Clinical trial results:
A multicenter, Phase 2a, open-label, non-randomized study evaluating the efficacy, safety, and tolerability of BIVV020 in adults with persistent/chronic immune thrombocytopenia (ITP)

Summary

EudraCT number	2020-004162-18
Trial protocol	DE CZ NL ES
Global end of trial date	07 February 2023

Results information

Result version number	v2 (current)
This version publication date	28 March 2024
First version publication date	21 February 2024
Version creation reason	• Correction of full data set EudraCT results are updated to maintain the consistency between EudraCT results and Clinicaltrials.gov results.

Trial information

Trial identification

Sponsor protocol code	PDY16894

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04669600
WHO universal trial number (UTN)	U1111-1253-2343

Notes:

Sponsors

Sponsor organisation name	Sanofi aventis recherche & développement
Sponsor organisation address	1 avenue Pierre Brossolette, Chilly-Mazarin, France, 91380
Public contact	Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.com
Scientific contact	Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.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	05 May 2023
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	07 February 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the effect of SAR445088 on the durability of platelet response in subjects with persistent/chronic immune thrombocytopenia (ITP).

Protection of trial subjects:

Subjects were fully informed of all pertinent aspects of clinical trial as well as the possibility to discontinue at any time in language and terms appropriate for the subject and considering the local culture. During the course of the trial, subjects were provided with individual subject cards indicating the nature of the trial the subject is participating, contact details and any information needed in the event of a medical emergency.
Collected personal data and human biological samples were processed in compliance with the Sanofi-Aventis Group Personal Data Protection Charter ensuring that the Group abides by the laws governing personal data protection in force in all countries in which it operates.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	04 February 2021
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	Germany: 1
Country: Number of subjects enrolled	Netherlands: 2
Country: Number of subjects enrolled	Spain: 2
Country: Number of subjects enrolled	United States: 7
Worldwide total number of subjects	12
EEA total number of subjects	5

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)	10
From 65 to 84 years	2
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study was conducted at 8 centres in 5 countries. A total of 20 subjects were screened from 04 Feb 2021 to 07 Sep 2021, of which 8 were screen failures due to not meeting eligibility criteria.

Pre-assignment

Screening details:

The study consisted of a screening period (up to 56 days), treatment period (up to 81 weeks), and follow-up visits (up to 22 weeks). A total of 12 subjects [either switchers: who had received and responded to sutimlimab (BIVV009) in study TDR16218 (NCT03275454) or naïve: who have not previously received sutimlimab] were enrolled 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

Arm title	SAR445088

Arm description:

Subjects received a loading dose of SAR445088 (BIVV020) 50 milligram per kilogram (mg/kg) intravenously (IV) on Day 1, followed by maintenance doses of 600 mg subcutaneous (SC) weekly starting on Day 8 until the last subject enrolled completed 52 weeks of treatment. The maximum duration of treatment for an individual subject was up to 81 weeks.

Arm type	Experimental
Investigational medicinal product name	SAR445088
Investigational medicinal product code	
Other name	BIVV020
Pharmaceutical forms	Concentrate for solution for injection/infusion
Routes of administration	Intravenous use, Subcutaneous use

Dosage and administration details:

BIVV020 was supplied in single use vial and administered as a loading dose of 50 mg/kg IV infusion on Day 1, followed by maintenance doses of 600 mg SC weekly starting on Day 8 until the last subject completed 52 weeks of treatment.

Number of subjects in period 1	SAR445088
Started	12
Completed	5
Not completed	7
Consent withdrawn by subject	1
Unspecified	6

Baseline characteristics

Reporting groups	
Reporting group title	SAR445088

Reporting group description:

Subjects received a loading dose of SAR445088 (BIVV020) 50 milligram per kilogram (mg/kg) intravenously (IV) on Day 1, followed by maintenance doses of 600 mg subcutaneous (SC) weekly starting on Day 8 until the last subject enrolled completed 52 weeks of treatment. The maximum duration of treatment for an individual subject was up to 81 weeks.

Reporting group values	SAR445088	Total	
Number of subjects	12	12	
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	54.7		
standard deviation	± 10.1	-	
Gender categorical Units: Subjects			
Female	4	4	
Male	8	8	
Race Units: Subjects			
American Indian or Alaska Native	0	0	
Asian	0	0	
Native Hawaiian or Other Pacific Islander	0	0	
Black or African American	3	3	
White	7	7	
More than one race	0	0	
Unknown or Not Reported	2	2	

End points

End points reporting groups

Reporting group title	SAR445088

Reporting group description:

Subjects received a loading dose of SAR445088 (BIVV020) 50 milligram per kilogram (mg/kg) intravenously (IV) on Day 1, followed by maintenance doses of 600 mg subcutaneous (SC) weekly starting on Day 8 until the last subject enrolled completed 52 weeks of treatment. The maximum duration of treatment for an individual subject was up to 81 weeks.

Primary: Percentage of Subjects With a Durable Platelet Response

End point title	Percentage of Subjects With a Durable Platelet Response[1]

End point description:

A naive subject was a subject who did not use sutimlimab prior to enrollment. A switcher was a subject who used sutimlimab prior to enrollment. A naive subject was a responder if the platelet count was >=50 × 10^9/litre (L) at >=50 percent (%) of scheduled visits, or for subjects with baseline platelet count <15 × 10^9/L, a >=20 × 10^9/L increase in platelet count from baseline at >=50% of scheduled visits, without receiving rescue ITP therapy. A switcher was a responder if the maintenance platelet count was >=30 × 10^9/L at >=50% of scheduled visits, without receiving rescue ITP therapy. Results are based on the number of subjects analysed = intent-to-treat (ITT) population which consisted of all exposed subjects. Number analysed (n) = number of subjects for each category (naive subject and switcher).

End point type	Primary

End point timeframe:

From Week 3 to Week 24

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: As the endpoint is descriptive in nature, no statistical analysis is provided.

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: percentage of subjects				
number (confidence interval 95%)				
Naive subject (n = 8) Switcher (n = 4)	0 (0.0 to 36.9) 25 (19.4 to 99.4)			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Treatment Emergent Adverse Events (TEAEs) and Treatment Emergent Serious AEs (SAEs)

End point title	Number of Subjects With Treatment Emergent Adverse Events (TEAEs) and Treatment Emergent Serious AEs (SAEs)

End point description:

An AE was defined as any untoward medical occurrence in a subject temporally associated with the use of study treatment, whether or not considered related to the study treatment. SAEs were any untoward

medical occurrence that at any dose: resulted in death, was life-threatening, required inpatient hospitalisation or prolongation of existing hospitalisation, resulted in persistent or significant disability/incapacity, was a congenital anomaly/birth defect, was a medically important event. TEAEs were AEs that developed after the first study treatment administration in the safety analysis period which was defined as the period from the first study intervention administration to the end of study (EOS) visit (up to Week 103). The Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment.

End point type	Secondary

End point timeframe:

From first study treatment administration (Day 1) up to Week 103

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
Any TEAE	8			
Any Treatment Emergent SAE	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Hematology

End point title	Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Hematology

End point description:

Criteria for potentially clinically significant laboratory abnormalities: White blood cells (WBCs): less than (<)3.0 Giga (G)/L(Non-Black [NB]) or <2.0 G/L(Black [B]),greater than or equal to(>=)16.0 G/L; Lymphocytes: greater than (>)4.0 G/L; Neutrophils:<1.5 G/L (NB) or <1.0 G/L (B);Monocytes:>0.7 G/L; Basophils:>0.1 G/L; Eosinophils:>0.5 /L or >upper limit of normal(ULN)(if ULN >=0.5 G/L);Hemoglobin: less than or equal to(<=)115 grams (g)/L (Male[M]) or <=95 g/L(Female[F]),>=185 g/L(M) or >=165 g/L (F),Decrease from baseline(DFB) >=20 g/L; Hematocrit:<=0.37 volume/volume(v/v) (M) or <=0.32 v/v (F);Red blood cells (RBC): >=6 Tera/L; Platelets:<100 G/L, >=700 G/L. Number of subjects analysed=safety population. Number analysed (n) = number of subjects in 'safety population' with available data for the corresponding categories. Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment.

End point type	Secondary

End point timeframe:

On Days 1, 15, 29, at Weeks 8, 12, 24, and then every 8 weeks until the end of study (EOS) (Week 103)

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
WBCs: <3.0 G/L (NB) or <2.0 G/L (B) (n=12)	0			
WBCs: >=16.0 G/L (n=12)	3			
Lymphocytes: >4.0 G/L (n=12)	2			
Neutrophils: <1.5 G/L (NB) or <1.0 G/L (B) (n=11)	0			
Monocytes: >0.7 G/L (n=12)	7			
Basophils: >0.1 G/L (n=12)	3			
Eosinophils:>0.5 G/L or >ULN(if ULN>=0.5G/L (n=12)	1			
Hemoglobin:<=115 g/L (M) or <=95 g/L (F) (n=12)	0			
Hemoglobin: >=185 g/L (M) or >=165 g/L (F) (n=12)	0			
Hemoglobin: DFB >=20 g/L (n=12)	0			
Hematocrit: <=0.37 v/v (M) or <=0.32 v/v(F) (n=12)	0			
RBC: >=6 Tera/L (n=12)	0			
Platelets: <100 G/L (n=12)	11			
Platelets: >=700 G/L (n=12)	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Clinical Chemistry

End point title	Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Clinical Chemistry

End point description:

Criteria for PCSA: Blood Urea Nitrogen: >=17 millimole (mmol)/L; Creatinine: >=150 micromole (mcmol)/L (Adults), >=30% and <100% change from baseline, >=100% change from baseline; Potassium: <3 mmol/L, >=5.5 mmol/L; Sodium: <=129 mmol/L, >=160 mmol/L; Aspartate Aminotransferase (AST): >3 ULN, >5 ULN, >10 ULN, >20 ULN; Alanine Aminotransferase (ALT): >3 ULN, >5 ULN, >10 ULN, >20 ULN; Alkaline Phosphatase (ALP): >1.5 ULN; Bilirubin: >1.5 ULN, >2 ULN; ALT and Total Bilirubin (TBILI): ALT >3 ULN and TBILI >2 ULN. Only the worst case during the TE period for each subject with worsening from baseline is presented. The Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment.

End point type	Secondary

End point timeframe:

On Days 1, 15, 29, Weeks 8, 12 and 24, then every 8 weeks until the EOS (Week 103)

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
Blood Urea Nitrogen: >=17 mmol/L	0			
Creatinine: >=150 mcmol/L (Adults)	1			
Creatinine: >=30% and < 100% from baseline	4			
Creatinine:>=100% from baseline	1			
Potassium: <3 mmol/L	0			
Potassium: >=5.5 mmol/L	1			
Sodium:<=129 mmol/L	0			
Sodium: >=160 mmol/L	0			
AST: >3 ULN	0			
AST: >5 ULN	0			
AST: >10 ULN	0			
AST: >20 ULN	0			
ALT: >3 ULN	0			
ALT: >5 ULN	0			
ALT: >10 ULN	0			
ALT: >20 ULN	0			
ALP: > 1.5 ULN	1			
Bilirubin: >1.5 ULN	0			
Bilirubin: >2 ULN	0			
ALT and total bilirubin: ALT>3ULN and TBILI >2ULN	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Coagulation

End point title	Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Coagulation

End point description:

The number of subjects with PCSA for coagulation parameters during the TE period without PCSA definition by biological function are presented. The parameters evaluated were prothrombin time (PT), prothrombin international normalised ratio (INR) and activated partial thromboplastin time (APTT). The Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment. Number of subjects analysed = Number of subjects in the 'safety population' with available data for this outcome measure. Number analysed (n) = Number of subjects in the 'safety population' with available data for the corresponding categories. Some subjects in 'Number Analysed' were common for 2 or for all the 3 categories (Prothrombin time, Prothrombin International Normalized Ratio and APTT).

End point type	Secondary

End point timeframe:

On Days 1, 15, 29, Weeks 8, 12 and 24, then every 8 weeks until the EOS (Week 103)

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: subjects				
number (not applicable)				
PT: <Lower limit of normal (LLN) (n=3)	1			
PT: >ULN (n=3)	2			
INR: <LLN (n=4)	2			
INR: >ULN (n=4)	2			
APTT: <LLN (n=4)	1			
APTT: >ULN (n=4)	3			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Urinalysis

End point title	Number of Subjects With Potentially Clinically Significant Laboratory Abnormalities: Urinalysis

End point description:

Criteria for PCSA: potential of Hydrogen (pH) <=4.6, >=8. Only the worst case during the TE period for each subject with worsening from baseline is presented. The Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment.

End point type	Secondary

End point timeframe:

On Days 1, 15, 29, Weeks 8, 12 and 24, then every 8 weeks until the EOS (Week 103)

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
pH: <=4.6	0			
pH: >=8	1			

Statistical analyses

No statistical analyses for this end point

Secondary: Plasma Concentrations of SAR445088 (BIVV020)

End point title	Plasma Concentrations of SAR445088 (BIVV020)

End point description:

Plasma samples were collected at specified timepoints. The Pharmacokinetic (PK) population consisted of all enrolled and treated subjects (safety population) with at least 1 post-baseline PK sample. Only those subjects with data available were included in the analysis and are denoted by 'n' in the category titles. Here '99999'= Standard deviation could not be derived for a single subject.

End point type	Secondary

End point timeframe:

1-hour post-dose on Day 1, on Days 8, 15, 29, 43, at Weeks 12, 16, 24, 32, 40, 48, 56, 64, 72, 80 and EOS visit, up to 103 weeks

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: microgram/millilitre (mcg/mL)				
arithmetic mean (standard deviation)				
Day 1: 1-hour post-dose (n=12)	1186.00 (± 254.24)			
Day 8 (n=11)	668.09 (± 139.19)			
Day 15 (n=11)	657.64 (± 107.33)			
Day 29 (n=10)	631.80 (± 64.52)			
Day 43 (n=9)	627.56 (± 84.39)			
Week 12 (n=9)	736.78 (± 164.07)			
Week 16 (n=1)	786.00 (± 99999)			
Week 24 (n=8)	781.63 (± 276.73)			
Week 32 (n=6)	695.67 (± 343.75)			
Week 40 (n=6)	602.33 (± 385.64)			
Week 48 (n=6)	559.33 (± 300.73)			
Week 56 (n=5)	602.80 (± 224.08)			
Week 64 (n=5)	615.80 (± 247.91)			
Week 72 (n=4)	584.25 (± 243.37)			
Week 80 (n=2)	602.50 (± 287.79)			
EOS (Week 103) (n=10)	206.46 (± 157.42)			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Responders to SAR445088 (BIVV020)

End point title	Number of Responders to SAR445088 (BIVV020)

End point description:

A subject was a responder if the platelet count was >=50 × 10^9/L and there was a greater than 2-fold increase from baseline, measured on 2 occasions at least 7 days apart with the absence of bleeding [bleeding score >=2 on the World Health Organization (WHO) bleeding scale] while the platelet counts were maintained above the threshold and lack of combination ITP therapy during this period. WHO bleeding scores: 1=Petechiae; 2=Mild blood loss; 3=Gross blood loss; and 4=Debilitating blood loss. The ITT population consisted of all exposed subjects.

End point type	Secondary

End point timeframe:

At Weeks 24 and 56

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
Week 24	2			
Week 56	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Time to First Platelet Response

End point title	Time to First Platelet Response

End point description:

Time to first platelet response was defined as greater than or equal to each of the following values: 50 × 10^9/L or 100 × 10^9/L (confirmed by 2 measurements at least 7 days apart). It was calculated as date of first occurrence of confirmed platelet count response before rescue therapy. Number of subjects analysed = ITT Population which consisted of all exposed subjects. Number analysed (n) = number of subjects in the 'ITT Population' who met the specified platelet counts (>=50 x10^9/L or >=100 x10^9/L) as confirmed by 2 measurements at least 7 days apart. Subjects who met the criteria ‘Platelet count >=100 x10^9/L’ were the same as who met the criteria ‘Platelet count >=50 x10^9/L’.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) up to Week 56

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: weeks				
median (inter-quartile range (Q1-Q3))				
Platelet count>=50 x 10^9/L (n=4)	18.5 (13.5 to 49.5)			
Platelet count>=100 x 10^9/L (n=4)	18.5 (13.5 to 49.5)			

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Subjects who did not Require Rescue Therapy for an Acute Episode of Thrombocytopenia After Week 3

End point title	Percentage of Subjects who did not Require Rescue Therapy for an Acute Episode of Thrombocytopenia After Week 3

End point description:

Data was collected to assess the effect of treatment with SAR445088 (BIVV020) on the requirement for rescue ITP therapy. The ITT population consisted of all exposed subjects.

End point type	Secondary

End point timeframe:

Up to Week 84

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: percentage of subjects				
number (not applicable)				
Week 3 to Week 24 during treatment period	75.0			
Week 3 to Week 56 during treatment period	75.0			
Week 3 to the end of on-treatment period	75.0			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects With Anti-Drug Antibody (ADAs) Response to SAR445088 (BIVV020)

End point title	Number of Subjects With Anti-Drug Antibody (ADAs) Response to SAR445088 (BIVV020)

End point description:

Plasma samples were analysed for the presence of ADAs for SAR445088 (BIVV020) using validated assays. Treatment-induced ADA was defined as ADAs that developed during the TE period and without pre-existing ADA. Pre-existing ADA was defined as ADAs present in samples drawn before first study treatment administration. Treatment-boosted ADA positive was defined as pre-existing ADA (i.e., ADA positive at baseline) that was boosted at least a 9-fold increase of titer values during the TE period than the baseline. The TE ADA positive was defined as either treatment-induced ADA positive or treatment-boosted ADA positive during the TE period. Inconclusive ADA was defined as the one that could not irrefutably be classified as with or without TE ADA. The ADA population consisted of all enrolled and treated subjects (safety population) with at least 1 post-baseline ADA sample. Negative: -ve; Treatment period: TP.

End point type	Secondary

End point timeframe:

Up to Week 103

End point values	SAR445088			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: subjects				
number (not applicable)				
Subjects with ADA -ve or missing at baseline	11			
Subjects with treatment-induced ADA	0			
Subjects with pre-existing ADA	1			
Subjects with treatment-boosted ADA	0			
Subjects with TE ADA during 24- week TP	0			
Subjects with TE ADA during 52- week TP	0			
Subjects without TE ADA	12			
Subjects with inconclusive ADA	0			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

The TEAEs were collected from first study treatment administration (Day 1) up to Week 103

Adverse event reporting additional description:

The Safety population consisted of all enrolled subjects who took at least 1 dose (including partial dose) of study treatment. As prespecified, AEs recorded for doses 50 mg/kg (loading dose) and 600 mg (maintenance dose) of SAR445088 are presented under the reporting group, SAR445088.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	25.1

Reporting groups

Reporting group title	SAR445088

Reporting group description:

Subjects received a loading dose of SAR445088 (BIVV020) 50 mg/kg IV on Day 1, followed by maintenance doses of 600 mg SC weekly starting on Day 8 until the last subject enrolled completed 52 weeks of treatment. The maximum duration of treatment for an individual subject was up to 81 weeks.

Serious adverse events	SAR445088		
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 12 (16.67%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Nervous system disorders			
Cerebrovascular Accident			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Abdominal Pain Upper			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Appendicitis			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	SAR445088		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	8 / 12 (66.67%)		
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
General disorders and administration site conditions			
Oedema Peripheral			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Injection Site Bruising			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Fatigue			
subjects affected / exposed	3 / 12 (25.00%)		
occurrences (all)	3		
Respiratory, thoracic and mediastinal disorders			
Sinus Congestion			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Cough			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Investigations			
Blood Cholesterol Increased			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Nervous system disorders			
Aphasia			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Burning Sensation			

subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	2		
Dizziness			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Syncope			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Ear and labyrinth disorders			
Tinnitus			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Eye disorders			
Eye Irritation			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Gastrointestinal disorders			
Abdominal Pain			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Constipation			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Diarrhoea			
subjects affected / exposed	2 / 12 (16.67%)		
occurrences (all)	2		
Dyspepsia			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Hepatobiliary disorders			
Cholecystitis Chronic			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		

Skin and subcutaneous tissue disorders			
Dermatitis Contact			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Psoriasis			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Arthritis			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Back Pain			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Myalgia			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Pain In Extremity			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Infections and infestations			
Covid-19			
subjects affected / exposed	3 / 12 (25.00%)		
occurrences (all)	4		
Influenza			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	2		
Post-Acute Covid-19 Syndrome			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Sinusitis			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		

Metabolism and nutrition disorders Decreased Appetite subjects affected / exposed occurrences (all) Hyperglycaemia subjects affected / exposed occurrences (all)	 1 / 12 (8.33%) 1 1 / 12 (8.33%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 December 2020	Changes in protocol were implemented to address requests received in a Voluntary Harmonisation Procedure communication. Added limit as a safety measure and added further information to support likelihood of long-term safety. Specified additional safety monitoring. Added mention of Data Monitoring Committee to synopsis, renamed "Data Monitoring Committee" subheading as "Study Monitoring Committee," and added a brief description of the Data Monitoring Committee, which included at least 2 investigators participating in the study. Required contraception extended due to the prolonged half-life of BIVV020. Additional details for investigational medicinal product administration added as per Health Authority request. Added statement referring to the Pharmacy Manual for procedure details. Clarified that thrombopoietin agonists may be restarted by the Investigator after BIVV020 administration if a subject has an insufficient response to BIVV020. Added guidance for thrombocytosis. Clarified that the Declaration of Helsinki guidelines referenced are the most recent guidelines from 2013. Double-barrier contraception removed as a highly effective contraceptive method as per the Clinical Trial Facilitation Group.
10 December 2020	The protocol was amended to address requests received from the United States Food and Drug Administration as well as for additional corrections and clarifications. Pre-dose weight added on Day 1. Clarified that clear titer cutoffs associated with protection do not exist for most of the required vaccines. Clarified an inclusion criterion: For subjects who have not previously received sutimlimab, one of their prior treatments must have been a thrombopoietin receptor agonist. Clarified that vaccinations, such as the seasonal influenza vaccine, are permitted during the study. Clarified that a dose increase in a concomitant ITP medication is considered rescue therapy. Clarified the testing to be performed in case of hypersensitivity/allergic reaction. Additional requirements and considerations for AE/SAE reporting clarified for subjects transitioning from study TDR16218. Addition of a dosing and enrollment hold for toxicity. Thrombopoietin removed from clinical chemistry panel as it was not essential for study and could not be performed through the central laboratory. Additional clarifications and corrections to typography and formatting to maintain consistency with document standards.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported