



Clinical trial results:

An exploratory, open-label, multicenter study to evaluate the safety and efficacy of ATIR, donor T-lymphocytes depleted ex vivo of host alloreactive T-cells, in patients with a hematologic malignancy, who received a CD34-selected hematopoietic stem cell transplantation from a haploidentical donor

Summary

EudraCT number	2012-004461-41
Trial protocol	BE DE GB
Global end of trial date	19 September 2017

Results information

Result version number	v1 (current)
This version publication date	07 January 2021
First version publication date	07 January 2021
Summary attachment (see zip file)	CSR CR-AIR-007 Report Synopsis FINAL Revised 18JUL2018 (CSR CR-AIR-007 Report Synopsis FINAL Revised 18JUL2018.pdf)

Trial information

Trial identification

Sponsor protocol code	CR-AIR-007
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01794299
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Kiadis Pharma Netherlands
Sponsor organisation address	Paasheuvelweg 25A, Amsterdam, Netherlands, 1105BP
Public contact	Senior Director Regulatory Affairs, Kiadis Pharma Netherlands B.V., +31 20 2405 277, l.gerez@kiadis.com
Scientific contact	Senior Director Regulatory Affairs, Kiadis Pharma Netherlands B.V., +31 20 2405 277, l.gerez@kiadis.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	18 July 2018
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	19 September 2017
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To study the safety and efficacy of ATIR at a dose of 2×10^6 viable T-cells/kg body weight in patients with a hematologic malignancy who received a haploidentical HSCT.

Protection of trial subjects:

Protocol and informed consent forms (ICFs) were reviewed and approved by appropriate Independent Ethics Committees (IECs). The study was conducted in accordance with the ethical principles that have their origin in the Declaration of Helsinki (and all amendments thereof) and that are consistent with the International Conference on Harmonization (ICH) Guideline for Good Clinical Practice (Topic E6 [R1]) as well as the applicable regulatory requirements.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	28 February 2013
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Canada: 18
Country: Number of subjects enrolled	Belgium: 4
Country: Number of subjects enrolled	United Kingdom: 1
Worldwide total number of subjects	23
EEA total number of subjects	5

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)	23

From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

In this study all eligible patients were planned to be treated with a single dose of ATIR101.

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	ATIR101
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Arm description:

T-lymphocyte enriched leukocyte preparation depleted ex vivo of host alloreactive T-cells using photodynamic treatment (ATIR)

Arm type	Experimental
Investigational medicinal product name	ATIR101
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

All patients received an ATIR101 dose of 2.0×10^6 viable T cells/kg at a median of 28 days (range 28–73) post HSCT.

Number of subjects in period 1	ATIR101
Started	23
Completed	9
Not completed	14
Adverse event, serious fatal	13
preferred to be treated in a hospital closer to ho	1

Baseline characteristics

Reporting groups

Reporting group title	ATIR101
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Reporting group description:

T-lymphocyte enriched leukocyte preparation depleted ex vivo of host alloreactive T-cells using photodynamic treatment (ATIR)

Reporting group values	ATIR101	Total	
Number of subjects	23	23	
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)	23	23	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical			
Units: Subjects			
Female	11	11	
Male	12	12	

End points

End points reporting groups

Reporting group title	ATIR101
Reporting group description: T-lymphocyte enriched leukocyte preparation depleted ex vivo of host alloreactive T-cells using photodynamic treatment (ATIR)	

Primary: Transplant Related Mortality (TRM)

End point title	Transplant Related Mortality (TRM) ^[1]
End point description:	

End point type	Primary
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End point timeframe:

The TRM rate is displayed as a function of time using the Kaplan-Meier method. The TRM rate at 6 months post HSCT is estimated from this analysis.

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: The primary endpoint is TRM until 6 months post HSCT. TRM is defined as death due to causes other than disease relapse or progression, or other causes which are unrelated to the transplantation procedure (e.g. accident, suicide). TRM is a common outcome measure for assessing the efficacy of HSCTs.

The primary efficacy endpoint, TRM at 6 months post HSCT, is estimated to be 13% (95% CI 5-36%).

End point values	ATIR101			
Subject group type	Reporting group			
Number of subjects analysed	23			
Units: Percentage	23			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

24 months

Assessment type	Systematic
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Dictionary used

Dictionary name	CTCAE
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Dictionary version	4
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Reporting groups

Reporting group title	ATIR101
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Reporting group description: -

Serious adverse events	ATIR101		
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 23 (13.04%)		
number of deaths (all causes)	14		
number of deaths resulting from adverse events			
Blood and lymphatic system disorders			
autoimmune hemolytic anemia			
subjects affected / exposed	1 / 23 (4.35%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Immune system disorders			
acute GVHD			
subjects affected / exposed	1 / 23 (4.35%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
chronic GVHD			
subjects affected / exposed	1 / 23 (4.35%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	ATIR101		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	4 / 23 (17.39%)		
Immune system disorders			
acute GVHD			
subjects affected / exposed	4 / 23 (17.39%)		
occurrences (all)	4		

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/32047237>