Clinical trial results:
A single-arm, open-label, Phase II study of sabatolimab in combination with azacitidine and venetoclax in adult participants with high or very high-risk myelodysplastic syndrome (MDS) as per IPSS-R criteria.

Due to EudraCT system limitations, which EMA is aware of, data using 999 as data points in this record are not an accurate representation of the clinical trial results. Please use https://www.novctrd.com/CtrdWeb/home.nov for complete trial results.

Summary

EudraCT number	2020-003669-21
Trial protocol	HU DE BE GR FR IT
Global end of trial date	08 May 2023

Results information

Result version number	v1 (current)
This version publication date	06 March 2024
First version publication date	06 March 2024

Trial information

Trial identification

Sponsor protocol code	CMBG453B12203

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04812548
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Novartis Pharma, AG
Sponsor organisation address	CH-4002, Basel, Switzerland,
Public contact	Clinical Disclosure Office, Novartis Pharma, AG, 41 613241111, novartis.email@novartis.com
Scientific contact	Clinical Disclosure Office, Novartis Pharma, AG, 41 613241111, novartis.email@novartis.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric	No

investigation plan (PIP)	
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	08 May 2023
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	08 May 2023
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

Safety run-in (Cohort 1 and Cohort 2 of Part 1):
To determine whether sabatolimab is safe when added to azacitidine + venetoclax in participants with high or very high risk MDS per IPSS-R criteria.

Cohort 2 of Safety run-in (Part 1) and Expansion (Part 2):
To determine the complete remission (CR) rate of sabatolimab in combination with azacitidine and venetoclax in participants with high or very high risk MDS as per IPSS-R criteria treated with sabatolimab at 800 mg Q4W.

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and the International Conference on Harmonization (ICH) Good Clinical Practice (GCP) guidelines. All the local regulatory requirements pertinent to safety of trial subjects were also followed during the conduct of the trial.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	31 May 2021
Long term follow-up planned	Yes
Long term follow-up rationale	Efficacy
Long term follow-up duration	22 Months
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	France: 5
Country: Number of subjects enrolled	Belgium: 2
Country: Number of subjects enrolled	Germany: 2
Country: Number of subjects enrolled	Greece: 5
Country: Number of subjects enrolled	Hungary: 1
Country: Number of subjects enrolled	Italy: 2

Country: Number of subjects enrolled	Spain: 3
Worldwide total number of subjects	20
EEA total number of subjects	20

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)	5
From 65 to 84 years	15
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Ten centers across 7 countries enrolled a total of 20 participants in this study.

Pre-assignment

Screening details:

Prior to dosing at Cycle 1 Day 1, participants who fulfilled all the inclusion/exclusion criteria were enrolled via IRT and a treatment number was provided for the study treatments sabatolimab, venetoclax, and azacitidine.

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	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)

Arm description:

Part 1 Cohort 1: Safety run-in cohort of a lower dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.

Arm type	Experimental
Investigational medicinal product name	sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Concentrate and solvent for solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

administered intravenously at 400 mg during Safety run-in Cohort 1 on Day 8 (Q4W).

Investigational medicinal product name	venetoclax
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

administered orally at 400 mgdaily for 14 consecutive days,during Safety run-in (Part 1)

Investigational medicinal product name	azacitidine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate and solvent for solution for infusion, Concentrate and solvent for solution for injection
Routes of administration	Intravenous use, Subcutaneous use

Dosage and administration details:

azacitidine was to be administered intravenously or subcutaneously at 75 mg/m2on Days 1 to 7 (or, at discretion of the investigator on Days 1-5 and Day 8-9), during Safety run-in (Part 1)

Arm title	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)

Arm description:

Part 1 Cohort 2: Safety run-in cohort of a higher dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.

Arm type	Experimental

Investigational medicinal product name	sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Concentrate and solvent for solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

administered intravenously at 800 mg during Safety run-in Cohort 2 on Day 8 (Q4W).

Investigational medicinal product name	venetoclax
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

administered orally at 400 mgdaily for 14 consecutive days,during Safety run-in (Part 1)

Investigational medicinal product name	azacitidine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate and solvent for solution for infusion, Concentrate and solvent for solution for injection
Routes of administration	Intravenous use, Subcutaneous use

Dosage and administration details:

azacitidine was to be administered intravenously or subcutaneously at 75 mg/m2on Days 1 to 7 (or, at discretion of the investigator on Days 1-5 and Day 8-9), during Safety run-in (Part 1)

Number of subjects in period 1	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)
Started	5	15
Did not enter post-treatment follow-up	1	5
Entered post-treatment follow-up	4	10
Completed	0	0
Not completed	5	15
Adverse event, serious fatal	-	1
Physician decision	1	-
Consent withdrawn by subject	-	1
Adverse event, non-fatal	2	7
Post Study Access to Treatment	-	1
Progressive Disease	-	2
HSCT Planned	2	3

Baseline characteristics

Reporting groups	
Reporting group title	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)
Reporting group description:	
Part 1 Cohort 1: Safety run-in cohort of a lower dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.	
Reporting group title	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)
Reporting group description:	
Part 1 Cohort 2: Safety run-in cohort of a higher dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.	

Reporting group values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)	Total
Number of subjects	5	15	20
Age categorical Units: Subjects			
Adults (18-64 years)	2	3	5
From 65-84 years	3	12	15
Age Continuous Units: Years			
arithmetic mean	67.2	69.3	
standard deviation	± 11.28	± 9.45	-
Sex: Female, Male Units: Participants			
Female	0	4	4
Male	5	11	16
Race/Ethnicity, Customized Units: Subjects			
White	4	14	18
Unknown	1	1	2

End points

End points reporting groups

Reporting group title	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)
Reporting group description:	
Part 1 Cohort 1: Safety run-in cohort of a lower dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.	
Reporting group title	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)
Reporting group description:	
Part 1 Cohort 2: Safety run-in cohort of a higher dose of sabatolimab in combination with fixed dose of venetoclax and azacitidine.	

Primary: Incidence of dose limiting toxicities (DLTs) - All grades (Safety run-in patients only)

End point title	Incidence of dose limiting toxicities (DLTs) - All grades (Safety run-in patients only)[1]
End point description:	
Assessment of tolerability of sabatolimab (MBG453) in combination with venetoclax and azacitidine	
End point type	Primary
End point timeframe:	
From Cycle 1 Day 8 to end of Cycle 2 (Cycle = 28 Days)	

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 done for this endpoint

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	4	13		
Units: Participants				
Number of participants with at least one event	0	2		
Blood and lymph. syst disorders (Thrombocytopenia)	0	1		
Nerv. Syst. disorders (Haemorrhage intracranial)	0	1		

Statistical analyses

No statistical analyses for this end point

Primary: Percentage of participants (receiving 800mg sabatolimab) achieving complete remission (CR) per investigator assessment

End point title	Percentage of participants (receiving 800mg sabatolimab) achieving complete remission (CR) per investigator assessment[2][3]

End point description:

This endpoint assessed Complete Remission (CR) Rate of participants from Cohort 2 of Part 1 and Part 2 according to Investigator assessment per modified IWG-MDS - Cheson 2006 criteria. CR is defined as follows: bone marrow blasts <=5%, hemoglobin level ≥ 10 g/dL, platelets count ≥ 100*10^9/L, neutrophils count ≥ 1.0*10^9/L, absence of blasts in peripheral blood.

End point type	Primary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[2] - 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 done for this endpoint

[3] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: Participants	1			

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of transfusion independence

End point title	Duration of transfusion independence

End point description:

Sum of each period of the transfusion independence for participants with at least one period of transfusion independence post-baseline by dose level for the safety run-in part (Cohort 1 (400 mg Q4W) and Cohort 2 (800 mg Q4W)) and for participants treated with sabatolimab 800 mg (Q4W) (Cohort 2 of safety run-in and expansion parts) for both red blood cells and platelets.

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Weeks				
arithmetic mean (standard deviation)				
Packed Red Blood Cells (n = 2, 5)	16.43 (± 0.808)	23.11 (± 12.636)		

Platelets (n = 2, 7)	18.93 (± 2.727)	24.02 (± 16.423)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants who are RBC/platelets transfusion independent

End point title	Percentage of participants who are RBC/platelets transfusion independent

End point description:

Improvement in red blood cells (RBC)/platelets transfusion post-baseline as per International Working Group - Myelodysplastic syndromes (IWG-MDS) by dose level for the safety run-in part (Cohort 1 (400 mg Q4W) and Cohort 2 (800 mg Q4W)) and for participants treated with sabatolimab 800 mg (Q4W) (Cohort 2 of safety run-in and expansion parts).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Participants				
RBC	2	5		
Platelets	2	7		

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Response Rate (ORR) of participants who achieved hematologic improvement (HI) or better as best response

End point title	Overall Response Rate (ORR) of participants who achieved hematologic improvement (HI) or better as best response

End point description:

The percentage of participants achieving [CR + mCR + partial remission (PR) + hematologic improvement (HI)], per modified IWG-MDS Cheson 2006 criteria

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Participants	4	13		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of subjects achieving a complete remission (CR) + morphologic complete remission (mCR): Safety run-in (Part 1)

End point title	Percentage of subjects achieving a complete remission (CR) + morphologic complete remission (mCR): Safety run-in (Part 1)

End point description:

Assessed the durability of complete remission (CR) or morphologic complete remission (mCR) rate (defined as the percentage of participants with best overall response of either CR or mCR).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Participants	4	13		

Statistical analyses

No statistical analyses for this end point

Secondary: Trough Serum Concentration (Cmin) sabatolimab

End point title	Trough Serum Concentration (Cmin) sabatolimab

End point description:

Concentration of sabatolimab prior to next dosing or after end of treatment by dose level for the safety run-in part (Cohort 1 (400 mg Q4W) and Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Continuously collected for patients during treatment with sabatolimab up to 150 days after last treatment, approx. 14.2 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	13		
Units: ug/ml				
geometric mean (geometric coefficient of variation)				
Cycle (C) 1 Day (D) 8 (n = 4, 12)	0.0 (± 0.0)	0.0 (± 0.0)		
C2D8 (n = 4, 10)	23.0 (± 137.0)	30.7 (± 41.7)		
C3D8 (n = 1, 6)	0.0 (± 0.0)	34.4 (± 72.2)		
C6D8 (n = 0, 3)	999 (± 999)	68.6 (± 11.5)		
C9D8 (n = 0, 2)	999 (± 999)	71.2 (± 33.8)		
C12D (n = 0, 1)	999 (± 999)	0.0 (± 0.0)		

Statistical analyses

No statistical analyses for this end point

Secondary: Peak Serum Concentration (Cmax) of sabatolimab

End point title	Peak Serum Concentration (Cmax) of sabatolimab

End point description:

Maximal concentration of sabatolimab for participants treated with sabatolimab by dose level for the safety run-in part (Cohort 1 (400 mg Q4W) and Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Continuously collected for patients during treatment with sabatolimab up to 150 days after last treatment, approx. 14.2 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[4]	0[5]		
Units: ug/ml				
geometric mean (geometric coefficient of variation)	()	()		

Notes:

[4] - No subjects were analyzed for this endpoint

[5] - No subjects were analyzed for this endpoint

Statistical analyses

No statistical analyses for this end point

Secondary: Anti-drug Antibody (ADA) prevalence at baseline and ADA incidence on-treatment by dose level

End point title	Anti-drug Antibody (ADA) prevalence at baseline and ADA incidence on-treatment by dose level

End point description:

Immunogenicity of sabatolimab prior to sabatolimab exposure and during treatment

End point type	Secondary

End point timeframe:

Continuously collected for patients during treatment with sabatolimab up to 150 days after last treatment, approx. 14.2 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	4	13		
Units: Participants				
ADA prevalence at baseline	0	2		
ADA incidence (i.e., ADA positive) on-treatment	1	2		
Treatment-induced ADA-positive	1	1		
Treatment-boosted ADA-positive	0	1		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to complete remission(CR)/marrow complete remission (mCR)

End point title	Time to complete remission(CR)/marrow complete remission (mCR)[6]

End point description:

Time to CR/mCR is defined as time from start of treatment to first occurrence of CR or mCR as per investigator assessment for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[6] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: months				
median (confidence interval 95%)	2.33 (1.61 to 2.83)			

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of complete remission (CR)

End point title	Duration of complete remission (CR)[7]

End point description:

Duration of CR is defined as time from first occurrence of CR to relapse from CR, progression or death due to any cause whichever occurs first for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[7] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: months				
median (confidence interval 95%)	999 (999 to 999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of response for participants who achieved hematologic improvement (HI) or better

End point title	Duration of response for participants who achieved hematologic

	improvement (HI) or better[8]

End point description:

The duration of response was derived for participants treated with sabatolimab at the higher dose who achieved HI or better as per investigator assessment and is defined from the first occurrence of complete response (CR), marrow complete response (mCR), partial response (PR) or hematologic improvement (HI) until relapse, progression or death due to any reason for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[8] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	13			
Units: months				
median (confidence interval 95%)	5.60 (4.14 to 99)			

Statistical analyses

No statistical analyses for this end point

Secondary: Leukemia-Free Survival (LFS)

End point title	Leukemia-Free Survival (LFS)[9]

End point description:

Time from start of treatment to transformation to acute leukemias per investigator assessment [as defined as ≥ 20% blasts in bone marrow/ peripheral blood (per WHO 2016 classification) or diagnosis of extramedullary acute leukemia or death due to any cause, whichever occurs first for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[9] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: months				
median (confidence interval 95%)	7.59 (4.24 to 999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Progression-Free Survival (PFS)

End point title	Progression-Free Survival (PFS)[10]

End point description:

Time from start of treatment to disease progression (including transformation to acute leukemia per WHO 2016 classification), relapse from CR or death due to any cause, whichever occurs first for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[10] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: months				
median (confidence interval 95%)	6.77 (3.71 to 999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Event-Free Survival (EFS)

End point title	Event-Free Survival (EFS)[11]

End point description:

Time from start of treatment to lack of reaching CR within the first 6 cycles, relapse from CR or death due to any cause, whichever occurs first for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[11] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: months				
median (confidence interval 95%)	0.03 (0.009 to 999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of complete response (CR)/marrow complete response (mCR)

End point title	Duration of complete response (CR)/marrow complete response (mCR)[12]

End point description:

Duration of CR/mCR is defined as time from first occurrence of CR/mCR to relapse from CR, progression or death due to any cause whichever occurs first for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Throughout study completion, approx. 22.4 months

Notes:

[12] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	13			
Units: months				
median (confidence interval 95%)	5.60 (4.14 to 999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Survival (OS)

End point title	Overall Survival (OS)[13]

End point description:

Time from start of treatment to death due to any cause for the safety run-in part (Cohort 2 (800 mg Q4W)).

End point type	Secondary

End point timeframe:

Date of start of treatment to date of death due to any reason, for up to approx. 22.4 months

Notes:

[13] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: No statistical analysis was done for this endpoint

End point values	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: months				
median (confidence interval 95%)	999 (677 to 999)			

Statistical analyses

No statistical analyses for this end point

Post-hoc: All Collected Deaths

End point title	All Collected Deaths

End point description:

On-treatment deaths were collected from start of study treatment (FPFT) up to 30 days after study drug discontinuation, for a maximum duration of approx. 13 months.

Post-treatment survival follow-up deaths were collected from Day 31 after last dose of study treatment to end of study up to approx. 18.4 months

All deaths refer to the sum of on-treatment and post-treatment survival follow-up deaths, approx. 22.4 months.

End point type	Post-hoc

End point timeframe:

On-treatment deaths: up to approx. 13 months, post-treatment deaths: up to approx 18.4 months

End point values	sabatolimab (MBG453) 400 mg + AZA + VEN (Part 1 Cohort 1)	sabatolimab (MBG453) 800 mg + AZA + VEN (Part 1 Cohort 2)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	5	15		
Units: Participants				
All deaths	1	6		
On-treatment deaths	0	1		

Post-treatment deaths	1	5		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Timeframe for AE	
Adverse event reporting additional description:	
AE additional description	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	26.0

Reporting groups	
Reporting group title	MBG453 800 mg@+ AZA + VEN
Reporting group description:	
MBG453 800 mg@+ AZA + VEN	
Reporting group title	MBG453 400 mg@+ AZA + VEN
Reporting group description:	
MBG453 400 mg@+ AZA + VEN	

Serious adverse events	MBG453 800 mg@+ AZA + VEN	MBG453 400 mg@+ AZA + VEN	
Total subjects affected by serious adverse events			
subjects affected / exposed	14 / 15 (93.33%)	3 / 5 (60.00%)	
number of deaths (all causes)	1	0	
number of deaths resulting from adverse events	1	0	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Paraneoplastic syndrome			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Lung adenocarcinoma			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Investigations			
Medical observation			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Injury, poisoning and procedural complications			
Limb injury			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Wound haemorrhage			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (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			
Haemorrhage intracranial			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	5 / 15 (33.33%)	2 / 5 (40.00%)	
occurrences causally related to treatment / all	3 / 5	1 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Febrile bone marrow aplasia			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Anaemia			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	2 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Neutropenia			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Thrombocytopenia			

subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pancytopenia			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Immune system disorders			
Autoinflammatory disease			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Large intestine perforation			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal haemorrhage			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Bacterial sepsis			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
COVID-19			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Peritonitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis viral			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Escherichia bacteraemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (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	1 / 15 (6.67%)	1 / 5 (20.00%)	
occurrences causally related to treatment / all	1 / 2	1 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Septic shock			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	1 / 1	0 / 0	

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

Non-serious adverse events	MBG453 800 mg@+ AZA + VEN	MBG453 400 mg@+ AZA + VEN	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	15 / 15 (100.00%)	5 / 5 (100.00%)	
Vascular disorders			
Thrombosis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hypotension			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hypertension			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Haematoma			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
General disorders and administration site conditions			
Infusion site haematoma			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Injection site erythema			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Injection site pain			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Injection site reaction			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Oedema peripheral			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Hyperthermia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Fatigue			
subjects affected / exposed	2 / 15 (13.33%)	1 / 5 (20.00%)	
occurrences (all)	3	1	
Device related thrombosis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Chills			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Catheter site inflammation			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Asthenia			
subjects affected / exposed	3 / 15 (20.00%)	1 / 5 (20.00%)	
occurrences (all)	3	1	
Peripheral swelling			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Pyrexia			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Reproductive system and breast disorders			
Uterine haemorrhage			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Heavy menstrual bleeding			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Cervical polyp			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Respiratory, thoracic and mediastinal disorders			
Pleural effusion			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Epistaxis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Cough			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Psychiatric disorders			

Confusional state			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Anxiety			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Investigations			
Blood bilirubin increased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Blood folate decreased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Blood lactate dehydrogenase increased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	3	0	
Blood urea increased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
C-reactive protein increased			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Neutrophil count decreased			
subjects affected / exposed	2 / 15 (13.33%)	1 / 5 (20.00%)	
occurrences (all)	4	1	
Platelet count decreased			
subjects affected / exposed	3 / 15 (20.00%)	0 / 5 (0.00%)	
occurrences (all)	5	0	
Serum ferritin decreased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Troponin T increased			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Weight decreased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
White blood cell count decreased			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	3	0	
Cardiac disorders			
Sinus bradycardia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Haemorrhage intracranial			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Dizziness			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	7 / 15 (46.67%)	1 / 5 (20.00%)	
occurrences (all)	17	1	
Bone marrow failure			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Febrile neutropenia			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	3	0	
Thrombocytosis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Thrombocytopenia			

subjects affected / exposed	7 / 15 (46.67%)	3 / 5 (60.00%)	
occurrences (all)	10	4	
Pancytopenia			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Neutropenia			
subjects affected / exposed	8 / 15 (53.33%)	3 / 5 (60.00%)	
occurrences (all)	19	3	
Leukopenia			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Haemolysis			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	2 / 15 (13.33%)	2 / 5 (40.00%)	
occurrences (all)	2	4	
Constipation			
subjects affected / exposed	4 / 15 (26.67%)	2 / 5 (40.00%)	
occurrences (all)	4	2	
Abdominal pain upper			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Abdominal pain			
subjects affected / exposed	2 / 15 (13.33%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Eructation			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Vomiting			
subjects affected / exposed	3 / 15 (20.00%)	1 / 5 (20.00%)	
occurrences (all)	3	1	
Toothache			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	

Stomatitis			
subjects affected / exposed	3 / 15 (20.00%)	1 / 5 (20.00%)	
occurrences (all)	8	1	
Odynophagia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Nausea			
subjects affected / exposed	6 / 15 (40.00%)	1 / 5 (20.00%)	
occurrences (all)	10	1	
Lower gastrointestinal haemorrhage			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Haemorrhoidal haemorrhage			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Flatulence			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Haemorrhoids			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hepatobiliary disorders			
Cholestasis			
subjects affected / exposed	1 / 15 (6.67%)	1 / 5 (20.00%)	
occurrences (all)	2	2	
Hepatic cytolysis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Hepatotoxicity			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hyperbilirubinaemia			
subjects affected / exposed	1 / 15 (6.67%)	1 / 5 (20.00%)	
occurrences (all)	1	1	
Hypertransaminasaemia			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Jaundice			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Skin and subcutaneous tissue disorders			
Erythema			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Rash pruritic			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Rash maculo-papular			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Purpura			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Pruritus			
subjects affected / exposed	2 / 15 (13.33%)	1 / 5 (20.00%)	
occurrences (all)	2	1	
Dermatitis allergic			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Renal and urinary disorders			
Haematuria			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Renal impairment			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Musculoskeletal and connective tissue disorders			
Tendonitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Pain in extremity			

subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Myalgia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Arthralgia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Infections and infestations			
COVID-19			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Cellulitis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Clostridium bacteraemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Escherichia bacteraemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Oral herpes			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Sepsis			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Tooth abscess			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Vascular device infection			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Metabolism and nutrition disorders			
Vitamin K deficiency			

subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Tumour lysis syndrome			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Malnutrition			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hypophosphataemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	2	0	
Hypomagnesaemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hypokalaemia			
subjects affected / exposed	2 / 15 (13.33%)	1 / 5 (20.00%)	
occurrences (all)	4	3	
Cell death			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Decreased appetite			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Gout			
subjects affected / exposed	0 / 15 (0.00%)	1 / 5 (20.00%)	
occurrences (all)	0	1	
Hyperkalaemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hypernatraemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	
Hyperuricaemia			
subjects affected / exposed	1 / 15 (6.67%)	0 / 5 (0.00%)	
occurrences (all)	1	0	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
17 May 2021	The purpose of this amendment was to address Health Authorities' requests (from Belgium and France) to specify that women of childbearing potential using a hormonal contraception should add a barrier method, as stated in the venetoclax SmPC, and to add a cross-reference to venetoclax local label in the prohibited medication section. Additionally, preliminary results of the first cohort of the safety run-in of CMBG453C12201 study were included. The pharmaceutical dose form and route of administration terms for azacitidine were updated to be in alignment with other sabatolimab protocols.
06 October 2021	The purpose of this amendment was to clarify DLT criteria to specify that prolonged cytopenias beyond Day 42 from the start of a study treatment cycle should be considered DLTs and to clarify language around intercurrent events in the estimand section as well as analysis of duration of response. Additionally, language around COVID-19 vaccines and time frame for SAE follow-up reporting was added.

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.

Due to EudraCT system limitations, which EMA is aware of, data using 999 as data points in this record are not an accurate representation of the clinical trial results. Please use https://www.novctrd.com/CtrdWeb/home.nov for complete trial results.

Notes: