Clinical trial results:
A PHASE II STUDY TO INVESTIGATE THE EFFICACY OF CYCLOPHOSPHAMIDE (ENDOXAN) AS SOLE GRAFT-VERSUS-HOST-PROPHYLAXIS AFTER ALLOGENEIC STEM CELL TRANSPLANTATION (OCTET-CY)

Summary

EudraCT number	2010-022058-18
Trial protocol	DE
Global end of trial date	10 August 2013

Results information

Result version number	v1 (current)
This version publication date	18 September 2021
First version publication date	18 September 2021
Summary attachment (see zip file)	Octet-Cy_summary_report (Abschlussbericht OCTET 15012015.pdf) PharmNet_results (PharmNet_Bund_results.pdf)

Trial information

Trial identification

Sponsor protocol code	Uni-Koeln-1430

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01283776
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	University of Cologne
Sponsor organisation address	Albertus-Magnus-Platz, Cologne, Germany, 50923
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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	10 August 2013
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	10 August 2013
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the efficacy of post-transplantation cyclophosphamide as single-agent GvHD prophylaxis after allogeneic hematopoietic stem cell transplantation in patients with multiple myeloma or lymphoma and to describe the influence of the modified immunosuppression concept on relapse rates, minimal residual disease, immune reconstitution and chimerism.
Primary end point:

- Number of patients not requiring any additional immunosuppressive treatment until day 100 after allogeneic transplantation

Protection of trial subjects:

Patients will be treated according to current standard practices in the stem cell transplantation programme of the University Hospital Cologne. This includes prophylactic antibiotic, antifungal and antiviral agents. Corticosteroids are permitted in case of acute allergic reactions e.g. after transfusions. If an acute GvHD develops Corticosteroids and/or other immunosuppressive drugs such as calcineurin inhibitors may be used at the discretion of the treating physicians at any time.

Background therapy: -
Evidence for comparator: -

Actual start date of recruitment	14 March 2011
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	Germany: 13
Worldwide total number of subjects	13
EEA total number of subjects	13

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)	13
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

moncentric trial, Germany; recruiting period March 2011 - August 2013

Pre-assignment

Screening details:

non screening has taken place. All patients included in the study fulfilled the inclusion cri-teria.
No protocol deviations were recorded.
All patients were considered for safety analysis.

Period 1

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

Arms

Arm title	allogeneic hematopoietic stem cell transplantation

Arm description:

Patients received cyclophosphamide once daily (50mg/kg bodyweight) intravenous infusion on day +3 and +4 after standard allogeneic stem cell transplantation

Arm type	Experimental
Investigational medicinal product name	Cyclophosphamide
Investigational medicinal product code	
Other name	Endoxan (trade name)
Pharmaceutical forms	Infusion
Routes of administration	Intravenous use

Dosage and administration details:

50mg/kg bodyweight once daily intravenous infusion on days +3 and +4 after standard allogenic stem cell transplantation

Number of subjects in period 1	allogeneic hematopoietic stem cell transplantation
Started	13
Completed	13

Baseline characteristics

Reporting groups	
Reporting group title	Overall trial
Reporting group description:	
Patients with multiple myeloma, Non-Hodgkin’s lymphoma or Hodgkin’s disease after allogeneic stem cell transplantation with reduced intensity conditioning	

Reporting group values	Overall trial	Total	
Number of subjects	13	13	
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)	13	13	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical Units: Subjects			
Female	3	3	
Male	10	10	

End points

End points reporting groups

Reporting group title	allogeneic hematopoietic stem cell transplantation

Reporting group description:

Patients received cyclophosphamide once daily (50mg/kg bodyweight) intravenous infusion on day +3 and +4 after standard allogeneic stem cell transplantation

Primary: Number of patients not requiring any additional immuno-sup-pressive treatment until day 100 after allogeneic transplantation.

End point title	Number of patients not requiring any additional immuno-sup-pressive treatment until day 100 after allogeneic transplantation.[1]

End point description:

Number of patients not requiring any additional immunosup-pressive treatment until day 100 after allogeneic transplantation. - As the main aim of this pilot study is to test post-transplant Cy as single-agent immunosuppression, the absence of systemic immunosuppression at d+100 has been defined as primary endpoint. It is measured as the proportion of patients not requring additional immunosuppressive treatment for GVHD until day 100.

End point type	Primary

End point timeframe:

14.03.2011 – 10.08.2013

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: see attached Summary report and publication reference

End point values	allogeneic hematopoietic stem cell transplantation			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: number of patients				
number of patients	3			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events:	
Advers events were collected from day +3 (beginning of IMP administration) until day 100 after transplantation	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	12.1

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

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: For safety analysis see attached report summary

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported

Online references

http://www.ncbi.nlm.nih.gov/pubmed/25703164