Clinical trial results:
A PHASE 3, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY TO COMPARE EFFICACY AND SAFETY OF ORAL AZACITIDINE PLUS BEST SUPPORTIVE CARE VERSUS BEST SUPPORTIVE CARE AS MAINTENANCE THERAPY IN SUBJECTS WITH ACUTE MYELOID LEUKEMIA IN COMPLETE REMISSION

Summary

EudraCT number	2012-003457-28
Trial protocol	BE CZ PT LT IT ES FI DE AT PL IE GB FR
Global end of trial date	18 June 2024

Results information

Result version number	v1 (current)
This version publication date	04 July 2025
First version publication date	04 July 2025

Trial information

Trial identification

Sponsor protocol code	CC-486-AML-001

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Bristol-Myers Squibb
Sponsor organisation address	Chaussée de la Hulpe 185, Brussels, Belgium, 1170
Public contact	Global Submission Management, Clinical Trials, Bristol-Myers Squibb International Corporation, mg-gsm-ct@bms.com
Scientific contact	Bristol-Myers Squibb Study Director, Bristol-Myers Squibb, mg-gsm-ct@bms.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	06 August 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	18 June 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the study is to evaluate whether maintenance therapy with oral azacitidine improves OS compared with placebo in subjects with AML, age ≥ 55 years, who have achieved first CR or CRi after induction with intensive chemotherapy with or without consolidation chemotherapy.

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and in compliance with all International Conference on Harmonization Good Clinical Practice Guidelines. All the local regulatory requirements pertinent to safety of trial subjects were followed.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	24 April 2013
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	Italy: 75
Country: Number of subjects enrolled	Spain: 38
Country: Number of subjects enrolled	United Kingdom: 35
Country: Number of subjects enrolled	Germany: 32
Country: Number of subjects enrolled	Russian Federation: 19
Country: Number of subjects enrolled	Türkiye: 18
Country: Number of subjects enrolled	Portugal: 15
Country: Number of subjects enrolled	Austria: 14
Country: Number of subjects enrolled	Poland: 14
Country: Number of subjects enrolled	France: 13
Country: Number of subjects enrolled	Belgium: 11
Country: Number of subjects enrolled	Czechia: 10
Country: Number of subjects enrolled	Finland: 9
Country: Number of subjects enrolled	Israel: 9
Country: Number of subjects enrolled	Ireland: 1
Country: Number of subjects enrolled	Lithuania: 1
Country: Number of subjects enrolled	United States: 60
Country: Number of subjects enrolled	Canada: 17
Country: Number of subjects enrolled	Mexico: 2

Country: Number of subjects enrolled	Australia: 49
Country: Number of subjects enrolled	Korea, Republic of: 15
Country: Number of subjects enrolled	Taiwan: 8
Country: Number of subjects enrolled	Brazil: 7
Worldwide total number of subjects	472
EEA total number of subjects	233

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)	134
From 65 to 84 years	337
85 years and over	1

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

472 Participants Randomized, 469 participants treated

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Are arms mutually exclusive?	Yes
Arm title	Oral Azacitidine Plus Best Supportive Care

Arm description:

Participants received 300 mg azacitidine tablets once a day (QD) for the first 14 days of each 28-day treatment cycle until discontinuation, which includes the following reasons: disease relapse, withdrawal of consent, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, death, lost to follow-up, or protocol violation or until the end of the study.

Arm type	Experimental
Investigational medicinal product name	azacitidine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

150 and/or 200mg

Arm title	Placebo Plus Best Supportive Care

Arm description:

Participants received identically matching placebo tablets QD for the first 14 days of each 28-day treatment cycle until no longer receiving benefit, withdrawal of consent, disease relapse, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, lost to follow-up, or protocol violation or until the end of the study.

Arm type	Placebo
Investigational medicinal product name	placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

placebo

Number of subjects in period 1	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care
Started	238	234
Received Treatment	236	233
Completed	0	0
Not completed	238	234
Adverse event, serious fatal	2	2
Disease Relapse	151	181
Withdrew Consent	19	13
Physician decision	7	-
Other Reasons	28	27
Adverse event, non-fatal	31	11

Baseline characteristics

Reporting groups	
Reporting group title	Oral Azacitidine Plus Best Supportive Care
Reporting group description:	
Participants received 300 mg azacitidine tablets once a day (QD) for the first 14 days of each 28-day treatment cycle until discontinuation, which includes the following reasons: disease relapse, withdrawal of consent, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, death, lost to follow-up, or protocol violation or until the end of the study.	
Reporting group title	Placebo Plus Best Supportive Care
Reporting group description:	
Participants received identically matching placebo tablets QD for the first 14 days of each 28-day treatment cycle until no longer receiving benefit, withdrawal of consent, disease relapse, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, lost to follow-up, or protocol violation or until the end of the study.	

Reporting group values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care	Total
Number of subjects	238	234	472
Age Categorical Units: Participants			
18 to 64 Years	66	68	134
65 to 84 Years	171	166	337
≥ 85 years	1	0	1
Age Continuous Units: Years			
arithmetic mean	67.9	68.0	
standard deviation	± 5.72	± 5.62	-
Sex: Female, Male Units: Participants			
Female	120	107	227
Male	118	127	245
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	20	14	34
Not Hispanic or Latino	196	202	398
Unknown or Not Reported	22	18	40
Race/Ethnicity, Customized Units: Subjects			
White	216	197	413
Black or African-American	2	6	8
Asian	6	20	26
Other	12	11	23
Missing	2	0	2
Initial Acute Myeloid Leukemia (AML) Classification			
AML is classified using the WHO classification system based upon a combination of morphology, immunophenotype, genetics, and clinical features. There are several broad groups and include: 1. AML with genetic abnormalities; 2. AML with multilineage dysplasia 3. AML related to previous chemotherapy or radiation 4. Unspecified AML - do not fall into the above groups			
Units: Subjects			

AML with Recurrent Genetic Abnormalities	39	46	85
AML with Myelodysplasia - Related Changes	49	42	91
Therapy-related Myeloid Neoplasms	2	0	2
AML not Otherwise Specified	148	145	293
Missing	0	1	1
Type of Acute Myeloid Leukemia (AML)			
Primary AML is a cancer that originates in the blood and bone marrow. AML affects a group of white blood cells called myeloid cells, which normally develop into the various types of mature blood cells, such as red blood cells, white blood cells and platelets. Secondary acute myeloid leukemia (s-AML) refers to a leukemic process: (1) evolving from prior myelodysplasia (MDS), myeloproliferative disorder (MPN), or aplastic anemia (AA) with or without treatment or (2) as a product of previous exposure to a proven leukemogenic chemotherapeutic agent (therapy-related AML [t-AML]).			
Units: Subjects			
Primary (de novo)	213	216	429
Secondary	25	18	43
Cytogenetic Risk Category at Diagnosis			
Cytogenetic Risk - Intermediate -I is of a normal karyotype; Poor Risk - includes complex karyotypes having 3 or more cytogenetic abnormalities.			
Units: Subjects			
Intermediate	203	203	406
Poor	35	31	66
Eastern Cooperative Oncology Group (ECOG) Performance Status			
ECOG performance status is used to describe a patient's level of functioning in terms of their ability to care for themselves, daily activity, and physical ability (walking, working, etc.). The scale ranges from 0 to 5: 0 = Fully active, no restrictions; 1 = Restricted activity but ambulatory, able to carry out work of a light nature; 2 = Ambulatory and capable of all self-care but unable to carry out work activities; 3 = Capable to only limited self-care, confined to bed or chair more than 50% of waking hours; 4 = Completely disabled, no self-care, confined to bed or chair; 5 = Dead.			
Units: Subjects			
Grade 0	116	111	227
Grade 1	101	106	207
Grade 2	21	15	36
Grade 3	0	2	2

End points

End points reporting groups

Reporting group title	Oral Azacitidine Plus Best Supportive Care
Reporting group description:	
Participants received 300 mg azacitidine tablets once a day (QD) for the first 14 days of each 28-day treatment cycle until discontinuation, which includes the following reasons: disease relapse, withdrawal of consent, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, death, lost to follow-up, or protocol violation or until the end of the study.	
Reporting group title	Placebo Plus Best Supportive Care
Reporting group description:	
Participants received identically matching placebo tablets QD for the first 14 days of each 28-day treatment cycle until no longer receiving benefit, withdrawal of consent, disease relapse, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, lost to follow-up, or protocol violation or until the end of the study.	

Primary: Kaplan-Meier (K-M) Estimate for Overall Survival (OS)

End point title	Kaplan-Meier (K-M) Estimate for Overall Survival (OS)
End point description:	
Overall survival was defined as time from randomization to death from any cause; participants surviving at the end of the follow-up period, or who withdraw consent, or who were lost to follow up were censored at the date last known alive.	
End point type	Primary
End point timeframe:	
Day 1 (randomization) up to data cut off date of 15 July 2019; median follow-up for OS estimated by the reverse K-M method was 41.2 months for all participants.	

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	238	234		
Units: Months				
median (confidence interval 95%)	24.7 (18.7 to 30.5)	14.8 (11.7 to 17.6)		

Statistical analyses

Statistical analysis title	Statistical Analysis for OS
Comparison groups	Oral Azacitidine Plus Best Supportive Care v Placebo Plus Best Supportive Care

Number of subjects included in analysis	472
Analysis specification	Pre-specified
Analysis type	
P-value	= 0.0009 [1]
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.69
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.55
upper limit	0.86

Notes:

[1] - The p-value is 2-sided from a log-rank test stratified by age, cytogenetic risk category, and received consolidation therapy or not.

Secondary: Kaplan-Meier Estimate of Relapse Free Survival (RFS)

End point title	Kaplan-Meier Estimate of Relapse Free Survival (RFS)

End point description:

RFS was defined as the time from the date of randomization to the date of documented relapse or death from any cause, whichever occurred first. Participants who were still alive without documented relapse, or who were lost to follow-up or withdrew consent without documented relapse, were censored at the date of their last bone marrow assessment, prior to receiving any other therapy for AML. Documented relapse was defined as the earliest date of the following: • ≥ 5% bone marrow blasts (myeloblasts) from Central Pathology report, or • appearance of > 0% blasts in the peripheral blood with a later bone marrow confirmation (bone marrow blast [myeloblasts] ≥ 5%) within 100 days, or • at least 2 peripheral blasts ≥ 5% within 30 days.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	238	234		
Units: Months				
median (confidence interval 95%)	10.2 (7.9 to 12.9)	4.8 (4.6 to 6.4)		

Statistical analyses

Statistical analysis title	Statistical Analysis for RFS
Comparison groups	Oral Azacitidine Plus Best Supportive Care v Placebo Plus Best Supportive Care

Number of subjects included in analysis	472
Analysis specification	Pre-specified
Analysis type	
P-value	< 0.0001 [2]
Method	Logrank
Parameter estimate	Hazard ratio (HR)
Point estimate	0.65
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.52
upper limit	0.8

Notes:

[2] - The p-value is 2-sided from a log-rank test stratified by age, cytogenetic risk category, and received consolidation therapy or not.

Secondary: Kaplan-Meier Estimate of Time to Relapse

End point title	Kaplan-Meier Estimate of Time to Relapse

End point description:

Time to relapse was defined as the interval (in months) from the date of randomization to the date of documented relapse. Estimates of relapse rate were based on the cumulative incidence function from a competing risk analysis with death as a competing risk for relapse from complete remission (CR)/ complete remission with incomplete blood count recovery (CRi). Documented relapse was defined as, the earliest date of the following: • ≥ 5% bone marrow blasts (myeloblasts) from Central Pathology report, or • appearance of > 0% blasts in the peripheral blood with a later bone marrow confirmation (bone marrow blast [myeloblasts] ≥ 5%) within 100 days, or • at least 2 peripheral blasts ≥ 5% within 30 days.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	238	234		
Units: months				
median (confidence interval 95%)	10.2 (8.3 to 13.4)	4.9 (4.6 to 6.4)		

Statistical analyses

No statistical analyses for this end point

Secondary: Kaplan-Meier Estimates of Time to Discontinuation from Treatment

End point title	Kaplan-Meier Estimates of Time to Discontinuation from Treatment

End point description:

Time to discontinuation from treatment was assessed and defined as the interval from the date of

randomization to the date of discontinuation from study drug. Participants who were receiving treatment at the time of study closure were censored at the date of last visit. Estimates of relapse rate were based on the cumulative incidence function from a competing risk analysis with death as a competing risk for relapse from CR/ CRi.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	238	234		
Units: months				
median (confidence interval 95%)	14.6 (11.3 to 20.1)	6.9 (5.3 to 7.9)		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants with Treatment Emergent Adverse Events (TEAEs)

End point title	Number of Participants with Treatment Emergent Adverse Events (TEAEs)

End point description:

TEAEs include AEs that started between first dose date and 28 days after the last dose of study drug. A serious adverse event (SAE) is: • Death • Life-threatening event • Inpatient hospitalization or prolongation of existing hospitalization • Persistent or significant disability or incapacity • Congenital anomaly or birth defect • Other important medical event The severity of AEs were assessed by the investigator according to the Common Terminology Criteria for Adverse Events (CTCAE) Version 4.0: Grade 1 (Mild): asymptomatic/mild symptoms; clinical or diagnostic observations only; intervention not indicated. Grade 2 (Moderate): minimal, local or noninvasive intervention indicated; limiting age-appropriate activities of daily living. Grade 3: Severe or medically significant but not immediately life-threatening; hospitalization or prolongation of hospitalization indicated; disabling; limiting self care Grade 4: Life-threatening; urgent intervention indicated. Grade 5: Death due to AE.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	236	233		
Units: Participants				
≥ 1 TEAE	235	233		

≥ 1 TEAE Related to Study Treatment	213	121		
≥ 1 Serious TEAE	110	109		
≥ 1 Treatment Related Serious TEAE	22	5		
≥ 1 Grade 3/4 TEAE	207	201		
≥ 1 Treatment Related Grade 3/4 TEAE	113	55		
≥ 1 TEAE Leading to Death	15	11		
≥ 1 TEAE Leading to Dose Reduction (Red)	37	6		
≥ 1 TEAE Leading to Dose Interruption	107	43		
≥ 1 TEAE Leading to Dose Red and Interruption	25	3		
≥ 1 TEAE Leading to Study Drug Discontinuation	155	170		

Statistical analyses

No statistical analyses for this end point

Secondary: Mean Change in the Functional Assessment of Chronic Illness Therapy (FACIT-Fatigue Scale V 4.0) Score from Baseline

End point title	Mean Change in the Functional Assessment of Chronic Illness Therapy (FACIT-Fatigue Scale V 4.0) Score from Baseline

End point description:

The functional assessment of chronic illness therapy (FACIT-Fatigue Scale V 4.0) is a subscale of the FACIT-F and has been validated in the oncology setting. The FACIT-Fatigue Scale is a short, 13-item, self-administered tool that measures the level of fatigue in an individual during usually daily activities over the past week. The level of fatigue is measured on a 5-point Likert scale (0 = not at all; 4 = very much. The scores range from 0 to 52, with higher scores indicating less fatigue. If there were missing items, but the participant answered at least 50% of the items, then subscores were prorated.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	165	150		
Units: units on a scale				
arithmetic mean (standard deviation)	-3.7 (± 10.92)	-2.5 (± 9.93)		

Statistical analyses

No statistical analyses for this end point

Secondary: Mean Change in the European Quality of Life-Five Dimensions-Three Levels (EQ-5D-3L) Score from Baseline

End point title	Mean Change in the European Quality of Life-Five Dimensions-Three Levels (EQ-5D-3L) Score from Baseline

End point description:

The EQ-5D-3L is a self-administered questionnaire consisting of 5 questions, pertaining to specific health dimensions (ie, mobility, self-care, pain, usual activities, and anxiety/depression) and a health status scale. Each question has 3 levels of severity, corresponding to no problems, moderate problems and severe problems. Canadian population sample weights were used to derive health utility scores. A higher utility score represents a better health state. A clinically meaningful improvement or worsening was defined as at least 0.08 points of improvement or 0.10 points of worsening from baseline, respectively, for the EQ-5D-3L Health Utility Index. The instrument is scored using the United Kingdom (UK) index ranges from -0.594 to 1, where 0 equates to death and 1 equates to full health; -0.594 is considered 'worse than death'.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	164	150		
Units: Units on a scale				
arithmetic mean (standard deviation)	-0.0416 (± 0.15467)	-0.0152 (± 0.14799)		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Definitive Clinically Meaningful Deterioration for ≥ 2 Consecutive Visits as Measured Using the EQ-5D HRQoL Scale

End point title	Time to Definitive Clinically Meaningful Deterioration for ≥ 2 Consecutive Visits as Measured Using the EQ-5D HRQoL Scale

End point description:

Clinically meaningful deterioration is defined as a decrease of at least 0.10 points from baseline for at least 2 consecutive visits on the EQ-5D Health Utility Index. The EQ-5D-3L is a self-administered questionnaire with 5 questions covering health dimensions like mobility, self-care, pain, usual activities, and anxiety/depression, plus a health status scale. Each question has 3 severity levels: no problems, moderate problems, and severe problems. Canadian population sample weights derive health utility scores, where higher scores indicate better health. Clinically meaningful improvement or worsening is defined as at least 0.08 points of improvement or 0.10 points of worsening from baseline. The EQ-5D-3L uses the UK index, ranging from -0.594 to 1, where 0 equates to death and 1 to full health; -0.594 is considered 'worse than death'.

here "99999" means NA

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 15 July 2019; approximately 74 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	225	217		
Units: Weeks				
median (confidence interval 95%)	99999 (135.1 to 99999)	99999 (122.9 to 99999)		

Statistical analyses

Statistical analysis title	Statistical Analysis for EQ-5D
Comparison groups	Oral Azacitidine Plus Best Supportive Care v Placebo Plus Best Supportive Care
Number of subjects included in analysis	442
Analysis specification	Pre-specified
Analysis type	
P-value	= 0.7522 [3]
Method	Regression, Cox
Parameter estimate	Hazard ratio (HR)
Point estimate	0.9345
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.6136
upper limit	1.4231

Notes:

[3] - Stratification factors: • Age (at induction therapy): 55 to 64 years and ≥ 65 years • Prior history of MDS: yes/no • Cytogenetic risk (at induction therapy): intermediate-risk/poor-risk • Received consolidation therapy following induction: yes/no

Secondary: Healthcare Resource Utilization (HRU): Rate of Hospital Events Per Person Year

End point title	Healthcare Resource Utilization (HRU): Rate of Hospital Events Per Person Year

End point description:

HRU is defined as any consumption of healthcare resources directly or indirectly related to the treatment of the patient. HRU is a key component to understand treatment costs and budget impact of new treatments from a provider perspective.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	236	233		
Units: Hospitalizations per person-years				
number (confidence interval 95%)	0.36 (0.31 to 0.42)	0.63 (0.54 to 0.74)		

Statistical analyses

No statistical analyses for this end point

Secondary: Healthcare Resource Utilization (HRU): Number of Days Hospitalized Per Person-Year

End point title	Healthcare Resource Utilization (HRU): Number of Days Hospitalized Per Person-Year

End point description:

HRU is defined as any consumption of healthcare resources directly or indirectly related to the treatment of the patient. HRU is a key component to understand treatment costs and budget impact of new treatments from a provider perspective.

End point type	Secondary

End point timeframe:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

End point values	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	236	233		
Units: Days per person-years				
number (confidence interval 95%)	6.00 (5.78 to 6.22)	13.13 (12.68 to 13.60)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From day 1 (randomization) up to data cut off date of 06 August 2024; approximately 135.5 months

Adverse event reporting additional description:

The number at Risk for All-Cause Mortality represents all Randomized Participants. The number at Risk for Serious Adverse Events and Other (Not Including Serious) Adverse Events represents all participants that received at least 1 dose of study medication

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	27

Reporting groups

Reporting group title	Oral Azacitidine Plus Best Supportive Care

Reporting group description:

Participants received 300 mg azacitidine tablets once a day (QD) for the first 14 days of each 28-day treatment cycle until discontinuation, which includes the following reasons: disease relapse, withdrawal of consent, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, death, lost to follow-up, or protocol violation or until the end of the study.

Reporting group title	Placebo Plus Best Supportive Care

Reporting group description:

Participants received identically matching placebo tablets QD for the first 14 days of each 28-day treatment cycle until no longer receiving benefit, withdrawal of consent, disease relapse, adverse events, participant became eligible for allogeneic bone marrow or stem cell transplantation during the treatment period, lost to follow-up, or protocol violation or until the end of the study.

Serious adverse events	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care	
Total subjects affected by serious adverse events			
subjects affected / exposed	110 / 236 (46.61%)	109 / 233 (46.78%)	
number of deaths (all causes)	171	175	
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Central nervous system leukaemia			
subjects affected / exposed	2 / 236 (0.85%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 3	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Basal cell carcinoma			
subjects affected / exposed	4 / 236 (1.69%)	3 / 233 (1.29%)	
occurrences causally related to treatment / all	0 / 5	0 / 3	
deaths causally related to treatment / all	0 / 0	0 / 0	

Acute myeloid leukaemia recurrent			
subjects affected / exposed	37 / 236 (15.68%)	58 / 233 (24.89%)	
occurrences causally related to treatment / all	0 / 37	0 / 58	
deaths causally related to treatment / all	0 / 3	0 / 3	
Acute myeloid leukaemia			
subjects affected / exposed	2 / 236 (0.85%)	5 / 233 (2.15%)	
occurrences causally related to treatment / all	0 / 2	0 / 5	
deaths causally related to treatment / all	0 / 1	0 / 4	
Cholangiocarcinoma			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Chloroma			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Endometrial cancer			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gliomatosis cerebri			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Prostate cancer			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Malignant melanoma			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Meningioma			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Metastases to meninges			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (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	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Squamous cell carcinoma			
subjects affected / exposed	0 / 236 (0.00%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Prostatic adenoma			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Squamous cell carcinoma of lung			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Squamous cell carcinoma of skin			
subjects affected / exposed	2 / 236 (0.85%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 3	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Vascular disorders			
Deep vein thrombosis			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypertension			

subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypotension			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Peripheral artery stenosis			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Thrombophlebitis			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Fatigue			
subjects affected / exposed	2 / 236 (0.85%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	1 / 2	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Multiple organ dysfunction syndrome			
subjects affected / exposed	1 / 236 (0.42%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	0 / 1	0 / 2	
deaths causally related to treatment / all	0 / 1	0 / 2	
General physical health deterioration			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 1	
Pyrexia			

subjects affected / exposed	6 / 236 (2.54%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 6	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Immune system disorders			
Allergy to vaccine			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Asthma			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dyspnoea			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Epistaxis			
subjects affected / exposed	0 / 236 (0.00%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonitis			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pulmonary embolism			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pulmonary haemorrhage			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Respiratory failure			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Psychiatric disorders			
Completed suicide			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 1	0 / 0	
Confusional state			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Investigations			
C-reactive protein increased			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Alanine aminotransferase increased			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
General physical condition abnormal			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Troponin increased			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

Weight decreased			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Injury, poisoning and procedural complications			
Hip fracture			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Foot fracture			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Femoral neck fracture			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cataract traumatic			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Chemical peritonitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Fall			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Traumatic intracranial haemorrhage			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Subdural haematoma			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Spinal fracture			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Post-traumatic pain			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Congenital, familial and genetic disorders			
Hydrocele			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Acute myocardial infarction			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Angina pectoris			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Angina unstable			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Aortic valve disease			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Atrial fibrillation			
subjects affected / exposed	3 / 236 (1.27%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 3	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac failure congestive			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 2	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiogenic shock			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 1	0 / 0	
Coronary artery disease			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Stress cardiomyopathy			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Cerebral ischaemia			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cerebral infarction			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cerebral haemorrhage			
subjects affected / exposed	2 / 236 (0.85%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 2	0 / 1	
deaths causally related to treatment / all	0 / 2	0 / 1	
Central nervous system inflammation			

subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Haemorrhage intracranial			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 1	0 / 0	
Syncope			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Transient ischaemic attack			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ischaemic cerebral infarction			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	2 / 236 (0.85%)	3 / 233 (1.29%)	
occurrences causally related to treatment / all	1 / 2	1 / 3	
deaths causally related to treatment / all	0 / 0	0 / 0	
Disseminated intravascular coagulation			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Febrile neutropenia			
subjects affected / exposed	16 / 236 (6.78%)	9 / 233 (3.86%)	
occurrences causally related to treatment / all	5 / 16	2 / 9	
deaths causally related to treatment / all	0 / 0	0 / 0	
Lymphadenitis			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Neutropenia			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	2 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Thrombocytopenia			
subjects affected / exposed	2 / 236 (0.85%)	3 / 233 (1.29%)	
occurrences causally related to treatment / all	0 / 2	0 / 3	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pancytopenia			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Thrombocytosis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Eye disorders			
Iridocyclitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Keratitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ulcerative keratitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			

Abdominal pain			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Colitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Crohn's disease			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Constipation			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Diarrhoea			
subjects affected / exposed	3 / 236 (1.27%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 4	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dysphagia			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastritis			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	2 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis eosinophilic			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ileus paralytic			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (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 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Lower gastrointestinal haemorrhage			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Inguinal hernia			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Melaena			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nausea			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 2	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Neutropenic colitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Small intestinal haemorrhage			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pancreatitis acute			

subjects affected / exposed	1 / 236 (0.42%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	1 / 1	1 / 3	
deaths causally related to treatment / all	0 / 0	0 / 0	
Small intestinal obstruction			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Upper gastrointestinal haemorrhage			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Vomiting			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hepatobiliary disorders			
Cholecystitis			
subjects affected / exposed	3 / 236 (1.27%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	0 / 3	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cholecystitis acute			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cholelithiasis			
subjects affected / exposed	0 / 236 (0.00%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	0 / 0	0 / 3	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cholecystitis chronic			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hyperbilirubinaemia			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Skin and subcutaneous tissue disorders			
Acute febrile neutrophilic dermatosis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ureterolithiasis			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	3 / 236 (1.27%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 3	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bone pain			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Intervertebral disc protrusion			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Joint effusion			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

Osteoporotic fracture			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Osteoarthritis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Myalgia			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Spinal pain			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Atypical pneumonia			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bacteraemia			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bacterial infection			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
COVID-19			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bacterial sepsis			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cellulitis			
subjects affected / exposed	4 / 236 (1.69%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	1 / 4	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cholecystitis infective			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Clostridium difficile infection			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cystitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Device related infection			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Device related sepsis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Diverticulitis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Endophthalmitis			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (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 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis salmonella			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis Escherichia coli			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal infection			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Herpes zoster			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	1 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Influenza			
subjects affected / exposed	3 / 236 (1.27%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 3	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Klebsiella sepsis			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 1	0 / 0	
Lung abscess			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonia aspiration			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 1	0 / 0	
Pneumonia			
subjects affected / exposed	13 / 236 (5.51%)	7 / 233 (3.00%)	
occurrences causally related to treatment / all	8 / 14	0 / 8	
deaths causally related to treatment / all	0 / 0	0 / 0	
Osteomyelitis			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Neutropenic sepsis			
subjects affected / exposed	2 / 236 (0.85%)	2 / 233 (0.86%)	
occurrences causally related to treatment / all	1 / 2	0 / 2	
deaths causally related to treatment / all	0 / 1	0 / 0	
Pneumonia fungal			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pseudomonas infection			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Rectal abscess			

subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory tract infection			
subjects affected / exposed	0 / 236 (0.00%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Sepsis			
subjects affected / exposed	5 / 236 (2.12%)	4 / 233 (1.72%)	
occurrences causally related to treatment / all	2 / 5	0 / 5	
deaths causally related to treatment / all	0 / 2	0 / 0	
Septic shock			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 1	0 / 0	
Staphylococcal infection			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Staphylococcal sepsis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Superinfection bacterial			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Wound infection			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urinary tract infection bacterial			

subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urosepsis			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urinary tract infection			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dehydration			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 2	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hyperkalaemia			
subjects affected / exposed	2 / 236 (0.85%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypocalcaemia			
subjects affected / exposed	1 / 236 (0.42%)	0 / 233 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypokalaemia			
subjects affected / exposed	1 / 236 (0.42%)	1 / 233 (0.43%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	Oral Azacitidine Plus Best Supportive Care	Placebo Plus Best Supportive Care	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	235 / 236 (99.58%)	224 / 233 (96.14%)	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Acute myeloid leukaemia recurrent			
subjects affected / exposed	115 / 236 (48.73%)	126 / 233 (54.08%)	
occurrences (all)	117	128	
Vascular disorders			
Hypertension			
subjects affected / exposed	19 / 236 (8.05%)	16 / 233 (6.87%)	
occurrences (all)	21	18	
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	44 / 236 (18.64%)	12 / 233 (5.15%)	
occurrences (all)	60	13	
Oedema peripheral			
subjects affected / exposed	22 / 236 (9.32%)	24 / 233 (10.30%)	
occurrences (all)	26	33	
Fatigue			
subjects affected / exposed	71 / 236 (30.08%)	44 / 233 (18.88%)	
occurrences (all)	104	56	
Influenza like illness			
subjects affected / exposed	12 / 236 (5.08%)	7 / 233 (3.00%)	
occurrences (all)	19	10	
Pyrexia			
subjects affected / exposed	37 / 236 (15.68%)	43 / 233 (18.45%)	
occurrences (all)	48	62	
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	30 / 236 (12.71%)	39 / 233 (16.74%)	
occurrences (all)	47	47	
Dyspnoea			

subjects affected / exposed	13 / 236 (5.51%)	14 / 233 (6.01%)	
occurrences (all)	16	20	
Epistaxis			
subjects affected / exposed	16 / 236 (6.78%)	17 / 233 (7.30%)	
occurrences (all)	20	21	
Oropharyngeal pain			
subjects affected / exposed	15 / 236 (6.36%)	19 / 233 (8.15%)	
occurrences (all)	19	23	
Psychiatric disorders			
Anxiety			
subjects affected / exposed	17 / 236 (7.20%)	8 / 233 (3.43%)	
occurrences (all)	17	10	
Insomnia			
subjects affected / exposed	22 / 236 (9.32%)	23 / 233 (9.87%)	
occurrences (all)	26	25	
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	12 / 236 (5.08%)	4 / 233 (1.72%)	
occurrences (all)	15	7	
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	14 / 236 (5.93%)	4 / 233 (1.72%)	
occurrences (all)	16	4	
Nervous system disorders			
Headache			
subjects affected / exposed	25 / 236 (10.59%)	26 / 233 (11.16%)	
occurrences (all)	36	34	
Dizziness			
subjects affected / exposed	25 / 236 (10.59%)	21 / 233 (9.01%)	
occurrences (all)	32	22	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	51 / 236 (21.61%)	42 / 233 (18.03%)	
occurrences (all)	77	59	
Febrile neutropenia			

subjects affected / exposed	14 / 236 (5.93%)	10 / 233 (4.29%)	
occurrences (all)	16	12	
Thrombocytopenia			
subjects affected / exposed	81 / 236 (34.32%)	62 / 233 (26.61%)	
occurrences (all)	185	73	
Neutropenia			
subjects affected / exposed	104 / 236 (44.07%)	61 / 233 (26.18%)	
occurrences (all)	420	87	
Leukopenia			
subjects affected / exposed	27 / 236 (11.44%)	19 / 233 (8.15%)	
occurrences (all)	62	28	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	32 / 236 (13.56%)	15 / 233 (6.44%)	
occurrences (all)	51	18	
Constipation			
subjects affected / exposed	92 / 236 (38.98%)	56 / 233 (24.03%)	
occurrences (all)	181	75	
Abdominal pain upper			
subjects affected / exposed	23 / 236 (9.75%)	13 / 233 (5.58%)	
occurrences (all)	31	15	
Flatulence			
subjects affected / exposed	12 / 236 (5.08%)	4 / 233 (1.72%)	
occurrences (all)	13	6	
Haemorrhoids			
subjects affected / exposed	12 / 236 (5.08%)	4 / 233 (1.72%)	
occurrences (all)	17	4	
Dyspepsia			
subjects affected / exposed	12 / 236 (5.08%)	5 / 233 (2.15%)	
occurrences (all)	12	7	
Diarrhoea			
subjects affected / exposed	119 / 236 (50.42%)	50 / 233 (21.46%)	
occurrences (all)	465	79	
Nausea			
subjects affected / exposed	153 / 236 (64.83%)	54 / 233 (23.18%)	
occurrences (all)	368	78	

Stomatitis			
subjects affected / exposed	8 / 236 (3.39%)	12 / 233 (5.15%)	
occurrences (all)	9	14	
Vomiting			
subjects affected / exposed	142 / 236 (60.17%)	23 / 233 (9.87%)	
occurrences (all)	308	25	
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	16 / 236 (6.78%)	18 / 233 (7.73%)	
occurrences (all)	21	18	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	27 / 236 (11.44%)	24 / 233 (10.30%)	
occurrences (all)	39	35	
Arthralgia			
subjects affected / exposed	45 / 236 (19.07%)	32 / 233 (13.73%)	
occurrences (all)	55	44	
Pain in extremity			
subjects affected / exposed	28 / 236 (11.86%)	12 / 233 (5.15%)	
occurrences (all)	32	13	
Infections and infestations			
Influenza			
subjects affected / exposed	17 / 236 (7.20%)	7 / 233 (3.00%)	
occurrences (all)	19	10	
Nasopharyngitis			
subjects affected / exposed	20 / 236 (8.47%)	16 / 233 (6.87%)	
occurrences (all)	36	20	
Bronchitis			
subjects affected / exposed	14 / 236 (5.93%)	9 / 233 (3.86%)	
occurrences (all)	18	10	
Upper respiratory tract infection			
subjects affected / exposed	32 / 236 (13.56%)	32 / 233 (13.73%)	
occurrences (all)	49	59	
Rhinitis			
subjects affected / exposed	13 / 236 (5.51%)	4 / 233 (1.72%)	
occurrences (all)	18	5	

Oral herpes			
subjects affected / exposed	13 / 236 (5.51%)	6 / 233 (2.58%)	
occurrences (all)	15	10	
Urinary tract infection			
subjects affected / exposed	18 / 236 (7.63%)	13 / 233 (5.58%)	
occurrences (all)	28	16	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	31 / 236 (13.14%)	15 / 233 (6.44%)	
occurrences (all)	42	16	
Hypokalaemia			
subjects affected / exposed	21 / 236 (8.90%)	20 / 233 (8.58%)	
occurrences (all)	34	22	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 November 2018	The primary purpose of this protocol amendment is to add an extension phase (EP) to allow all subjects who are on treatment with oral azacitidine and demonstrating clinical benefit in this protocol to continue to do so in an extension phase. In addition, all subjects who were discontinued from the treatment phase (irrespective of randomization arm) and continuing in the Follow-up Phase, will be followed for survival for at least another 12 months, until death, withdrawal of consent, study closure or until the subject is lost to follow-up.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported