



Clinical trial results:

A Phase 2b, randomized, double-blind, placebo-controlled, dose-ranging, multi-center study to evaluate the efficacy and safety of GB001 as maintenance therapy in adult subjects with moderate to severe asthma

Summary

EudraCT number	2018-002242-36
Trial protocol	CZ DE BE AT ES GB PL
Global end of trial date	18 August 2020

Results information

Result version number	v1 (current)
This version publication date	04 September 2021
First version publication date	04 September 2021

Trial information

Trial identification

Sponsor protocol code	GB001-2001
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03683576
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	GB001, Inc, a wholly owned subsidiary of Gossamer Bio, Inc.
Sponsor organisation address	3013 Science Park Road, San Diego, United States, 92121
Public contact	GB001, Inc., Study Director, GB001, Inc., wholly owned subsidiary of Gossamer Bio Inc., 866 668-4083, ClinicalTrials@gossamerbio.com
Scientific contact	GB001, Inc., Study Director, GB001, Inc., wholly owned subsidiary of Gossamer Bio Inc., 866 668-4083, ClinicalTrials@gossamerbio.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	18 August 2020
Is this the analysis of the primary completion data?	Yes
Primary completion date	23 July 2020
Global end of trial reached?	Yes
Global end of trial date	18 August 2020
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the effect of GB001 (the investigational product) compared to placebo on reducing asthma worsening.

Protection of trial subjects:

This study was conducted in accordance with consensus ethical principles derived from international guidelines including the Declaration of Helsinki, Council for International Organizations of Medical Sciences (CIOMS) International Ethical Guidelines and International Conference on Harmonization (ICH) Good Clinical Practice (GCP) guidelines. All applicable local laws and regulations regarding patient safety were also followed.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	22 October 2018
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	Austria: 17
Country: Number of subjects enrolled	Belgium: 8
Country: Number of subjects enrolled	Canada: 3
Country: Number of subjects enrolled	Czechia: 54
Country: Number of subjects enrolled	France: 7
Country: Number of subjects enrolled	Germany: 43
Country: Number of subjects enrolled	Poland: 86
Country: Number of subjects enrolled	Spain: 16
Country: Number of subjects enrolled	Ukraine: 141
Country: Number of subjects enrolled	United Kingdom: 4
Country: Number of subjects enrolled	United States: 101
Worldwide total number of subjects	480
EEA total number of subjects	231

Notes:

Subjects enrolled per age group

In utero	0
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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)	408
From 65 to 84 years	72
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants were recruited from Austria, Belgium, Canada, Czechia, France, Germany, Poland, Spain, Ukraine, United Kingdom, and United States.

Pre-assignment

Screening details:

The study included a run-in period, during which eligibility for randomization was determined. 731 participants entered the run-in period, 481 of whom were randomized. One participant randomized to GB001 40 mg was never treated and was therefore excluded from the Intent-to-Treat (ITT) and Safety Analysis Populations.

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, Monitor, Data analyst, Assessor

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo

Arm description:

Placebo QD for 24 weeks

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

Dosage and administration details:

once per day (QD) for 24 weeks

Arm title	GB001 20 mg
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Arm description:

GB001 20 mg QD for 24 weeks

Arm type	Experimental
Investigational medicinal product name	GB001 20 mg
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

GB001 20 mg QD for 24 weeks

Arm title	GB001 40 mg
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Arm description:

GB001 40 mg QD for 24 weeks

Arm type	Experimental
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Investigational medicinal product name	GB001 40 mg
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use
Dosage and administration details: GB001 40 mg QD for 24 weeks	
Arm title	GB001 60 mg
Arm description: GB001 60 mg QD for 24 weeks	
Arm type	Experimental
Investigational medicinal product name	GB001 60 mg
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use
Dosage and administration details: GB001 60 mg QD for 24 weeks	

Number of subjects in period 1	Placebo	GB001 20 mg	GB001 40 mg
Started	120	120	118
Completed	114	116	106
Not completed	6	4	12
Consent withdrawn by subject	2	-	8
Adverse event, non-fatal	3	-	-
Other	-	2	-
Lost to follow-up	-	1	2
Lack of efficacy	1	-	1
Protocol deviation	-	1	1

Number of subjects in period 1	GB001 60 mg
Started	122
Completed	114
Not completed	8
Consent withdrawn by subject	3
Adverse event, non-fatal	4
Other	-
Lost to follow-up	-
Lack of efficacy	1
Protocol deviation	-

Baseline characteristics

Reporting groups

Reporting group title	Placebo
Reporting group description: Placebo QD for 24 weeks	
Reporting group title	GB001 20 mg
Reporting group description: GB001 20 mg QD for 24 weeks	
Reporting group title	GB001 40 mg
Reporting group description: GB001 40 mg QD for 24 weeks	
Reporting group title	GB001 60 mg
Reporting group description: GB001 60 mg QD for 24 weeks	

Reporting group values	Placebo	GB001 20 mg	GB001 40 mg
Number of subjects	120	120	118
Age categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	51.5 ± 11.91	52.8 ± 11.81	52.9 ± 13.32
Gender categorical Units: Subjects			
Female	76	86	74
Male	44	34	44

Reporting group values	GB001 60 mg	Total	
Number of subjects	122	480	
Age categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	49.9 ± 14.37	-	
Gender categorical Units: Subjects			
Female	72	308	
Male	50	172	

End points

End points reporting groups

Reporting group title	Placebo
Reporting group description: Placebo QD for 24 weeks	
Reporting group title	GB001 20 mg
Reporting group description: GB001 20 mg QD for 24 weeks	
Reporting group title	GB001 40 mg
Reporting group description: GB001 40 mg QD for 24 weeks	
Reporting group title	GB001 60 mg
Reporting group description: GB001 60 mg QD for 24 weeks	

Primary: Proportion of Participants Who Experience Worsening of Asthma by Week 24

End point title	Proportion of Participants Who Experience Worsening of Asthma by Week 24
End point description: Proportion of participants who experience worsening of asthma by Week 24 as defined by at least 1 of the following: <ul style="list-style-type: none">On 2 consecutive days, morning (AM) peak expiratory flow (PEF) $\leq 75\%$ of mean AM PEF measured over the last 7 days of the Run-inForced expiratory volume in 1 second (FEV1) $< 80\%$ of baselineIncrease in rescue medication use of ≥ 6 puffs/day on 2 consecutive days compared to mean use over the last 7 days of the Run-inIncrease in Asthma Control Questionnaire 5 (ACQ-5; see Secondary: Change From Baseline to Week 24 in ACQ-5 Score for description) score of ≥ 0.5 compared to baselineThe occurrence of a severe asthma exacerbation (asthma attack) defined as deterioration of asthma that leads to the use of systemic corticosteroids for at least 3 days, hospitalization, or an Emergency Department visit.	
Analysis Population Description ITT Population: all participants who were randomized and received at least 1 dose of study treatment	
End point type	Primary
End point timeframe: up to Week 24	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: proportion of participants				
number (confidence interval 95%)	0.658 (0.570 to 0.737)	0.567 (0.477 to 0.652)	0.568 (0.478 to 0.654)	0.557 (0.469 to 0.642)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1425
Method	Regression, Logistic
Parameter estimate	Odds ratio (OR)
Point estimate	0.674
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.398
upper limit	1.142

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo
Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1482
Method	Regression, Logistic
Parameter estimate	Odds ratio (OR)
Point estimate	0.677
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.399
upper limit	1.149

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1086
Method	Regression, Logistic
Parameter estimate	Odds ratio (OR)
Point estimate	0.651
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.385
upper limit	1.1

Secondary: Change From Baseline to Week 24 in ACQ-5 Score

End point title	Change From Baseline to Week 24 in ACQ-5 Score
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End point description:

The ACQ-5 is a 5-item questionnaire which has been developed as a measure of the participant's asthma control that can be quickly and easily completed. The questions are designed to be self-completed by the participant. The 5 questions enquire about the frequency and/or severity of symptoms in the prior week (nocturnal awakening, activity limitation, shortness of breath, wheeze). The response options for each of these questions consists of a zero (no impairment/limitation) to 6 (total impairment/limitation) scale.

Analysis Population Description

ITT Population: all participants who were randomized and received at least 1 dose of study treatment.

End point type	Secondary
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End point timeframe:

Baseline, Week 24

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: score on a scale				
least squares mean (confidence interval 95%)	-0.89 (-1.05 to -0.73)	-1.04 (-1.20 to -0.89)	-1.04 (-1.20 to -0.88)	-1.08 (-1.24 to -0.92)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1647
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	-0.15
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.36
upper limit	0.06

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo

Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1737
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	-0.15
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.37
upper limit	0.07

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0879
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	-0.19
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.4
upper limit	0.03

Secondary: Change From Baseline to Week 24 in Pre-Bronchodilator FEV1

End point title	Change From Baseline to Week 24 in Pre-Bronchodilator FEV1
End point description: Pre-albuterol/salbutamol morning FEV1 was measured using electronic spirometry.	
Analysis Population Description ITT Population: all participants who were randomized and received at least 1 dose of study treatment.	
End point type	Secondary
End point timeframe: Baseline, Week 24	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: litres (L)				
least squares mean (confidence interval 95%)	0.105 (0.027 to 0.182)	0.121 (0.041 to 0.200)	0.146 (0.064 to 0.227)	0.180 (0.102 to 0.257)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7718
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	0.016
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.091
upper limit	0.123

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo
Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4562
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	0.041
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.067
upper limit	0.149

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo

Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1631
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	0.075
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.03
upper limit	0.18

Secondary: Time to First Asthma Worsening

End point title	Time to First Asthma Worsening
End point description:	
Time to first asthma worsening is defined as the time from the date of the first dose of study treatment to the first date that any of the components of asthma worsening endpoint is met. See Primary: Proportion of Participants Who Experience Worsening of Asthma by Week 24 description for the definition of asthma worsening.	
Analysis Population Description	
ITT Population: all participants who were randomized and received at least 1 dose of study treatment.	
'99999' indicates the value is not estimable due to an insufficient number of observed events.	
End point type	Secondary
End point timeframe:	
up to Week 24	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: weeks				
median (confidence interval 95%)	10.57 (7.857 to 16.286)	17.43 (12.143 to 99999)	17.57 (13.429 to 24.286)	19.86 (14.857 to 99999)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo

Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0466
Method	Regression, Cox
Parameter estimate	Hazard ratio (HR)
Point estimate	0.719
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.519
upper limit	0.995

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo
Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1222
Method	Regression, Cox
Parameter estimate	Hazard ratio (HR)
Point estimate	0.773
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.558
upper limit	1.071

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0304
Method	Regression, Cox
Parameter estimate	Hazard ratio (HR)
Point estimate	0.698
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.505
upper limit	0.967

Secondary: Annualized Rate of Severe Asthma Exacerbations

End point title	Annualized Rate of Severe Asthma Exacerbations
End point description:	
A severe asthma exacerbation is defined as deterioration of asthma that leads to the use of systemic corticosteroids for at least 3 days, hospitalization, or an Emergency Department visit.	
Analysis Population Description	
ITT Population: all participants who were randomized and received at least 1 dose of study treatment.	
End point type	Secondary
End point timeframe:	
up to Week 24	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: events/year				
least squares mean (confidence interval 95%)	0.933 (0.664 to 1.311)	0.744 (0.517 to 1.070)	0.698 (0.480 to 1.015)	0.829 (0.585 to 1.174)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3382
Method	Negative binomial regression model
Parameter estimate	Rate ratio
Point estimate	0.797
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.501
upper limit	1.268

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo
Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2248
Method	Negative binomial regression model
Parameter estimate	Rate ratio
Point estimate	0.748

Confidence interval	
level	95 %
sides	2-sided
lower limit	0.469
upper limit	1.195

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.609
Method	Negative binomial regression model
Parameter estimate	Rate ratio
Point estimate	0.889
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.565
upper limit	1.397

Secondary: Change From Baseline to Week 24 in Post-Bronchodilator FEV1

End point title	Change From Baseline to Week 24 in Post-Bronchodilator FEV1
End point description:	
Post-albuterol/salbutamol morning FEV1 was measured using electronic spirometry.	
Analysis Population Description	
ITT Population: all participants who were randomized and received at least 1 dose of study treatment.	
End point type	Secondary
End point timeframe:	
Baseline, Week 24	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: litres (L)				
least squares mean (confidence interval 95%)	0.012 (-0.064 to 0.088)	-0.011 (-0.088 to 0.066)	0.047 (-0.037 to 0.131)	0.091 (0.015 to 0.166)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6645
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	-0.023
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.127
upper limit	0.081

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo
Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5288
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	0.035
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.074
upper limit	0.144

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1362
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	0.079
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.025
upper limit	0.182

Secondary: Change From Baseline to Week 24 in AM PEF

End point title	Change From Baseline to Week 24 in AM PEF
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End point description:

AM PEF was measured by participants using an electronic diary.

Analysis Population Description

ITT Population: all participants who were randomized and received at least 1 dose of study treatment.

End point type	Secondary
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End point timeframe:

Baseline, Week 24

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: L/minute				
least squares mean (confidence interval 95%)	8.993 (-1.514 to 19.499)	15.115 (4.779 to 25.451)	22.941 (12.042 to 33.839)	14.581 (4.140 to 25.021)

Statistical analyses

Statistical analysis title	Statistical Analysis 1
Comparison groups	GB001 20 mg v Placebo
Number of subjects included in analysis	240
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3957
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	6.122
Confidence interval	
level	95 %
sides	2-sided
lower limit	-8.007
upper limit	20.251

Statistical analysis title	Statistical Analysis 2
Comparison groups	GB001 40 mg v Placebo

Number of subjects included in analysis	238
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0598
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	13.948
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.578
upper limit	28.474

Statistical analysis title	Statistical Analysis 3
Comparison groups	GB001 60 mg v Placebo
Number of subjects included in analysis	242
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4376
Method	ANCOVA
Parameter estimate	Mean difference (net)
Point estimate	5.588
Confidence interval	
level	95 %
sides	2-sided
lower limit	-8.522
upper limit	19.698

Secondary: Incidence of Treatment-Emergent Adverse Events (TEAEs)

End point title	Incidence of Treatment-Emergent Adverse Events (TEAEs)
End point description:	
An adverse event (AE) is any untoward medical occurrence in a participant, whether or not considered related to study treatment. Abnormal laboratory test results or other safety assessments, including those that worsened from baseline, that were considered clinically significant in the medical and scientific judgment of the investigator were to be reported as AEs.	
Analysis Population Description	
Safety Population: all participants who received at least 1 dose of study treatment.	
End point type	Secondary
End point timeframe:	
From first dose of study treatment through Week 28	

End point values	Placebo	GB001 20 mg	GB001 40 mg	GB001 60 mg
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	120	120	118	122
Units: percentage of participants				
number (not applicable)	65.8	65.8	69.5	68.0

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From first dose of study treatment through Week 28

Adverse event reporting additional description:

[Not specified]

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	23.0
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Reporting groups

Reporting group title	Placebo
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Reporting group description: -

Reporting group title	GB001 20 mg
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Reporting group description: -

Reporting group title	GB001 40 mg
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Reporting group description: -

Reporting group title	GB001 60 mg
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Reporting group description: -

Serious adverse events	Placebo	GB001 20 mg	GB001 40 mg
Total subjects affected by serious adverse events			
subjects affected / exposed	9 / 120 (7.50%)	5 / 120 (4.17%)	5 / 118 (4.24%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Chronic myeloid leukaemia			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Small cell lung cancer			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vascular disorders			
Hypertension			

subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Reproductive system and breast disorders			
Metrorrhagia			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Uterine haemorrhage			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Uterine polyp			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Allergic bronchitis			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Asthma			
subjects affected / exposed	2 / 120 (1.67%)	2 / 120 (1.67%)	2 / 118 (1.69%)
occurrences causally related to treatment / all	0 / 3	0 / 2	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic obstructive pulmonary disease			

subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumothorax			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Foot fracture			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Fracture displacement			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Acute myocardial infarction			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Left ventricular failure			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Myocardial infarction			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Myasthenia gravis			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Gastrointestinal disorders			
Gastroesophageal reflux disease			
subjects affected / exposed	0 / 120 (0.00%)	1 / 120 (0.83%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Liver injury			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin and subcutaneous tissue disorders			
Angioedema			
subjects affected / exposed	0 / 120 (0.00%)	1 / 120 (0.83%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Ureterolithiasis			
subjects affected / exposed	0 / 120 (0.00%)	1 / 120 (0.83%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Influenza			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory tract infection			

subjects affected / exposed	1 / 120 (0.83%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sinusitis			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	1 / 118 (0.85%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Mineral metabolism disorder			
subjects affected / exposed	0 / 120 (0.00%)	0 / 120 (0.00%)	0 / 118 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	GB001 60 mg		
Total subjects affected by serious adverse events			
subjects affected / exposed	7 / 122 (5.74%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events	1		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Chronic myeloid leukaemia			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Small cell lung cancer			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Reproductive system and breast disorders			
Metrorrhagia			

subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Uterine haemorrhage			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Uterine polyp			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Allergic bronchitis			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Asthma			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Chronic obstructive pulmonary disease			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumothorax			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural			

complications			
Foot fracture			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Fracture displacement			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Acute myocardial infarction			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Left ventricular failure			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Myocardial infarction			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Myasthenia gravis			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Gastrooesophageal reflux disease			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Liver injury			

subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Skin and subcutaneous tissue disorders			
Angioedema			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Ureterolithiasis			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Influenza			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory tract infection			
subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Sinusitis			

subjects affected / exposed	0 / 122 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Metabolism and nutrition disorders			
Mineral metabolism disorder			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Placebo	GB001 20 mg	GB001 40 mg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	45 / 120 (37.50%)	53 / 120 (44.17%)	59 / 118 (50.00%)
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	1 / 120 (0.83%)	2 / 120 (1.67%)	3 / 118 (2.54%)
occurrences (all)	1	2	6
Aspartate aminotransferase increased			
subjects affected / exposed	2 / 120 (1.67%)	2 / 120 (1.67%)	4 / 118 (3.39%)
occurrences (all)	2	2	7
Vascular disorders			
Hypertension			
subjects affected / exposed	2 / 120 (1.67%)	3 / 120 (2.50%)	6 / 118 (5.08%)
occurrences (all)	3	3	7
Nervous system disorders			
Headache			
subjects affected / exposed	11 / 120 (9.17%)	14 / 120 (11.67%)	14 / 118 (11.86%)
occurrences (all)	12	24	22
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	3 / 120 (2.50%)	6 / 120 (5.00%)	1 / 118 (0.85%)
occurrences (all)	3	8	1
Skin and subcutaneous tissue disorders			
Pruritus			

subjects affected / exposed occurrences (all)	1 / 120 (0.83%) 2	1 / 120 (0.83%) 1	2 / 118 (1.69%) 2
Infections and infestations			
Bronchitis			
subjects affected / exposed	6 / 120 (5.00%)	4 / 120 (3.33%)	1 / 118 (0.85%)
occurrences (all)	7	4	1
Nasopharyngitis			
subjects affected / exposed	19 / 120 (15.83%)	23 / 120 (19.17%)	29 / 118 (24.58%)
occurrences (all)	26	26	41
Rhinitis			
subjects affected / exposed	6 / 120 (5.00%)	1 / 120 (0.83%)	2 / 118 (1.69%)
occurrences (all)	8	1	2
Sinusitis			
subjects affected / exposed	3 / 120 (2.50%)	4 / 120 (3.33%)	10 / 118 (8.47%)
occurrences (all)	4	4	10
Upper respiratory tract infection			
subjects affected / exposed	7 / 120 (5.83%)	3 / 120 (2.50%)	8 / 118 (6.78%)
occurrences (all)	7	4	9

Non-serious adverse events	GB001 60 mg		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	55 / 122 (45.08%)		
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	13 / 122 (10.66%)		
occurrences (all)	15		
Aspartate aminotransferase increased			
subjects affected / exposed	13 / 122 (10.66%)		
occurrences (all)	14		
Vascular disorders			
Hypertension			
subjects affected / exposed	5 / 122 (4.10%)		
occurrences (all)	5		
Nervous system disorders			
Headache			
subjects affected / exposed	13 / 122 (10.66%)		
occurrences (all)	24		

Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	4 / 122 (3.28%)		
occurrences (all)	5		
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	8 / 122 (6.56%)		
occurrences (all)	11		
Infections and infestations			
Bronchitis			
subjects affected / exposed	1 / 122 (0.82%)		
occurrences (all)	1		
Nasopharyngitis			
subjects affected / exposed	17 / 122 (13.93%)		
occurrences (all)	24		
Rhinitis			
subjects affected / exposed	7 / 122 (5.74%)		
occurrences (all)	8		
Sinusitis			
subjects affected / exposed	3 / 122 (2.46%)		
occurrences (all)	3		
Upper respiratory tract infection			
subjects affected / exposed	4 / 122 (3.28%)		
occurrences (all)	4		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
28 February 2019	Amendment 1 (protocol version 2)
28 August 2019	Amendment 2 (protocol version 3)
18 February 2020	Amendment 3 (protocol version 4)
16 April 2020	Amendment 4 (protocol version 5)

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported