Clinical trial results:
Irinotecan single drug treatment for children with refractory or relapsed hepatoblastoma

Summary

EudraCT number	2005-002925-29
Trial protocol	AT
Global end of trial date	31 December 2005

Results information

Result version number	v1 (current)
This version publication date	09 April 2021
First version publication date	09 April 2021

Trial information

Trial identification

Sponsor protocol code	200605

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Medical University Innsbruck
Sponsor organisation address	Christoph-Probst-Platz 1, Innrain 52 A, Innsbruck, Austria, 6020
Public contact	Ao. Univ.Prof. Dr. Bernhard Meister, Paediatrics I (hematoloy, oncoloy and stem cell transplantation) Anichstrasse 35, 6020 Innsbruck, +43 (0)512-504-23525, bernhard.meister@tirol-kliniken.at
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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	31 December 2005
Is this the analysis of the primary completion data?	Yes
Primary completion date	31 December 2005
Global end of trial reached?	Yes
Global end of trial date	31 December 2005
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the biologic activity of Irinotecan single drug treatment - when given on a prolonged schedule - in children with refractory or recurrent hepatoblastoma measured by tumour response rate and rate of early progression.

Protection of trial subjects:

A new course of therapy should not begin until the absolute neutrophil count (ANC) has recovered to ≥ 1.0 G/L, the platelet count has recovered to ≥ 75 G/L, and treatement related diarrhea or other non-hematological toxicity is fully resolved. If the patient has not recovered after a 2 week delay, consideration should be given to discontinuing the trial. In patients who experience reversible grade 3-4 toxicity , a 20% dose reduction is permitted for the subsequent courses.

Background therapy:

Management of side effects:
1. Administration of intravenous or subcutaneous atropine should be considered in patients experiencing diaphoresis, abdominal cramping or early diarrhea (dose of atropine 0.01mg/kg, maximum 0.4mg)
2. Late-onset diarrhea (onset more than 2 hours after completion of the Irinotecan infusion) should be treated with Loperamide with a maximum of 0.5mg/kg/day.
3. Administration of Irinotecan should be interrupted if neutropenic fever occurs.These patients should be hospitalised and treated with antibiotics. Subsequent doses of Irinotecan should be reduced.
4: Nausea and vomiting: Pre-medication and treatment with antiemetic agents - preferably the combination of a 5-HT3 blocker and dexamethasone - is recommended.

Evidence for comparator:

No evidence for a comparator.

Actual start date of recruitment	20 September 2005
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	Austria: 1
Worldwide total number of subjects	1
EEA total number of subjects	1

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37	0

wk	
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	1
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study was open for children and young people with a refractory or recurrent hepatoblastoma - with or without metastases - after first or second line treatment.

Pre-assignment

Screening details:

Within the restrictions definded in the eligibility and exclusion criteria, all patients may entered the trial regardless of the type and duration of prior chemotherapy.

Period 1

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

Arms

Arm title	Irinotecan

Arm description:

All patients included in the trial were treated with a total of 4 courses of Irinotecan given 3 weekly as single agent chemotherapy unless tumor progression occured or resectability of the tumor was achieved.

Arm type	Experimental
Investigational medicinal product name	Irinotecan
Investigational medicinal product code	
Other name	Campto
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

Irinotecan was available in 2mL and 5mL vials containing 40mg and 100mg, respectively. The approriate amount of irinotecan was mixed with 5% dextrose injection. Irinotecan was administered as a 60 minutes intravenous infusion at a dose of 20g/ m2 daily for 5 consecutive days and for 2 consecutive weeks repeated every 21 days (day 1-5 and day 8-12).

Number of subjects in period 1	Irinotecan
Started	1
Completed	1

Baseline characteristics

Reporting groups

Reporting group title	Treatment period

Reporting group description: -

Reporting group values	Treatment period	Total	
Number of subjects	1	1	
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)	1	1	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	0	0	
From 65-84 years	0	0	
85 years and over	0	0	
Age continuous Units: years			
arithmetic mean	4		
standard deviation	± 0	-	
Gender categorical Units: Subjects			
Female	0	0	
Male	1	1	

End points

End points reporting groups

Reporting group title	Irinotecan

Reporting group description:

All patients included in the trial were treated with a total of 4 courses of Irinotecan given 3 weekly as single agent chemotherapy unless tumor progression occured or resectability of the tumor was achieved.

Primary: Response rate

End point title	Response rate[1]

End point description:

Subject showed CR

End point type	Primary

End point timeframe:

The duration of overall response was measured from the time measurement criteria, that were met for CR (Complete Response) or PR (Partial Response) until the first date that recurrent or progressive disease was objectively documented.

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 only one patient was enrolled in this trial no statistical analysis was done.

End point values	Irinotecan			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: Lesions [mm]				
number (not applicable)	1			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 20.09.2005-31.12.2005	
Assessment type	Systematic
Dictionary used	
Dictionary name	CTCAE
Dictionary version	2.0
Reporting groups	
Reporting group title	Irinotecan
Reporting group description: All patients included in he trial will be treated with a total of 4 courses of irinotecan given 3 weekly as single agent chemotherapy unless tuor progression occurs or resectability of the tumor is achieved.	

Serious adverse events	Irinotecan		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 1 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

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

Non-serious adverse events	Irinotecan		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 1 (100.00%)		
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	1 / 1 (100.00%)		
occurrences (all)	1		

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

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

Only one subject was enrolled in this trial.

Notes:

Online references

http://www.ncbi.nlm.nih.gov/pubmed/22835780