



Clinical trial results:

A Phase 3 Study to Evaluate the Safety and Efficacy of OMS721 for the Treatment of Atypical Hemolytic Uremic Syndrome (aHUS) in Adults and Adolescents.

Summary

EudraCT number	2017-002057-11
Trial protocol	LT PL
Global end of trial date	27 November 2023

Results information

Result version number	v1 (current)
This version publication date	28 June 2026
First version publication date	28 June 2026

Trial information

Trial identification

Sponsor protocol code	OMS721-HUS-002
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03205995
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Omeros Corporation
Sponsor organisation address	201 Elliott Avenue West, Seattle, United States, 98119
Public contact	Steve Whitaker, Omeros Corporation, 206 676-5000,
Scientific contact	Steve Whitaker, Omeros Corporation, 206 676-5000,

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	02 February 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	02 February 2021
Global end of trial reached?	Yes
Global end of trial date	27 November 2023
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study is to evaluate the effect of OMS721 in subjects with aHUS on:

- Platelet count change from baseline

Protection of trial subjects:

N/A

Background therapy:

N/A

Evidence for comparator:

N/A

Actual start date of recruitment	06 September 2017
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	Poland: 3
Country: Number of subjects enrolled	Lithuania: 3
Worldwide total number of subjects	6
EEA total number of subjects	6

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)	6
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study was conducted globally at different sites between 02 May 2018 and 27 Nov 2023.

Pre-assignment

Screening details:

Overall, 6 subjects were enrolled and randomized into this uncontrolled, open-label study.

Period 1

Period 1 title	Treatment Induction Period (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Blinding implementation details:

N/A

Arms

Arm title	Narsoplimab
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Arm description:

Patients received narsoplimab 370 mg IV on Days 1 and 4. Beginning on the day of the first dose (Day 1), patients also began treatment with narsoplimab 150 mg subcutaneously (SC) once daily. If a patient received plasma therapy during the Treatment Induction Period, the patient received supplemental narsoplimab 185 mg IV. If the patient received plasma therapy on Day 1 or Day 4 of the Treatment Induction Period, the regularly scheduled narsoplimab dose was administered within 1 hour after plasma exchange or within 1 hour before plasma infusion.

Arm type	Experimental
Investigational medicinal product name	Narsoplimab
Investigational medicinal product code	
Other name	OMS721
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

Patients received narsoplimab 370 mg IV on Days 1 and 4. Beginning on the day of the first dose (Day 1), patients also began treatment with narsoplimab 150 mg subcutaneously (SC) once daily. If a patient received plasma therapy during the Treatment Induction Period, the patient received supplemental narsoplimab 185 mg IV. If the patient received plasma therapy on Day 1 or Day 4 of the Treatment Induction Period, the regularly scheduled narsoplimab dose was administered within 1 hour after plasma exchange or within 1 hour before plasma infusion.

Number of subjects in period 1	Narsoplimab
Started	6
Completed	0
Not completed	6
Physician decision	2
Adverse event, non-fatal	1
Death	1
Protocol deviation	2

Baseline characteristics

Reporting groups

Reporting group title	Treatment Induction Period
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Reporting group description: -

Reporting group values	Treatment Induction Period	Total	
Number of subjects	6	6	
Age categorical			
Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	6	6	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical			
Units: Subjects			
Female	5	5	
Male	1	1	
Ethnicity			
Units: Subjects			
Hispanic or Latino	0	0	
Not Hispanic or Latino	6	6	
Not Reported/Unknown	0	0	
Race			
Units: Subjects			
American Indian or Alaska Native	0	0	
Asian	0	0	
Black or African American	0	0	
Native Hawaiian or Other Pacific Islander	0	0	
White	6	6	
Other	0	0	
Child Bearing Potential,			
Units: Subjects			
Yes	5	5	
No	0	0	
Not Applicable	1	1	
Baseline Weight			
Units: kg			
arithmetic mean	65.8		
standard deviation	± 13.78	-	
Baseline BMI			

Units: kg/m ²) arithmetic mean standard deviation	23.3 ± 4.66	-	
Baseline systolic blood pressure Units: mmHg arithmetic mean standard deviation	148 ± 13.8	-	
Baseline diastolic blood pressure Units: mmHg arithmetic mean standard deviation	87.5 ± 10.15	-	

End points

End points reporting groups

Reporting group title	Narsoplimab
Reporting group description:	
Patients received narsoplimab 370 mg IV on Days 1 and 4. Beginning on the day of the first dose (Day 1), patients also began treatment with narsoplimab 150 mg subcutaneously (SC) once daily. If a patient received plasma therapy during the Treatment Induction Period, the patient received supplemental narsoplimab 185 mg IV. If the patient received plasma therapy on Day 1 or Day 4 of the Treatment Induction Period, the regularly scheduled narsoplimab dose was administered within 1 hour after plasma exchange or within 1 hour before plasma infusion.	

Primary: Platelet Count (10⁹ Platelets/L) Change From Baseline at Week 26

End point title	Platelet Count (10 ⁹ Platelets/L) Change From Baseline at Week 26 ^[1]
End point description:	
The primary outcome to be measured is platelet count change from baseline.	
End point type	Primary
End point timeframe:	
Week 26	
Notes:	
[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.	
Justification: As the endpoint is descriptive in nature, no statistical analysis is provided.	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: 10 ⁹ platelets/L				
median (full range (min-max))	42 (-21 to 115.5)			

Statistical analyses

No statistical analyses for this end point

Secondary: Safety as Measured by Incidences of Adverse Events, Vital Signs, ECG, and Clinical Laboratory Tests

End point title	Safety as Measured by Incidences of Adverse Events, Vital Signs, ECG, and Clinical Laboratory Tests
End point description:	
Assessment of safety of OMS721 (narsoplimab) in participants with aHUS by incidence of Adverse Events, clinically significant vital sign abnormalities, ECG abnormalities, and clinical laboratory test abnormalities	
End point type	Secondary
End point timeframe:	
Pre-dose and up to 771 days post-dose	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Participants	6			

Statistical analyses

No statistical analyses for this end point

Secondary: Thrombotic Microangiopathies (TMA) Response

End point title	Thrombotic Microangiopathies (TMA) Response
End point description:	
Complete TMA response defined as normalization of platelet count, normalization of serum lactate dehydrogenase (LDH), and > 25% decrease in serum creatinine by at least 2 consecutive measures over at least 4 consecutive weeks, within the initial 26-week period	
End point type	Secondary
End point timeframe:	
26 weeks	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Participants	0			

Statistical analyses

No statistical analyses for this end point

Secondary: TMA Event-free Status

End point title	TMA Event-free Status
End point description:	
No decrease in platelet count of > 25% from baseline, no plasma exchange or plasma infusion, and no initiation of new dialysis over at least 12 consecutive weeks, within the initial 26-week period	
End point type	Secondary
End point timeframe:	
26 weeks	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Count of Participants	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Increase in Estimated Glomerular Filtration Rate (eGFR)

End point title	Increase in Estimated Glomerular Filtration Rate (eGFR)
End point description: Increase of greater than 15 ml/min/1.73 m2 in eGFR calculated by the modification of diet in renal disease (MDRD) Equation	
End point type	Secondary
End point timeframe: 26 weeks	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Count of Participants	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Hematological Normalization

End point title	Hematological Normalization
End point description: Normalization of platelet count and normalization of serum LDH by 2 consecutive measurements over at least 4 weeks, within the initial 26-week period	
End point type	Secondary
End point timeframe: 26 weeks	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Count of Participants	1			

Statistical analyses

No statistical analyses for this end point

Secondary: TMA Remission

End point title	TMA Remission
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End point description:

Platelet count greater than or equal to 150,000/ μ L on at least 2 consecutive measures over at least 2 consecutive weeks, within the initial 26-week period

End point type	Secondary
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End point timeframe:

26 weeks

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[2]			
Units: Count of Participants	2			

Notes:

[2] - Any patient who received drug

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of Antidrug Antibodies (ADA)

End point title	Incidence of Antidrug Antibodies (ADA)
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End point description:

Incidences of ADA in participants with aHUS, administered OMS721 (narsoplimab)

End point type	Secondary
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End point timeframe:

771 days post-dose

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[3]			
Units: Count of Participants	1			

Notes:

[3] - Any patient who received drug

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Serum Creatinine (mg/dL)

End point title	Change From Baseline in Serum Creatinine (mg/dL)
End point description:	Assessment of subject's change from baseline in serum creatinine.
End point type	Secondary
End point timeframe:	26 weeks

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[4]			
Units: mg/dL				
median (full range (min-max))	1.53 (-0.98 to 3.16)			

Notes:

[4] - Any patient who received drug

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Serum LDH (U/L)

End point title	Change From Baseline in Serum LDH (U/L)
End point description:	Assessment of subject's change from baseline in serum LDH
End point type	Secondary
End point timeframe:	26 weeks

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[5]			
Units: U/L				
median (full range (min-max))	-12 (-112 to 389)			

Notes:

[5] - Any patient who received drug

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Haptoglobin (mg/dL)

End point title	Change From Baseline in Haptoglobin (mg/dL)
End point description: Assessment of subject's change from baseline in haptoglobin	
End point type	Secondary
End point timeframe: 26 weeks	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[6]			
Units: mg/dL				
median (full range (min-max))	30 (4 to 56)			

Notes:

[6] - Any patient who received drug.

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics (PK): Trough Plasma Concentration, Lower Limit of Quantification (LLOQ)

End point title	Pharmacokinetics (PK): Trough Plasma Concentration, Lower Limit of Quantification (LLOQ)
End point description: Pharmacokinetics (PK): Trough plasma concentration, lower limit of quantification (LLOQ)	
End point type	Secondary
End point timeframe: Days 1-4; Treatment Maintenance (103 weeks): 17 visits; Rescue Therapy (RT) (if occurs): RT Days 1-4; Follow-Up at Day 771	

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: ng/mL				
geometric mean (standard deviation)	15477.2 (± 6471.62)			

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics (PK): Maximum Plasma Concentrations (Cmax)

End point title	Pharmacokinetics (PK): Maximum Plasma Concentrations (Cmax)
End point description:	Pharmacokinetics (PK): Maximum plasma concentrations (Cmax)
End point type	Secondary
End point timeframe:	Days 1-4; Treatment Maintenance (103 weeks): 17 visits; Rescue Therapy (if occurs): RT Days 1-4; Follow-Up at Day 771

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: ng/mL				
median (full range (min-max))	48350 (25100 to 86300)			

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacokinetics (PK): Area Under Time-concentration Curve (AUC)

End point title	Pharmacokinetics (PK): Area Under Time-concentration Curve (AUC)
End point description:	Pharmacokinetics (PK): Area under time-concentration curve (AUC) - Outcome measure data for this secondary outcome measure were not reported because no PK modeling could be performed due to insufficient data collection due to early study termination; therefore, there are no data or results to summarize.
End point type	Secondary
End point timeframe:	Days 1-4; Treatment Maintenance (103 weeks): 17 visits; Rescue Therapy (if occurs): RT Days 1-4; Follow-Up at Day 771

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[7]			
Units: ng/mL				
median (full range (min-max))	0 (0 to 0)			

Notes:

[7] - PK modeling for calculating the AUC was not performed because the study was not completed.

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacodynamics (PD): Inhibition of C3 Activity (%)

End point title	Pharmacodynamics (PD): Inhibition of C3 Activity (%)
End point description:	Pharmacodynamics (PD): Inhibition of C3 activity
End point type	Secondary
End point timeframe:	Days 1-4; Treatment Maintenance (103 weeks): 17 visits; Rescue Therapy (if occurs): RT Days 1-4; Follow-Up at Day 771

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6 ^[8]			
Units: Inhibition of C3 activity (%)				
median (full range (min-max))	84.4 (1.7 to 101.4)			

Notes:

[8] - Any patient who received drug and measurable C3 activity.

Statistical analyses

No statistical analyses for this end point

Secondary: Pharmacodynamics (PD): Inhibition of C4 Activity (%)

End point title	Pharmacodynamics (PD): Inhibition of C4 Activity (%)
End point description:	Pharmacodynamics (PD): Inhibition of C4 activity
End point type	Secondary
End point timeframe:	Days 1-4; Treatment Maintenance (103 weeks): 17 visits; Rescue Therapy (if occurs): RT Days 1-4; Follow-Up at Day 771

End point values	Narsoplimab			
Subject group type	Reporting group			
Number of subjects analysed	6			
Units: Inhibition of C4 activity (%)				
median (full range (min-max))	91.9 (11.9 to 99.9)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From the signing informed consent form to end of follow up

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	19
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Reporting groups

Reporting group title	Narsoplimab 370 mg IV + 150 mg SC
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Reporting group description:

Patients received narsoplimab 370 mg IV on Days 1 and 4. Beginning on the day of the first dose (Day 1), patients also began treatment with narsoplimab 150 mg subcutaneously (SC) once daily. If a patient received plasma therapy during the Treatment Induction Period, the patient received supplemental narsoplimab 185 mg IV. If the patient received plasma therapy on Day 1 or Day 4 of the Treatment Induction Period, the regularly scheduled narsoplimab dose was administered within 1 hour after plasma exchange or within 1 hour before plasma infusion

Serious adverse events	Narsoplimab 370 mg IV + 150 mg SC		
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 6 (83.33%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events	0		
Injury, poisoning and procedural complications			
Arteriovenous fistula aneurysm			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Shunt blood flow excessive			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			

Cardiac failure			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Cerebral haematoma			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Blood and lymphatic system disorders			
Haemolysis			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Leukopenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Thrombocytopenia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Immune system disorders			
Chronic allograft nephropathy			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Kidney transplant rejection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Hepatic failure			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory, thoracic and mediastinal disorders			
Pulmonary hypertension			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Skin and subcutaneous tissue disorders			
Purpura			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Chronic kidney disease			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Escherichia sepsis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Pneumonia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Renal graft infection			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Urinary tract infection			

subjects affected / exposed	2 / 6 (33.33%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		

Frequency threshold for reporting non-serious adverse events: 1 %

Non-serious adverse events	Narsoplimab 370 mg IV + 150 mg SC		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	6 / 6 (100.00%)		
Vascular disorders			
Alopecia			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Hypertension			
alternative assessment type: Non-systematic			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Purpura			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	3 / 6 (50.00%)		
occurrences (all)	3		
Injection site extravasation			
alternative assessment type: Non-systematic			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Injection site pain			
alternative assessment type: Non-systematic			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Injection site haematoma			
alternative assessment type: Non-systematic			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Oedema peripheral			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Immune system disorders			
Hepatic failure			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Reproductive system and breast disorders			
Chronic kidney disease			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Leukocyturia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Respiratory, thoracic and mediastinal disorders			
Amenorrhoea			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Psychiatric disorders			
Cerebral haematoma			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Epilepsy			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Headache			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Neurological symptom			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Investigations			
Electrocardiogram QT prolonged			
alternative assessment type: Non-systematic			

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Human chorionic gonadotropin abnormal			
alternative assessment type: Non-systematic			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Arteriovenous fistula aneurysm			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Post procedural haematoma			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Shunt blood flow excessive			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Subdural haematoma			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Injury, poisoning and procedural complications			
Bacterial infection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Clostridium difficile infection			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Escherichia sepsis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Molluscum contagiosum			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Pneumonia			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Ureaplasma infection			

subjects affected / exposed occurrences (all) Urinary tract infection enterococcal subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		
Congenital, familial and genetic disorders Hypertrophic cardiomyopathy subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Cardiac disorders Bradycardia subjects affected / exposed occurrences (all) Cardiac failure subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		
Nervous system disorders Back pain subjects affected / exposed occurrences (all) Pain in extremity subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		
Blood and lymphatic system disorders Haemolysis alternative assessment type: Non-systematic subjects affected / exposed occurrences (all) Iron deficiency anaemia subjects affected / exposed occurrences (all) Leukopenia subjects affected / exposed occurrences (all) Thrombocytopenia	2 / 6 (33.33%) 2 1 / 6 (16.67%) 1 1 / 6 (16.67%) 1		

subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Blood cholesterol increased			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Gastrointestinal disorders			
Ascites			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Diarrhoea			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Enterocolitis			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Gastrointestinal haemorrhage			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Nausea			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Oesophageal ulcer			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Pulmonary hypertension			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Renal and urinary disorders			
Haematuria			
subjects affected / exposed	2 / 6 (33.33%)		
occurrences (all)	2		
Aggression			
subjects affected / exposed	1 / 6 (16.67%)		
occurrences (all)	1		
Anxiety			

subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Insomnia subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Endocrine disorders Adrenal insufficiency subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Hyperthyroidism subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Hypocalcaemia subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Infections and infestations Arteriovenous fistula site infection subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Infection subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Renal graft infection subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Urinary tract infection subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Chronic allograft nephropathy subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Hypersensitivity			

subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Kidney transplant rejection subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Metabolism and nutrition disorders			
Hyperkalaemia subjects affected / exposed occurrences (all)	3 / 6 (50.00%) 3		
Hyperphosphataemia subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
Hyperuricaemia subjects affected / exposed occurrences (all)	2 / 6 (33.33%) 2		
C-reactive protein increased subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		
Treponema test positive subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
31 May 2017	Amendment #1: - Change to Sections 2, 6.2, 7.3.2. Rationale: To update the study's secondary objectives and secondary endpoints with these event times, which will be used in evaluating the efficacy of OMS721 in the treatment of aHUS subjects in the study. Added three secondary study objectives and secondary endpoints: * Time to TMA event-free status measured by the time to reach the first measurement of TMA event-free status from the first OMS721 dose * Time to eGFR increase of > 15 ml/min/1.73 m ² measured by the time to reach the first measurement increase in eGFR of > 15 ml/min/1.73 m ² from the first OMS721 dose * Time to hematological normalization measured by the time to reach the first measurement of hematological normalization from the first OMS721 dose

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported