eosinophilia [> 5%], and/or total bilirubin ≥ 1.5x ULN or international normalized ratio > 1.5)

Notes:

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.

The benefit-risk profile no longer supports continuing the studies. Therefore, the study was terminated.

Notes: