



Clinical trial results:

Prospective, Single-Arm, Multicenter Study to Evaluate the Efficacy, Safety, Pharmacokinetics, Pharmacodynamics, and Immunogenicity of Eculizumab in Complement Inhibitor Treatment-Naïve Pediatric and Adult Participants with Atypical Hemolytic Uremic Syndrome (aHUS) in Chin

Summary

EudraCT number	2025-000162-29
Trial protocol	Outside EU/EEA
Global end of trial date	07 May 2025

Results information

Result version number	v1 (current)
This version publication date	22 November 2025
First version publication date	22 November 2025

Trial information

Trial identification

Sponsor protocol code	ECU-aHUS-302
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Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Alexion Pharmaceuticals Inc.
Sponsor organisation address	121 Seaport Boulevard, Boston, MA, United States, 02210
Public contact	Alexion Pharmaceuticals Inc., European Clinical Trial Information, +35 3874162507, clinicaltrials.eu@alexion.com
Scientific contact	European Clinical Trial Information, Alexion Pharmaceuticals Inc., +35 +35 3874162507, clinicaltrials.eu@alexion.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	07 May 2025
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	07 May 2025
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of the trial was to assess the efficacy of eculizumab in the treatment of participants with aHUS.

Protection of trial subjects:

This study was conducted in accordance with the protocol and consensus ethical principles derived from international guidelines including the Declaration of Helsinki, Council for International Organizations of Medical Sciences (CIOMS) International Ethical Guidelines, applicable International Council for Harmonisation (ICH) Good Clinical Practice (GCP) Guidelines, and other applicable laws and regulations.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	14 July 2023
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	China: 25
Worldwide total number of subjects	25
EEA total number of subjects	0

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)	2
Children (2-11 years)	7
Adolescents (12-17 years)	2
Adults (18-64 years)	14
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

After providing informed consent/assent, participants were screened for eligibility for the study during the 7-day Screening Period.

Period 1

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

Arms

Arm title	Eculizumab
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Arm description:

Participants received eculizumab at a dose and schedule according to body weight for 26 weeks.

Arm type	Experimental
Investigational medicinal product name	Eculizumab
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administered as an intravenous (IV) infusion.

Number of subjects in period 1	Eculizumab
Started	25
Received at least 1 dose of study drug	25
Completed	22
Not completed	3
Study specific discontinuation criteria	1
Other than specified	2

Baseline characteristics

Reporting groups

Reporting group title	Eculizumab
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Reporting group description:

Participants received eculizumab at a dose and schedule according to body weight for 26 weeks.

Reporting group values	Eculizumab	Total	
Number of subjects	25	25	
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)	2	2	
Children (2-11 years)	7	7	
Adolescents (12-17 years)	2	2	
Adults (18-64 years)	14	14	
From 65-84 years	0	0	
85 years and over	0	0	
Age Continuous			
Units: years			
arithmetic mean	23.40		
standard deviation	± 15.95	-	
Gender Categorical			
Units: Subjects			
Female	6	6	
Male	19	19	
Race			
Units: Subjects			
Black or African American	0	0	
Asian	25	25	
White	0	0	
Other	0	0	
Ethnicity			
Units: Subjects			
Hispanic or Latino	0	0	
Not Hispanic or Latino	24	24	
Missing	1	1	

End points

End points reporting groups

Reporting group title	Eculizumab
Reporting group description:	
Participants received eculizumab at a dose and schedule according to body weight for 26 weeks.	

Primary: Percentage of Participants with a Complete Thrombotic Microangiopathy (TMA) Response

End point title	Percentage of Participants with a Complete Thrombotic Microangiopathy (TMA) Response ^[1]
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End point description:

The criteria for complete TMA response were:

1. Normalization of platelet count (defined as platelet count ≥ 150000 /microliter (ul).
2. Normalization of lactate dehydrogenase (LDH, defined as LDH $\leq$ upper limit of normal [ULN]).
3. $\geq 25\%$ improvement in serum creatinine from baseline.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose.

End point type	Primary
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End point timeframe:

Up to 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: No comparative statistical analyses were planned.

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: percentage of participants				
number (confidence interval 95%)	64.0 (42.5 to 82.0)			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants with an Adverse Event (AE)

End point title	Number of Participants with an Adverse Event (AE)
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End point description:

An AE was defined as any untoward medical occurrence in a participant or clinical investigation participant administered a pharmaceutical product and which did not necessarily have a causal relationship with this treatment.

A serious AE (SAE) was defined as any untoward medical occurrence that, at any dose:

- resulted in death,
- was life-threatening,
- required inpatient hospitalization or prolongation of existing hospitalization,
- resulted in persistent disability/incapacity,
- was a congenital anomaly/birth defect, or

- was an important medical event.

A summary of all Serious Adverse Events and Other Adverse Events (nonserious) regardless of causality is located in the 'Reported Adverse Events' Section.

Safety Set: Included all participants who received at least 1 dose of study intervention.

End point type	Secondary
End point timeframe:	
Up to Week 34	

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: participants				
Any AE	24			
Any SAE	8			

Statistical analyses

No statistical analyses for this end point

Secondary: Mean Serum Concentration of Eculizumab

End point title	Mean Serum Concentration of Eculizumab
End point description:	
Pharmacokinetic (PK) Analysis Set: Included all participants who received at least 1 dose of study intervention and had evaluable pharmacokinetic data. N = the number of participants evaluable at the specific timepoint.	
End point type	Secondary
End point timeframe:	
Pre-dose and post-dose at Days 1, 8, 29, 85 and 141; Pre-dose at Day 183	

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: micrograms per milliliter (ug/mL)				
geometric mean (geometric coefficient of variation)				
Day 1: Pre-dose (N=25)	4.690 (± 0.00)			
Day 1: Post-dose (N=25)	373.423 (± 47.59)			
Day 8: Pre-dose (N=24)	153.190 (± 69.69)			
Day 8: Post-dose (N=24)	498.262 (± 41.18)			
Day 29: Pre-dose (N=21)	353.726 (± 36.96)			

Day 29: Post-dose (N=21)	727.862 (± 32.22)			
Day 85: Pre-dose (N=21)	360.070 (± 43.89)			
Day 85: Post-dose (N=21)	728.624 (± 42.04)			
Day 141: Pre-dose (N=21)	433.737 (± 41.32)			
Day 141: Post-dose (N=21)	883.675 (± 38.74)			
Day 183: Pre-dose (N=21)	434.739 (± 38.66)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Serum Free Complement 5 (C5)

End point title	Change from Baseline in Serum Free Complement 5 (C5)
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End point description:

Pharmacodynamic (PD) Analysis Set: Included all participants who received at least 1 dose of study intervention and had evaluable PD data. N = the number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline (Day 1 pre-dose) to Days 1, 8, 29, 85 and 141 (pre-dose and post-dose) and pre-dose at Day 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: ug/mL				
arithmetic mean (standard deviation)				
Day 1: Post-dose (N=25)	-79.8842 (± 17.5197)			
Day 8: Pre-dose (N=24)	-79.6539 (± 17.8589)			
Day 8: Post-dose (N=24)	-79.6636 (± 17.8610)			
Day 29: Pre-dose (N=21)	-80.4277 (± 17.1241)			
Day 29: Post-dose (N=21)	-80.4284 (± 17.1241)			
Day 85: Pre-dose (N=21)	-80.0632 (± 17.3489)			
Day 85: Post-dose (N=21)	-80.0632 (± 17.3489)			
Day 141: Pre-dose (N=21)	-80.0625 (± 17.3485)			
Day 141: Post-dose (N=21)	-80.0632 (± 17.3489)			

Day 183: Pre-dose (N=21)	-80.0632 ($\pm$ 17.3489)			
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Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Serum Total C5

End point title	Change from Baseline in Serum Total C5
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End point description:

PD Analysis Set: Included all participants who received at least 1 dose of study intervention and had evaluable PD data. N = the number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline (Day 1 pre-dose) to Days 1, 8, 29, 85 and 141 (pre-dose and post-dose) and pre-dose at Day 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: ug/mL				
arithmetic mean (standard deviation)				
Day 1: Post-dose (N=25)	-12.0267 ($\pm$ 6.1396)			
Day 8: Pre-dose (N=24)	37.9293 ($\pm$ 21.3162)			
Day 8: Post-dose (N=24)	40.2831 ($\pm$ 23.0175)			
Day 29: Pre-dose (N=21)	65.9100 ($\pm$ 25.0734)			
Day 29: Post-dose (N=21)	64.4783 ($\pm$ 22.7695)			
Day 85: Pre-dose (N=21)	71.2418 ($\pm$ 25.7350)			
Day 85: Post-dose (N=21)	66.5267 ($\pm$ 26.7232)			
Day 141: Pre-dose (N=21)	71.8284 ($\pm$ 25.3689)			
Day 141: Post-dose (N=21)	67.0693 ($\pm$ 23.1231)			
Day 183: Pre-dose (N=21)	78.5497 ($\pm$ 27.1158)			

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants with an Anti-drug Antibody (ADA) Response

End point title	Number of Participants with an Anti-drug Antibody (ADA) Response
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End point description:

An ADA response was defined as a positive ADA sample at any time during the study.

Safety Set: Included all participants who received at least 1 dose of study intervention.

End point type	Secondary
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End point timeframe:

Up to Week 26

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Complete TMA Response

End point title	Time to Complete TMA Response
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End point description:

Time to complete TMA response was defined as the time from first infusion to the first time point at which all criteria for complete TMA response was met.

The criteria for complete TMA response were:

1. Normalization of platelet count (defined as platelet count $\geq 150000/\mu\text{L}$).
2. Normalization of LDH, defined as LDH $\leq \text{ULN}$.
3. $\geq 25\%$ improvement in serum creatinine from baseline.

Participants who did not have a response were censored at the date of last visit or study discontinuation at the time when the analysis was performed.

99999 = Upper limit was not reached due to limited number of events.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose.

End point type	Secondary
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End point timeframe:

Up to Week 26

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: days				
median (confidence interval 95%)	75.0 (22.0 to 99999)			

Statistical analyses

No statistical analyses for this end point

Secondary: Proportion of Participants On or Off Dialysis at Each Timepoint

End point title	Proportion of Participants On or Off Dialysis at Each Timepoint
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End point description:

Participants were considered as 'off' dialysis at a specific time point if they were dialysis free for more than 5 days prior to that time point. Participants were considered as 'on' dialysis at a specific time point if they were dialysis free to 5 days or less up prior to that time point. N = number of participants evaluable at the specific timepoint.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose.

End point type	Secondary
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End point timeframe:

Baseline and Days 22, 43, 71, 99, 113, 127, 155 and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: proportion of participants				
number (confidence interval 95%)				
Baseline: On Dialysis (N=25)	0.440 (0.244 to 0.651)			
Baseline: Off Dialysis (N=25)	0.560 (0.349 to 0.756)			
Day 22: On Dialysis (N=23)	0.261 (0.102 to 0.484)			
Day 22: Off Dialysis (N=23)	0.739 (0.516 to 0.898)			
Day 43: On Dialysis (N=17)	0.235 (0.068 to 0.499)			
Day 43: Off Dialysis (N=17)	0.765 (0.501 to 0.932)			
Day 71: On Dialysis (N=19)	0.211 (0.061 to 0.456)			
Day 71: Off Dialysis (N=19)	0.789 (0.544 to 0.939)			
Day 99: On Dialysis (N=17)	0.176 (0.038 to 0.434)			
Day 99: Off Dialysis (N=17)	0.824 (0.566 to 0.962)			

Day 113: On Dialysis (N=18)	0.167 (0.036 to 0.414)			
Day 113: Off Dialysis (N=18)	0.833 (0.586 to 0.964)			
Day 127: On Dialysis (N=18)	0.111 (0.014 to 0.347)			
Day 127: Off Dialysis (N=18)	0.889 (0.653 to 0.986)			
Day 155: On Dialysis (N=18)	0.167 (0.036 to 0.414)			
Day 155: Off Dialysis (N=18)	0.833 (0.586 to 0.964)			
Day 183: On Dialysis (N=18)	0.167 (0.036 to 0.414)			
Day 183: Off Dialysis (N=18)	0.833 (0.586 to 0.964)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Estimated Glomerular Filtration Rate (eGFR) at Each Scheduled Visit

End point title	Change from Baseline in Estimated Glomerular Filtration Rate (eGFR) at Each Scheduled Visit
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End point description:

Expressed in milliliter per minute per 1.73 square meters of body surface area.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose. N = number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline, Days 22, 43, 71, 99, 113, 127, 155 and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: mL/min/1.73 ²				
arithmetic mean (standard deviation)				
Day 22 (N=22)	39.82 (± 50.83)			
Day 43 (N=16)	32.72 (± 44.75)			
Day 71 (N=18)	40.33 (± 47.38)			
Day 99 (N=16)	37.53 (± 47.17)			
Day 113 (N=17)	35.88 (± 45.38)			
Day 127 (N=17)	35.85 (± 48.01)			

Day 155 (N=16)	43.33 (± 49.32)			
Day 183 (N=17)	36.29 (± 44.21)			

Statistical analyses

No statistical analyses for this end point

Secondary: Proportion of Participants with a Chronic Kidney Disease (CKD) Stage Shift Categorized as "Improved", "Stable", or "Worsened" at each Scheduled Visit Compared to Baseline

End point title	Proportion of Participants with a Chronic Kidney Disease (CKD) Stage Shift Categorized as "Improved", "Stable", or "Worsened" at each Scheduled Visit Compared to Baseline
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End point description:

CKD stage was classified based on the National Kidney Foundation Chronic Kidney Disease Stage where Stage 5 represents the most severe disease and Stage 1 represents the least severe disease.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose. N = the number of participants evaluable at the specific timepoint. "Improved" excluded participants with Stage 1 at baseline as there was no room for improvement. "Worsened" excludes participants with Stage 5 at baseline as there was no room to worsen.

End point type	Secondary
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End point timeframe:

Baseline to Days 22, 43, 71, 99, 113, 127, 155 and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: proportion of participants				
number (confidence interval 95%)				
Day 22: Improved (N=22)	0.545 (0.322 to 0.756)			
Day 22: Stable (N=22)	0.455 (0.244 to 0.678)			
Day 22: Worsened (N=7)	0 (0 to 0.410)			
Day 43: Improved (N=16)	0.688 (0.413 to 0.890)			
Day 43: Stable (N=16)	0.313 (0.110 to 0.587)			
Day 43: Worsened (N=4)	0 (0 to 0.602)			
Day 71: Improved (N=18)	0.722 (0.465 to 0.903)			
Day 71: Stable (N=18)	0.278 (0.097 to 0.535)			
Day 71: Worsened (N=5)	0 (0 to 0.522)			
Day 99: Improved (N=16)	0.750 (0.476 to 0.927)			
Day 99: Stable (N=16)	0.250 (0.073 to 0.524)			

Day 99: Worsened (N=5)	0 (0 to 0.522)			
Day 113: Improved (N=17)	0.765 (0.501 to 0.932)			
Day 113: Stable (N=17)	0.235 (0.068 to 0.499)			
Day 113: Worsened (N=6)	0 (0 to 0.459)			
Day 127: Improved (N=17)	0.765 (0.501 to 0.932)			
Day 127: Stable (N=17)	0.235 (0.068 to 0.499)			
Day 127: Worsened (N=6)	0 (0 to 0.459)			
Day 155: Improved (N=16)	0.875 (0.617 to 0.984)			
Day 155: Stable (N=16)	0.125 (0.016 to 0.383)			
Day 155: Worsened (N=5)	0 (0 to 0.522)			
Day 183: Improved (N=17)	0.824 (0.566 to 0.962)			
Day 183: Stable (N=17)	0.176 (0.038 to 0.434)			
Day 183: Worsened (N=5)	0 (0 to 0.522)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Platelets

End point title	Change from Baseline in Platelets
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End point description:

Platelet values obtained from the day of a blood transfusion of platelets through 3 days after the transfusion are excluded from all analysis.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose. N = the number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline, Days 22, 43, 71, 99, 113, 127, 155, and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: 10 ⁹ /liter (L)				
arithmetic mean (standard deviation)				
Day 22 (N=23)	76.5 (± 117.7)			
Day 43 (N=16)	65.5 (± 90.1)			
Day 71 (N=19)	71.3 (± 97.9)			
Day 99 (N=16)	79.4 (± 68.1)			
Day 113 (N=16)	60.1 (± 82.7)			
Day 127 (N=17)	61.8 (± 86.1)			

Day 155 (N=18)	76.8 (± 98.2)			
Day 183 (N=18)	80.3 (± 98.0)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in LDH

End point title	Change from Baseline in LDH
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End point description:

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose. N = the number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline, Days 22, 43, 71, 99, 113, 127, 155, and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: microkatal per liter (ukat/L)				
arithmetic mean (standard deviation)				
Day 22 (N=23)	-9.445 (± 12.459)			
Day 43 (N=17)	-6.147 (± 10.175)			
Day 71 (N=19)	-7.083 (± 10.099)			
Day 99 (N=17)	-5.861 (± 8.356)			
Day 113 (N=18)	-5.769 (± 8.041)			
Day 127 (N=18)	-5.444 (± 8.314)			
Day 155 (N=17)	-5.848 (± 8.478)			
Day 183 (N=18)	-5.680 (± 8.172)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Hemoglobin

End point title	Change from Baseline in Hemoglobin
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End point description:

Hemoglobin values obtained from the day of a blood transfusion of either whole blood or packed red blood cells through 7 days after the transfusion are excluded from all analysis.

Full Analysis Set: Included all participants who received at least 1 dose of study intervention and had at least 1 efficacy assessment post first dose. N = number of participants evaluable at the specific timepoint.

End point type	Secondary
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End point timeframe:

Baseline, Days 22, 43, 71, 99, 113, 127, 155, and 183

End point values	Eculizumab			
Subject group type	Reporting group			
Number of subjects analysed	25			
Units: grams per liter (g/L)				
arithmetic mean (standard deviation)				
Day 22 (N=23)	17.0 (± 18.3)			
Day 43 (N=16)	25.7 (± 20.1)			
Day 71 (N=19)	30.5 (± 22.6)			
Day 99 (N=17)	30.3 (± 19.3)			
Day 113 (N=18)	29.8 (± 17.8)			
Day 127 (N=18)	25.2 (± 16.4)			
Day 155 (N=18)	30.5 (± 14.9)			
Day 183 (N=18)	36.2 (± 15.8)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Up to Week 34

Adverse event reporting additional description:

Safety Set: Included all participants who received at least 1 dose of study intervention.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	27.1
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Reporting groups

Reporting group title	Eculizumab
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Reporting group description:

Participants received eculizumab at a dose and schedule according to body weight for 26 weeks.

Serious adverse events	Eculizumab		
Total subjects affected by serious adverse events			
subjects affected / exposed	8 / 25 (32.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Gastrointestinal disorders			
Retching			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Upper gastrointestinal haemorrhage			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Hepatic function abnormal			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Synovial cyst			

subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Bacterial infection			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Clostridium difficile infection			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Infection			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	2 / 25 (8.00%)		
occurrences causally related to treatment / all	1 / 2		
deaths causally related to treatment / all	0 / 0		
Rotavirus infection			
subjects affected / exposed	1 / 25 (4.00%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Eculizumab		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	24 / 25 (96.00%)		
Vascular disorders			
Hypertension			
subjects affected / exposed	2 / 25 (8.00%)		
occurrences (all)	2		
Blood and lymphatic system disorders			

Anaemia subjects affected / exposed occurrences (all)	2 / 25 (8.00%) 2		
Leukopenia subjects affected / exposed occurrences (all)	2 / 25 (8.00%) 2		
General disorders and administration site conditions Pyrexia subjects affected / exposed occurrences (all)	3 / 25 (12.00%) 3		
Gastrointestinal disorders Diarrhoea subjects affected / exposed occurrences (all) Vomiting subjects affected / exposed occurrences (all)	2 / 25 (8.00%) 2 5 / 25 (20.00%) 5		
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all)	2 / 25 (8.00%) 2		
Hepatobiliary disorders Hepatic function abnormal subjects affected / exposed occurrences (all)	4 / 25 (16.00%) 5		
Infections and infestations Bronchitis subjects affected / exposed occurrences (all) Infection subjects affected / exposed occurrences (all) Influenza subjects affected / exposed occurrences (all) Respiratory tract infection	2 / 25 (8.00%) 2 3 / 25 (12.00%) 4 2 / 25 (8.00%) 2		

subjects affected / exposed	4 / 25 (16.00%)		
occurrences (all)	5		
Upper respiratory tract infection			
subjects affected / exposed	5 / 25 (20.00%)		
occurrences (all)	8		
Metabolism and nutrition disorders			
Hypokalaemia			
subjects affected / exposed	2 / 25 (8.00%)		
occurrences (all)	2		
Electrolyte imbalance			
subjects affected / exposed	2 / 25 (8.00%)		
occurrences (all)	2		
Hyperkalaemia			
subjects affected / exposed	7 / 25 (28.00%)		
occurrences (all)	9		
Hyperphosphataemia			
subjects affected / exposed	3 / 25 (12.00%)		
occurrences (all)	3		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
20 June 2022	This amendment was initiated mainly to update exclusion criteria and pregnancy language.
26 April 2023	The primary reason for this amendment was to clarify activities to be followed when a change in weight causes a participant to move to a different weight cohort during the study period. Additionally, updates to estimand descriptions for endpoints were also made.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported