



Clinical trial results:

Early treatment of pediatric patients with typical hemolytic uremic syndrome with the anti-C5 monoclonal antibody eculizumab: a phase III prospective, randomized, placebo-controlled clinical trial

Summary

EudraCT number	2014-001169-28
Trial protocol	FR
Global end of trial date	26 June 2019

Results information

Result version number	v1 (current)
This version publication date	27 June 2026
First version publication date	27 June 2026
Summary attachment (see zip file)	Adverse event analysis report (Adverse event analysis report.pdf)

Trial information

Trial identification

Sponsor protocol code	13705201
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02205541
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Direction de la Recherche et de l'Innovation
Sponsor organisation address	2 Rue Charles Viguerie, Toulouse, France, 31059
Public contact	Caroline PEYROT, Centre Hospitalier Universitaire de Toulouse, DRI, Hôtel-Dieu, +33 561778486, peyrot.c@chu-toulouse.fr
Scientific contact	Arnaud GARNIER, Hôpital des Enfants - Centre Hospitalier Universitaire Purpan, +33 534558594, garnier.a@chu-toulouse.fr

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	21 April 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	26 June 2019
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the impact of early eculizumab (ECZ) treatment on the progression of acute kidney injury (IRA) in children with STEC-associated hemolytic uremic syndrome (SHU-STEC) in the context of a prospective, placebo-controlled therapeutic trial.

Protection of trial subjects:

The participants in the clinical trial were protected by a complete vaccination schedule including a tetravalent meningococcal vaccine, a meningococcal B vaccine, and oral antibiotic prophylaxis. The study was designed to limit eculizumab (ECZ) treatment to a short duration (maximum 5 weeks) in order to reduce the risks of side effects and infections. All the local regulatory requirements pertinent to safety of trial participants were followed.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	08 July 2015
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	France: 100
Worldwide total number of subjects	100
EEA total number of subjects	100

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)	100
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0

85 years and over	0
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Subject disposition

Recruitment

Recruitment details:

Recruitment planned over 38 months in 18 pediatric nephrology units across France. Estimated 200 hospitalizations of eligible patients during this period to reach 100 inclusions. Recruitment coordinated via the Société de Néphrologie Pédiatrique (SNP).

Pre-assignment

Screening details:

Screening in hospitalized pediatric patients with STEC-HUS and AKI. Exclusion of patients with severe extra-renal involvement. Written consent from both parents required. Approximately 200 patients screened to include 100. Vaccination and prophylaxis mandatory before treatment.

Pre-assignment period milestones

Number of subjects started	100
Number of subjects completed	100

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Eculizumab treatment arm
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	eculizumab
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate for solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Eculizumab IV infusion over 1 hour. Dosage based on weight: 300–1200 mg. Administered at D0, D7, D14, D21, D28 depending on patient weight. Pre-treatment includes mandatory meningococcal vaccination and oral antibiotic prophylaxis until 60 days post last dose.

Arm title	Placebo arm
Arm description: -	
Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate for solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Placebo: 5% glucose IV infusion over 1 hour, matching eculizumab schedule (D0, D7, D14, D21, D28) based on patient weight. Pre-treatment includes same vaccination and oral antibiotic prophylaxis as active arm, maintained for 60 days after last infusion.

Number of subjects in period 1	Eculizumab treatment arm	Placebo arm
Started	50	50
Completed	44	42
Not completed	6	8
Consent withdrawn by subject	1	1
Physician decision	1	-
Adverse event, non-fatal	3	2
Reason not specified	-	1
Protocol deviation	1	4

Baseline characteristics

Reporting groups

Reporting group title	Eculizumab treatment arm
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Reporting group description: -

Reporting group title	Placebo arm
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Reporting group description: -

Reporting group values	Eculizumab treatment arm	Placebo arm	Total
Number of subjects	50	50	100
Age categorical Units: Subjects			

Age continuous Units: years arithmetic mean standard deviation	3.89 ± 3	3.28 ± 2.32	-
Gender categorical Units: Subjects			
Female	31	23	54
Male	19	27	46

End points

End points reporting groups

Reporting group title	Eculizumab treatment arm
Reporting group description: -	
Reporting group title	Placebo arm
Reporting group description: -	

Primary: Efficacy analysis

End point title	Efficacy analysis
End point description: Occurrence of renal replacement therapy (hemodialysis, hemofiltration, or peritoneal dialysis) lasting 48 hours or more (binary yes/no criterion) after treatment initiation	
End point type	Primary
End point timeframe: From Visit 1 (inclusion visit) through Visit 6 (last treatment visit)	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: Number of participants with EER $\geq$ 48h				
Success	19	24		
Failure	31	26		

Statistical analyses

Statistical analysis title	Primary endpoint analysis
Comparison groups	Placebo arm v Eculizumab treatment arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.313
Method	Chi-squared
Parameter estimate	Odds ratio (OR)
Point estimate	1.506
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.679
upper limit	3.339

Secondary: Safety and tolerability profile of the treatment

End point title	Safety and tolerability profile of the treatment
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End point description:

End point type	Secondary
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End point timeframe:

Throughout the study , an average of 1 year

End point values	Ecuzumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50 ^[1]	50 ^[2]		
Units: Number of adverse events per group	297	253		

Notes:

[1] - The actual number of people who have received Ecuzumab treatment is 54

[2] - The actual number of people who have received Placebo is 46

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of acute kidney injury (creatinine)

End point title	Duration of acute kidney injury (creatinine)
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End point description:

The result is then described by estimating the coefficient β with a 95% confidence interval, corresponding to the average difference in creatinine level between the groups (ECZ group and Placebo group) between V2 and V7.

End point type	Secondary
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End point timeframe:

From V2 to V7

End point values	Ecuzumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: $\mu\text{mol/L}$				
least squares mean (confidence interval 95%)	9.72 (-9.65 to 29.09)	9.72 (-9.65 to 29.09)		

Statistical analyses

Statistical analysis title	SEP 2A analysis
Statistical analysis description: SEP : Secondary endpoint	
Comparison groups	Placebo arm v Eculizumab treatment arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.325
Method	Mixed models analysis

Secondary: Duration of acute kidney injury (creatinine clairance)

End point title	Duration of acute kidney injury (creatinine clairance)
End point description: The result is then described by estimating the coefficient β with a 95% confidence interval, corresponding to the average difference in creatinine clairance between the groups (ECZ group and Placebo group) between V2 and V7.	
End point type	Secondary
End point timeframe: From V2 to V7	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: (mL/min/1.73m2)				
least squares mean (confidence interval 95%)	-1.43 (-11.16 to 8.3)	-1.43 (-11.16 to 8.6)		

Statistical analyses

Statistical analysis title	SEP 2B analysis
Statistical analysis description: SEP : Secondary endpoint	
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.773
Method	Mixed models analysis

Secondary: Renal sequelae

End point title	Renal sequelae
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End point description:

Evaluation of renal sequelae through the number of children with renal sequelae (definition of renal sequelae based on blood pressure measurement, creatinine clearance, proteinuria and microalbuminuria as ProtU/creatU>0.02) 6 months and 12 months after the last injection of ECZ or placebo. Renal sequelae : defined by a ProtU/creatU ratio > 0.02 .

End point type	Secondary
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End point timeframe:

at V8 (6 months post treatment) & at V9 (12 months post treatment)

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50 ^[3]	50 ^[4]		
Units: participants with renal sequelae				
number (not applicable)				
12 months post treatment	20	29		
6 months post treatment	26	21		

Notes:

[3] - number of children with presence of renal sequelae

[4] - number of children with presence of renal sequelae

Statistical analyses

Statistical analysis title	SEP 3 analysis (6 months)
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Statistical analysis description:

SEP : Secondary endpoint

Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.394
Method	Chi-squared

Statistical analysis title	SEP 3 analysis (12 months)
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Statistical analysis description:

SEP : Secondary endpoint

Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.045
Method	Chi-squared

Secondary: Evolution of hematological abnormalities (LDH level)

End point title	Evolution of hematological abnormalities (LDH level)
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End point description:

Evaluation of the evolution of hematological abnormalities based on the evolution of the difference in LDH levels between the experimental group and the placebo group between visits V2 and V7

End point type Secondary

End point timeframe:

from V2 to V7

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: UI/l				
arithmetic mean (confidence interval 95%)				
LDH level at V2	3308.264 (2706.485 to 3910.043)	2537.549 (2120.574 to 2954.523)		
LDH level at V7	281.65 (203.641 to 359.66)	340.663 (276.341 to 404.985)		

Statistical analyses

No statistical analyses for this end point

Secondary: Evolution of hematological abnormalities (platelet count)

End point title Evolution of hematological abnormalities (platelet count)

End point description:

Evaluation of the evolution of hematological abnormalities based on the platelet count in each group at visits V3 and V4

End point type Secondary

End point timeframe:

At V3 and V4

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: G/L				
arithmetic mean (standard deviation)				
Platelet count at V3	278.71 ($\pm$ 167.62)	266.84 ($\pm$ 145.40)		
Platelet count at V4	393.98 ($\pm$ 127.92)	366.1 ($\pm$ 144.27)		

Statistical analyses

Statistical analysis title	SEP 4B analysis (V3)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7223
Method	t-test, 2-sided

Statistical analysis title	SEP 4B analysis (V4)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3507
Method	t-test, 2-sided

Secondary: Evolution of hematological abnormalities (schistocytes count)

End point title	Evolution of hematological abnormalities (schistocytes count)
End point description: Evaluation of the evolution of hematological abnormalities based on the schistocytes count in each group at visits V3 and V4	
End point type	Secondary
End point timeframe: at V3 & V4	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: percentage of schistocyte				
arithmetic mean (standard deviation)				
Schistocytes count at V3	5.67 (± 9)	3.83 (± 2.17)		
Schistocytes count at V4	2.67 (± 1.36)	2.4 (± 1.36)		

Statistical analyses

Statistical analysis title	SEP 4C analysis (V3)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.333
Method	Wilcoxon (Mann-Whitney)

Statistical analysis title	SEP 4C analysis (V4)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.333
Method	Wilcoxon (Mann-Whitney)

Secondary: Evolution of hematological abnormalities (hemoglobin level)

End point title	Evolution of hematological abnormalities (hemoglobin level)
End point description: Evaluation of the evolution of hematological abnormalities based on the evolution of the difference in hemoglobin levels between the experimental group and the placebo group between visits V2 and V7	
End point type	Secondary
End point timeframe: From V2 to V7	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: G/dL				
arithmetic mean (confidence interval 95%)				
Hemoglobin level at V2	9.096 (8.572 to 9.620)	8.329 (7.958 to 8.7)		
hemoglobin level at V7	11.403 (11.131 to 11.674)	11.73 (11.468 to 11.991)		

Statistical analyses

No statistical analyses for this end point

Secondary: Evolution of hematological abnormalities (red blood cell transfusion)

End point title	Evolution of hematological abnormalities (red blood cell transfusion)
End point description: Evaluation of the evolution of hematological abnormalities based on information regarding the use of red blood cell transfusion in each group after the initiation of treatment. The measures concern the number of participants who required a transfusion (Yes/No) after the initiation of treatment.	
End point type	Secondary
End point timeframe: After the initiation of treatment	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: number of participants				
Yes	36	35		
No	13	13		

Statistical analyses

Statistical analysis title	SEP 4E analysis
Comparison groups	Placebo arm v Eculizumab treatment arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.951
Method	Chi-squared

Secondary: Evolution of hematological abnormalities (platelet transfusion)

End point title	Evolution of hematological abnormalities (platelet transfusion)
End point description: Evaluation of the evolution of hematological abnormalities based on information regarding the use of platelet transfusion in each group after the initiation of treatment. The measures concern the number of participants who required a transfusion (Yes/No) after the initiation of treatment.	

End point type	Secondary
End point timeframe:	
After the initiation of the treatment	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: number of participants				
Yes	5	6		
No	44	42		

Statistical analyses

Statistical analysis title	SEP 4F analysis
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.721
Method	Chi-squared

Secondary: Evolution of biological parameters of VAC activation (plasma dosage)

End point title	Evolution of biological parameters of VAC activation (plasma dosage)
End point description:	
Evaluation of the biological parameters of VAC activation based on the plasma measurement of the C3 fraction in each group at V3 and V5	
End point type	Secondary
End point timeframe:	
At V3 & V5	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: mg/ml				
arithmetic mean (standard deviation)				
C3 fraction at V3	1345.86 (± 293.6)	1396.06 (± 381.71)		
C3 fraction at V5	1233.81 (± 274.78)	1157.5 (± 290.88)		

Statistical analyses

Statistical analysis title	SEP 5A analysis (V3)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.537
Method	t-test, 2-sided

Statistical analysis title	SEP 5A analysis (V4)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.274
Method	t-test, 2-sided

Secondary: Evolution of biological parameters of VAC activation (membrane expression of CD46)

End point title	Evolution of biological parameters of VAC activation (membrane expression of CD46)
End point description: Evaluation of the biological parameters of VAC activation based on the plasma measurement of the membrane expression of CD46 in each group at V3 and V5	
End point type	Secondary
End point timeframe: At V3 & V5	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: MFI				
arithmetic mean (standard deviation)				
Membrane expression of CD46 at V3	14.96 (± 2.92)	15.43 (± 3.31)		
Membrane expression of CD46 at V5	15.89 (± 2.69)	14.88 (± 3.2)		

Statistical analyses

Statistical analysis title	SEP 5B analysis (V3)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.54
Method	t-test, 2-sided

Statistical analysis title	SEP 5B analysis (V5)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.207
Method	t-test, 2-sided

Secondary: CAM inhibition (CH50 measurement)

End point title	CAM inhibition (CH50 measurement)
End point description: CAM inhibition assessed by CH50 measurement in each group at visits V3 and V5. The measures concern the number of participants with a CH 50 $\leq$ to 20%.	
End point type	Secondary
End point timeframe: At V3 & V5	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: Number of participants				
participants with a CH 50 $\leq$ to 20% at V3	19	1		
participants with a CH 50 $\leq$ to 20% at V5	16	2		

Statistical analyses

Statistical analysis title	SEP 6A analysis (V3)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	> 0.001
Method	Chi-squared

Statistical analysis title	SEP 6A analysis (V5)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.001
Method	Chi-squared

Secondary: CAM inhibition (free ECZ)

End point title	CAM inhibition (free ECZ)
End point description: CAM inhibition assessed by free ECZ plasma assays in each group at visits V3 and V5. The evaluation of this endpoint was only performed for the Eculizumab group.	
End point type	Secondary
End point timeframe: At V3 & V5	

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: mg/l				
arithmetic mean (standard deviation)				
free ECZ plasma assays at V3	252.63 (± 181.2)	0 (± 0)		
free ECZ plasma assays at V5	254.24 (± 191.49)	0 (± 0)		

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of extra-renal manifestations

End point title	Incidence of extra-renal manifestations
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End point description:

The measures concern the number of cases of neurological, cardiac, and digestive disorders during initial hospitalization and after the initiation of treatment.

End point type	Secondary
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End point timeframe:

Throughout the study

End point values	Eculizumab treatment arm	Placebo arm		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	50	50		
Units: number of cases				
Neurological impairment	4	6		
Cardiac disorder	1	5		
Digestive disorder	9	12		

Statistical analyses

Statistical analysis title	SEP 7 analysis (neurologic)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.52
Method	Fisher exact

Statistical analysis title	SEP 7 analysis (cardiac)
Comparison groups	Eculizumab treatment arm v Placebo arm

Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.111
Method	Fisher exact

Statistical analysis title	SEP 7 analysis (digestive)
Comparison groups	Eculizumab treatment arm v Placebo arm
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.459
Method	Chi-squared

Adverse events

Adverse events information^[1]

Timeframe for reporting adverse events:

From first study drug administration up to the end of the 12-month follow-up period, including the safety visits at Months 1, 6, and 12.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
Dictionary version	25.0

Reporting groups

Reporting group title	AE Eculizumab arm
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Reporting group description:

The number of people in this reporting group corresponds to the number of patients who actually received eculizumab treatment.

Reporting group title	AE Placebo arm
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Reporting group description:

The number of people in this reporting group corresponds to the number of patients who actually received placebo.

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.

Justification: Due to the large number of AEs, the list and the number of AEs (serious and non-serious) collected during the study is presented in the adverse event analysis report attached in the "summary attachment" section.

Serious adverse events	AE Eculizumab arm	AE Placebo arm	
Total subjects affected by serious adverse events			
subjects affected / exposed	11 / 54 (20.37%)	7 / 46 (15.22%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Investigations			
Pancreatic enzymes increased			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Vascular disorders			
Venous thrombosis			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Cardiac disorder			

subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Paralysis			
subjects affected / exposed	0 / 54 (0.00%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tonic clonic movements			
subjects affected / exposed	0 / 54 (0.00%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorder			
subjects affected / exposed	1 / 54 (1.85%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Status epilepticus			
subjects affected / exposed	2 / 54 (3.70%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Fever			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Oedema			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Gastrointestinal disorder			
subjects affected / exposed	2 / 54 (3.70%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Abdominal discomfort			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (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			
Pulmonary disorder			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hepatobiliary disorders			
Hepatocellular injury			
subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (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			
Proteinuria			
subjects affected / exposed	1 / 54 (1.85%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Otitis media acute			
subjects affected / exposed	0 / 54 (0.00%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis			
subjects affected / exposed	0 / 54 (0.00%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pyelonephritis			
subjects affected / exposed	1 / 54 (1.85%)	1 / 46 (2.17%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Peritonitis			

subjects affected / exposed	1 / 54 (1.85%)	0 / 46 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	AE Eculizumab arm	AE Placebo arm	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 54 (0.00%)	0 / 46 (0.00%)	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
26 January 2016	Update: - of the drug circuit (labeling) - of the conditions imposed by the risk management plan to which Soliris® is subject (vaccinations and antibiotic prophylaxis) - of the dosages performed at visits 7, 8, and 9 (harmonization between the flowchart and the visit descriptions) - of the randomization procedure - of the conditions for patient participation in another study and discontinuation of the research - of the timelines for reporting adverse events - of the list of investigators Removal of one investigation site (Réunion)
23 March 2017	Extension of the inclusion period and therefore of the study duration by 14 months.
18 September 2017	Addition of a co-coordinator due to the absence of the principal investigator coordinator of the study

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Premature termination leading to a small number of analyzed subjects

Notes: