Clinical trial results:
Phase Ib/II, open label study of sabatolimab as a treatment for patients with acute myeloid leukemia and presence of measurable residual disease after allogeneic stem cell transplantation.

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 for complete trial results.

Summary

EudraCT number	2020-000869-17
Trial protocol	FR DE IT
Global end of trial date	22 August 2024

Results information

Result version number	v1
This version publication date	09 March 2025
First version publication date	09 March 2025

Trial information

Trial identification

Sponsor protocol code	CMBG453F12201

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04623216
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 Pharmaceuticals, 41 613241111, novatis.email@novartis.com
Scientific contact	Clinical Disclosure Office, Novartis Pharmaceuticals, 41 613241111, novatis.email@novartis.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?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	22 August 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	22 August 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

For adults only (from the safety run-in part): To determine whether sabatolimab as monotherapy at the two tested dose levels (400 mg and 800 mg Q4W) leads to an unacceptable level of toxicity when administered to adult participants with AML who are in complete remission but are MRD+ post-aHSCT.

For adults only (from both safety run-in and expansion parts): To evaluate preliminary efficacy of sabatolimab (at the recommended dose level for expansion) as monotherapy and in combination with azacitidine on prevention of hematologic relapse by assessing the proportion of adult participants with AML and MRD+ post-aHSCT,) who remain with no evidence of hematologic relapse after 6 cycles of study treatment.

For adolescents only: To determine whether sabatolimab as monotherapy at the recommended dose level for adults leads to an unacceptable level of toxicity when administered to adolescent participants with AML who are in complete remission but are MRD+ post-aHSCT.

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	14 September 2021
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	France: 3
Country: Number of subjects enrolled	Germany: 8

Country: Number of subjects enrolled	Italy: 10
Country: Number of subjects enrolled	Spain: 3
Worldwide total number of subjects	24
EEA total number of subjects	24

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)	1
Adults (18-64 years)	16
From 65 to 84 years	7
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

A total of 24 participants were enrolled in this study: 21 in Safety run-in sabatolimab monotherapy and 3 in Expansion part.

Pre-assignment

Screening details:

Due to the recruitment halt by Novartis, recruitment in the expansion phase was not completed.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Sabatolimab 400mg mono adults

Arm description:

Safety run-in cohort: Adult participants in this arm received sabatolimab 400mg only.

Arm type	Experimental
Investigational medicinal product name	Sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

sabatolimab 400 mg/4ml (MBG453 400 mg liquid in vial 4 ml)

Arm title	Sabatolimab 800mg mono adults

Arm description:

Safety run-in cohort: Adult participants in this arm received sabatolimab 800mg only.

Arm type	Experimental
Investigational medicinal product name	Sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

sabatolimab 800 mg/4ml (MBG453 400 mg liquid in vial 4 ml)

Arm title	Sabatolimab 800mg + Azacitidine adults

Arm description:

Expansion cohort: Adult participants in this arm received sabatolimab 800 mg + azacitidine combination.

Arm type	Experimental
Investigational medicinal product name	azacitidine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use, Subcutaneous use

Dosage and administration details: azacitidine 100 mg (azacitidine 100 mg lyophilizate in vial)	
Investigational medicinal product name	Sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use
Dosage and administration details: sabatolimab 800 mg/4ml (MBG453 400 mg liquid in vial 4 ml)	
Arm title	Sabatolimab 800mg mono adolescent
Arm description: Adolescent safety cohort: ≥12 to < 18-year-old adolescent participants in this arm received sabatolimab 800mg only.	
Arm type	Experimental
Investigational medicinal product name	Sabatolimab
Investigational medicinal product code	MBG453
Other name	
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use
Dosage and administration details: sabatolimab 800 mg/4ml (MBG453 400 mg liquid in vial 4 ml)	

Number of subjects in period 1	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults
Started	10	11	2
Didn't enter post-trtmnt follow-up (f/u)	7	9	2
Entered post-treatment f/u, discontinued	3	2	0
Completed treatment	2	0	0
Completed	1	7	0
Not completed	9	4	2
Adverse event, serious fatal	5	2	-
Terminated by Sponsor	3	2	2
Physician decision	1	-	-

Number of subjects in period 1	Sabatolimab 800mg mono adolescent
Started	1
Didn't enter post-trtmnt follow-up (f/u)	1
Entered post-treatment f/u, discontinued	0
Completed treatment	0
Completed	1
Not completed	0
Adverse event, serious fatal	-
Terminated by Sponsor	-
Physician decision	-

Baseline characteristics

Reporting groups	
Reporting group title	Sabatolimab 400mg mono adults
Reporting group description:	
Safety run-in cohort: Adult participants in this arm received sabatolimab 400mg only.	
Reporting group title	Sabatolimab 800mg mono adults
Reporting group description:	
Safety run-in cohort: Adult participants in this arm received sabatolimab 800mg only.	
Reporting group title	Sabatolimab 800mg + Azacitidine adults
Reporting group description:	
Expansion cohort: Adult participants in this arm received sabatolimab 800 mg + azacitidine combination.	

Reporting group title	Sabatolimab 800mg mono adolescent
Reporting group description:	
Adolescent safety cohort: ≥12 to < 18-year-old adolescent participants in this arm received sabatolimab 800mg only.	

Reporting group values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults
Number of subjects	10	11	2
Age Categorical Units: Participants			
Category 1 : 12 - <18 years	0	0	0
Category 1 : 18 - <65 years	8	7	1
Category 1 : 65 - <85 years	2	4	1
Sex: Female, Male Units: Participants			
Female	5	6	2
Male	5	5	0
Race/Ethnicity, Customized Units: Subjects			
White	9	9	2
Unknown	1	2	0

Reporting group values	Sabatolimab 800mg mono adolescent	Total	
Number of subjects	1	24	
Age Categorical Units: Participants			
Category 1 : 12 - <18 years	1	1	
Category 1 : 18 - <65 years	0	16	
Category 1 : 65 - <85 years	0	7	
Sex: Female, Male Units: Participants			
Female	1	14	
Male	0	10	
Race/Ethnicity, Customized Units: Subjects			
White	1	21	
Unknown	0	3	

End points

End points reporting groups

Reporting group title	Sabatolimab 400mg mono adults
Reporting group description:	
Safety run-in cohort: Adult participants in this arm received sabatolimab 400mg only.	
Reporting group title	Sabatolimab 800mg mono adults
Reporting group description:	
Safety run-in cohort: Adult participants in this arm received sabatolimab 800mg only.	
Reporting group title	Sabatolimab 800mg + Azacitidine adults
Reporting group description:	
Expansion cohort: Adult participants in this arm received sabatolimab 800 mg + azacitidine combination.	
Reporting group title	Sabatolimab 800mg mono adolescent
Reporting group description:	
Adolescent safety cohort: ≥12 to < 18-year-old adolescent participants in this arm received sabatolimab 800mg only.	

Primary: Rate of dose limiting toxicities (Safety Run-in in adult sabatolimab 400mg & 800mg only)

End point title	Rate of dose limiting toxicities (Safety Run-in in adult sabatolimab 400mg & 800mg only)[1][2]
End point description:	
Assessment of tolerability of sabatolimab in adults and adolescents in the post allogenic stem cell transplantation setting. This was determined by the number of participants with at least one event - All grades. A dose-limiting toxicity (DLT) is defined as an adverse event or abnormal laboratory value considered by the Investigator to be at least possibly related to sabatolimab as a single contributor that occurs during the DLT observation period and meets the severity criteria as per protocol.	
End point type	Primary
End point timeframe:	
From Cycle 1 Day 1 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 formal statistical test for this endpoint

[2] - 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 formal statistical test for this endpoint

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	8	10		
Units: Participants	0	1		

Statistical analyses

No statistical analyses for this end point

Primary: Percentage of adult subjects with absence of hematologic relapse per Investigator assessment (Safety Run-in and Expansion)

End point title	Percentage of adult subjects with absence of hematologic relapse per Investigator assessment (Safety Run-in and Expansion)[3]

End point description:

The percentage of adult participants for whom no evidence of hematologic relapse (no evidence of bone marrow blasts ≥5%, no evidence of reappearance of blasts in the blood; no evidence of development of extramedullary disease) has been documented after 6 cycles of study treatment or earlier discontinuation at the recommended dose of MBG453 800 mg.

End point type	Primary

End point timeframe:

From Cycle 1 Day 1 to end of Cycle 6; Cycle = 28 Days

Notes:

[3] - 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 formal statistical test for this endpoint

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	0[4]	11	2	0[5]
Units: Percentage of participants				
number (confidence interval 95%)	(to)	36.4 (10.9 to 69.2)	999 (999 to 999)	(to)

Notes:

[4] - No subjects in this group were analyzed for this endpoint

[5] - No subjects in this group were analyzed for this endpoint

Statistical analyses

No statistical analyses for this end point

Primary: Rate of dose limiting toxicities (Safety confirmation in adolescent cohort only)

End point title	Rate of dose limiting toxicities (Safety confirmation in adolescent cohort only)[6][7]

End point description:

Assessment of tolerability of sabatolimab in adolescent participants in the post allogeneic stem cell transplantation setting. This was determined by the number of participants with at least one event - All grades. A dose-limiting toxicity (DLT) is defined as an adverse event or abnormal laboratory value considered by the Investigator to be at least possibly related to sabatolimab as a single contributor that occurs during the DLT observation period and meets the severity criteria as per protocol.

End point type	Primary

End point timeframe:

From Cycle 1 Day 1 to end of Cycle 2; Cycle =28 Days

Notes:

[6] - 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 formal statistical test for this endpoint

[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 formal statistical test for this endpoint

End point values	Sabatolimab 800mg mono adolescent			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: Participants	999			

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of moderate to severe Chronic GVHD (cGvHD)

End point title	Incidence of moderate to severe Chronic GVHD (cGvHD)

End point description:

Assessment of the treatment emergent moderate or severe cGvHD. Chronic GvHD: Moderate chronic GvHD of the lungs, Severe chronic GvHD.

End point type	Secondary

End point timeframe:

From start of treatment up to 36 months from last patient first treatment

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	11	2	1
Units: Participants	999	999	999	999

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of grade III or IV acute Graft versus Host Disease (aGvHD)

End point title	Incidence of grade III or IV acute Graft versus Host Disease (aGvHD)

End point description:

Assessment of the treatment emergent grade III or IV aGvHD. Acute GvHD: Grade IV acute GvHD, Stage ≥3 lower GI acute GvHD (consistent with Grade III acute GvHD) or Stage ≥3 liver acute GvHD (consistent with Grade III GvHD).

End point type	Secondary

End point timeframe:

From start of treatment up to 36 months from last patient first treatment

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	11	2	1
Units: Participants	999	999	999	999

Statistical analyses

No statistical analyses for this end point

Secondary: Peak of Serum Concentration (Cmax) sabatolimab

End point title	Peak of Serum Concentration (Cmax) sabatolimab

End point description:

Cmax is the maximal serum concentration of sabatolimab.

End point type	Secondary

End point timeframe:

Cycle 1 Day 5 (end of infusion) and Cycle 3 Day 1 or Day 5 (end of infusion) and Cycle 24 Day 1 (end of infusion)

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	6	7	1	0[8]
Units: ug/ml				
geometric mean (geometric coefficient of variation)				
Cycle1 Day5 at 2 hrs (end of infusion) (n=0,0,1,0)	999 (± 999)	999 (± 999)	256 (± 0.0)	()
Cycle3 Day1 at 2 hr (end of infusion) (n=6,7,0, 0)	137 (± 52.7)	304 (± 31.4)	999 (± 999)	()
Cycle3 Day5 at 2 hr (end of infusion) (n=0,0,1,0)	999 (± 999)	999 (± 999)	315 (± 0.0)	()
Cycle24 Day1 at 2 hr (end of infusion) (n=2,0,0,0)	163 (± 23.7)	999 (± 999)	999 (± 999)	()

Notes:

[8] - No subjects in this group were analyzed for this endpoint

Statistical analyses

No statistical analyses for this end point

Secondary: Trough serum concentration of (Cmin) sabatolimab

End point title	Trough serum concentration of (Cmin) sabatolimab

End point description:

Cmin is the concentration of sabatolimab prior to next dosing or after end of treatment.

End point type	Secondary

End point timeframe:

Adult cohorts: Pre-dose on Day 1 (safety run-in) or Day 5 (expansion) of Cycle 1, 3, 6 and 24 (safety run-in only); Adolescent cohort: Pre-dose on Day 1 of Cycle 1, 2, 3 and 6; Cycle = 28 Days

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	6	7	2	1
Units: µg/ml				
geometric mean (geometric coefficient of variation)				
0-hour (hr) Pre-dose at Cycle1 Day1 (n=6,7,0,1)	0.00 (± 0.0)	0.00 (± 0.0)	999 (± 999)	0.00 (± 0.0)
0 hr (pre-dose) at Cycle1 Day5 (0, 0, 2, 0)	999 (± 999)	999 (± 999)	0.00 (± 0.0)	999 (± 999)
0 hr (pre-dose) at Cycle2 Day1 (0, 0, 0, 1)	999 (± 999)	999 (± 999)	999 (± 999)	70.7 (± 0.0)
0 hr (pre-dose) at Cycle3 Day1 (6, 7, 0, 1)	40.4 (± 20.2)	70.7 (± 47.5)	999 (± 999)	129 (± 0.0)
0 hr (pre-dose) at Cycle 3 Day 5 (0, 0, 1, 0)	999 (± 999)	999 (± 999)	42.9 (± 0.0)	999 (± 999)
0 hr (pre-dose) at Cycle 6 Day 1 (4, 5, 0, 1)	46.4 (± 66.4)	99.8 (± 52.1)	999 (± 999)	219 (± 0.0)
0 hr (pre-dose) at Cycle 6 Day 5 (0, 0, 1, 0)	999 (± 999)	999 (± 999)	71.9 (± 0.0)	999 (± 999)
0 hr (pre-dose)at Cycle 24 Day 1 (2, 0, 0, 0)	49.7 (± 44.1)	999 (± 999)	999 (± 999)	999 (± 999)

Statistical analyses

No statistical analyses for this end point

Secondary: Graft versus host disease (GvHD)-free/relapse-free survival (GRFS)

End point title	Graft versus host disease (GvHD)-free/relapse-free survival (GRFS)

End point description:

Time from start of treatment to the date of first documented occurrence or worsening of treatment emergent grade III or IV aGvHD or moderate to severe cGvHD requiring initiation of systemic treatment, morphologic/hematologic relapse, or death due to any cause, whichever occurs first

End point type	Secondary

End point timeframe:

From start of treatment to up to 36 months from last patient first treatment

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	11	2	1
Units: Months				
median (confidence interval 95%)	999 (999 to 999)	999 (999 to 999)	999 (999 to 999)	999 (999 to 999)

Statistical analyses

No statistical analyses for this end point

Secondary: Relapse-free survival (RFS)

End point title	Relapse-free survival (RFS)

End point description:

Time from start of treatment to the date of first documented hematologic relapse or death due to any cause, whichever occurs first.

End point type	Secondary

End point timeframe:

From start of treatment to up to 36 months from last patient first treatment.

End point values	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adults	Sabatolimab 800mg + Azacitidine adults	Sabatolimab 800mg mono adolescent
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	11	2	1
Units: Months				
median (confidence interval 95%)	2.56 (0.95 to 999)	6.74 (0.95 to 999)	999 (999 to 999)	7.16 (0 to 999)

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Adverse events and deaths are described from the start of treatment up to 30 days after the last dose of treatment for a max. approx. 25 months

Adverse event reporting additional description:

An Adverse Event is any sign or symptom occurring during a trial and safety follow-up. Death data covers all enrolled patients, while Adverse Event data was reported from those receiving at least one study drug dose.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	27.0

Reporting groups

Reporting group title	Sabatolimab 400mg mono adults

Reporting group description:

Safety run-in cohort: Adult participants in this arm received sabatolimab 400mg only.

Reporting group title	Sabatolimab 800mg mono adolescent

Reporting group description:

Adolescent safety cohort: ≥12 to < 18-year-old adolescent participants in this arm received sabatolimab 800mg only.

Reporting group title	Sabatolimab 800mg + Azacitidine adults

Reporting group description:

Expansion cohort: Adult participants in this arm received sabatolimab 800 mg + azacitidine combination.

Reporting group title	Sabatolimab 800mg mono adults

Reporting group description:

Safety run-in cohort: Adult participants in this arm received sabatolimab 800mg only.

Serious adverse events	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adolescent	Sabatolimab 800mg + Azacitidine adults
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 10 (20.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
number of deaths (all causes)	1	0	0
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Femoral neck fracture			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (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			
Myocarditis			

subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (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
Nervous system disorders			
Nervous system disorder			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (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
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (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
Infections and infestations			
Pneumonia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (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
Bronchitis			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (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
Febrile infection			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (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
Oral herpes			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (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	Sabatolimab 800mg mono adults		
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 11 (27.27%)		
number of deaths (all causes)	0		

number of deaths resulting from adverse events	0		
Injury, poisoning and procedural complications			
Femoral neck fracture			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Myocarditis			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Nervous system disorder			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Pneumonia			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Bronchitis			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Febrile infection			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Oral herpes			

subjects affected / exposed	1 / 11 (9.09%)		
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	Sabatolimab 400mg mono adults	Sabatolimab 800mg mono adolescent	Sabatolimab 800mg + Azacitidine adults
Total subjects affected by non-serious adverse events			
subjects affected / exposed	9 / 10 (90.00%)	1 / 1 (100.00%)	2 / 2 (100.00%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Basal cell carcinoma			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Vascular disorders			
Hypertensive crisis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Influenza like illness			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Oedema peripheral			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Pyrexia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Asthenia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	2
Chest pain			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Fatigue			

subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Injection site pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Mucosal inflammation			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Non-cardiac chest pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Oedema			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Immune system disorders			
Chronic graft versus host disease			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Lung disorder			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Dyspnoea exertional			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Investigations			
Blood cholesterol increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Blood alkaline phosphatase increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Aspartate aminotransferase increased			

subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Amylase increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Alanine aminotransferase increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
C-reactive protein increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Brain natriuretic peptide increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Blood potassium increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Blood creatinine increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Lipase increased			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
White blood cell count increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Troponin T increased			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			

Fall			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Immunisation reaction			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Anaemia postoperative			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Neuropathy peripheral			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Paraesthesia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Somnolence			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Neutropenia			
subjects affected / exposed	2 / 10 (20.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	2	0	0
Thrombocytopenia			
subjects affected / exposed	3 / 10 (30.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	3	0	0
Disseminated intravascular coagulation			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0

Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	2 / 2 (100.00%)
occurrences (all)	1	0	2
Stomatitis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Pruritus			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Intertrigo			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Telangiectasia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Musculoskeletal and connective tissue disorders			
Pain in extremity			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Bone pain			

subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Arthralgia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Myalgia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Infections and infestations			
Influenza			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
COVID-19			
subjects affected / exposed	2 / 10 (20.00%)	1 / 1 (100.00%)	1 / 2 (50.00%)
occurrences (all)	2	1	1
Viral infection			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Pneumonia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Otitis media			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Folliculitis			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Febrile infection			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Clostridium difficile colitis			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Bronchitis			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	2	0	0

Rhinitis			
subjects affected / exposed	0 / 10 (0.00%)	1 / 1 (100.00%)	1 / 2 (50.00%)
occurrences (all)	0	1	1
Herpes zoster			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Metapneumovirus infection			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Oral bacterial infection			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Oral candidiasis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Hyperkalaemia			
subjects affected / exposed	0 / 10 (0.00%)	1 / 1 (100.00%)	0 / 2 (0.00%)
occurrences (all)	0	1	0
Hypertriglyceridaemia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Cachexia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Hyperferritinaemia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Tumour lysis syndrome			
subjects affected / exposed	1 / 10 (10.00%)	0 / 1 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0

Non-serious adverse events	Sabatolimab 800mg mono adults		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	9 / 11 (81.82%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			

Basal cell carcinoma			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Vascular disorders			
Hypertensive crisis			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
General disorders and administration site conditions			
Influenza like illness			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Oedema peripheral			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Pyrexia			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	2		
Asthenia			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Chest pain			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Fatigue			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Injection site pain			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Mucosal inflammation			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Non-cardiac chest pain			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Oedema			

subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Immune system disorders			
Chronic graft versus host disease			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	2 / 11 (18.18%)		
occurrences (all)	2		
Lung disorder			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Dyspnoea exertional			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Investigations			
Blood cholesterol increased			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Blood alkaline phosphatase increased			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Aspartate aminotransferase increased			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Amylase increased			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Alanine aminotransferase increased			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
C-reactive protein increased			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Gamma-glutamyltransferase			

increased subjects affected / exposed occurrences (all) Brain natriuretic peptide increased subjects affected / exposed occurrences (all) Blood potassium increased subjects affected / exposed occurrences (all) Blood creatinine increased subjects affected / exposed occurrences (all) Lipase increased subjects affected / exposed occurrences (all) White blood cell count increased subjects affected / exposed occurrences (all) Troponin T increased subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1 0 / 11 (0.00%) 0 0 / 11 (0.00%) 0 1 / 11 (9.09%) 1 0 / 11 (0.00%) 0 0 / 11 (0.00%) 0 1 / 11 (9.09%) 1		
Injury, poisoning and procedural complications Fall subjects affected / exposed occurrences (all) Immunisation reaction subjects affected / exposed occurrences (all) Anaemia postoperative subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1 0 / 11 (0.00%) 0 0 / 11 (0.00%) 0		
Cardiac disorders Tachycardia subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Nervous system disorders			

Neuropathy peripheral subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Paraesthesia subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Somnolence subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Blood and lymphatic system disorders			
Anaemia subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Neutropenia subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Thrombocytopenia subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Disseminated intravascular coagulation subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Gastrointestinal disorders			
Diarrhoea subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 2		
Vomiting subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Nausea subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Stomatitis subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Skin and subcutaneous tissue disorders			

Rash subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Pruritus subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Intertrigo subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Telangiectasia subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Renal and urinary disorders Dysuria subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Musculoskeletal and connective tissue disorders Pain in extremity subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Bone pain subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Arthralgia subjects affected / exposed occurrences (all)	0 / 11 (0.00%) 0		
Myalgia subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
Infections and infestations Influenza subjects affected / exposed occurrences (all)	1 / 11 (9.09%) 1		
COVID-19 subjects affected / exposed occurrences (all)	2 / 11 (18.18%) 2		

Viral infection				
subjects affected / exposed	1 / 11 (9.09%)			
occurrences (all)	1			
Pneumonia				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Otitis media				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Folliculitis				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Febrile infection				
subjects affected / exposed	1 / 11 (9.09%)			
occurrences (all)	1			
Clostridium difficile colitis				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Bronchitis				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Rhinitis				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Herpes zoster				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Metapneumovirus infection				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Oral bacterial infection				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			
Oral candidiasis				
subjects affected / exposed	0 / 11 (0.00%)			
occurrences (all)	0			

Metabolism and nutrition disorders			
Hyperkalaemia			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Hypertriglyceridaemia			
subjects affected / exposed	1 / 11 (9.09%)		
occurrences (all)	1		
Cachexia			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Hyperferritinaemia			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		
Tumour lysis syndrome			
subjects affected / exposed	0 / 11 (0.00%)		
occurrences (all)	0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
16 April 2021	Amendment #1: - In response to a request from health authorities, the list of inclusion and exclusion criteria was revised. The modification includes the addition of required minimum hemoglobin level, as well as restrictions for prior cancer-directed treatment or investigational modalities and for patient with BCR-ABL mutations eligible for post-transplant tyrosine kinase inhibitor therapies; - Clarification was provided on the dose used in cohorts 3-5, to define DLTs and dose modifications handling for sabatolimab by the request of health authorities; - Revisions were made to the visit schedule assessments tables to add/correct the visit windows. additionally, amendment was implemented to differentiate the collection of antineoplastic therapies/medications since discontinuation which was previously included in collection concomitant medication and therapies; - Changes included removal of immunogenicity (IG) sample collection after sabatolimab post-infusion at Cycle 24, as pre-infusion collected sample at this visit was sufficient.
24 March 2022	Amendment #2: - The restrictive list of inclusion and exclusion criteria was modified per feedback provided by the Principal Investigators to increase the recruitment rate. These changes did not substantially impact the study design or the primary objectives, which included safety and preliminary efficacy assessments. An amended protocol was implemented to include the determination of eligibility for enrollment based on central MRD assay. These modifications allowed for a more comprehensive assessment of patient eligibility during the course of the study.

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 for complete trial results.

Notes: