



## Clinical trial results:

### An Open-Label Extension of the Dose Finding study (DSC/08/2357/36) in patients with polyarticular course Juvenile Idiopathic Arthritis (poly JIA) Summary

EudraCT number	2011-003341-18
Trial protocol	CZ
Global end of trial date	27 January 2014

#### Results information

Result version number	v2 (current)
This version publication date	31 July 2019
First version publication date	25 May 2019
Version creation reason	<ul style="list-style-type: none"><li>• Correction of full data set</li></ul> Short name should be changed.

#### Trial information

##### Trial identification

Sponsor protocol code	DSC/11/2357/42
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##### Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01557452
WHO universal trial number (UTN)	-

Notes:

#### Sponsors

Sponsor organisation name	Italfarmaco S.p.A.
Sponsor organisation address	Via dei Laboratori, 54, Milan, Italy, 20092
Public contact	Clinical Trial Transparency Manager, Italfarmaco S.p.A., Italfarmaco S.p.A., +39 0264432584, info@italfarmaco.com
Scientific contact	Clinical Trial Transparency Manager, Italfarmaco S.p.A., Italfarmaco S.p.A., +39 0266041503, info@italfarmaco.com

Notes:

#### Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMA-000551-PIP01-09
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?	Yes

Notes:

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**Results analysis stage**

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Analysis stage	Final
Date of interim/final analysis	27 January 2014
Is this the analysis of the primary completion data?	Yes
Primary completion date	27 January 2014
Global end of trial reached?	Yes
Global end of trial date	27 January 2014
Was the trial ended prematurely?	No

Notes:

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**General information about the trial**

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Main objective of the trial:

The purpose of this extension study is to determine the safety of Givinostat in a long term treatment of patients who participated in DSC/08/2357/36 study with good results (clinical benefit at least pediACR30 response)

Protection of trial subjects:

The study was conducted under the provisions of the Declaration of Helsinki and in accordance with the International Conference on Harmonization (ICH) Consolidated Guideline on Good Clinical Practice (GCP).

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	28 December 2011
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

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**Population of trial subjects**

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**Subjects enrolled per country**

Country: Number of subjects enrolled	Czech Republic: 1
Worldwide total number of subjects	1
EEA total number of subjects	1

Notes:

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**Subjects enrolled per age group**

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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)	1
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details:

Subjects who had successfully completed the previous Dose Finding Study (2010-019094-15) with a confirmed diagnosis of polyarticular course JIA (RF positive and negative polyarthritis, systemic arthritis without systemic features and extended oligoarthritis) were eligible for entry into this study.

### Pre-assignment

Screening details:

"Eligible patients" are those who have completed the previous dose-finding study achieving a clinical benefit: i.e. patients achieving at least an ACR Paediatric 30 response.

Only one patient was enrolled in the study and was treated for more than two years (from 28/12/2011 to 27/01/2014).

### Period 1

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

Blinding implementation details:

Not applicable; the study was open-label

### Arms

<b>Arm title</b>	Givinostat 0.75 mg/kg bid
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Arm description:

The patient was treated at the dose of 0.75 mg/kg BID from December 28th, 2011 to January 27th, 2014 when the study was terminated as a consequence of Sponsor decision to stop development of Givinostat in polyarticular course Juvenile Idiopathic Arthritis.

Arm type	Experimental
Investigational medicinal product name	Givinostat
Investigational medicinal product code	
Other name	ITF2357, histone deacetylase inhibitor
Pharmaceutical forms	Oral suspension
Routes of administration	Oral use

Dosage and administration details:

Givinostat was supplied as oral suspension for administration at the dose 0,75 mg/kg BID, in fed conditions. The patient was treated from December 28th, 2011 to January 27th, 2014.

<b>Number of subjects in period 1</b>	Givinostat 0.75 mg/kg bid
Started	1
Completed	1

## Baseline characteristics

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### Reporting groups

Reporting group title	Givinostat 0.75 mg/kg bid
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Reporting group description:

The patient was treated at the dose of 0.75 mg/kg BID from December 28th, 2011 to January 27th, 2014 when the study was terminated as a consequence of Sponsor decision to stop development of Givinostat in polyarticular course Juvenile Idiopathic Arthritis.

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Reporting group values	Givinostat 0.75 mg/kg bid	Total	
Number of subjects	1	1	
Age categorical Units: Subjects			
Adolescents (12-17 years)	1	1	
Gender categorical Units: Subjects			
Female	1	1	
Male	0	0	

## End points

### End points reporting groups

Reporting group title	Givinostat 0.75 mg/kg bid
Reporting group description: The patient was treated at the dose of 0.75 mg/kg BID from December 28th, 2011 to January 27th, 2014 when the study was terminated as a consequence of Sponsor decision to stop development of Givinostat in polyarticular course Juvenile Idiopathic Arthritis.	

### Primary: Incidence of SAEs and AEs of interest

End point title	Incidence of SAEs and AEs of interest <sup>[1]</sup>
End point description: During the entire study period it was reported only one adverse event considered not drug related by the investigator (Mild flu at week 107 of study treatment). No action was taken and the patient recovered spontaneously.	
End point type	Primary
End point timeframe: At Day 1, every 12 weeks of treatment, at End of treatment.	
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 data available	

End point values	Givinostat 0.75 mg/kg bid			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: number of patients	1			

### Statistical analyses

No statistical analyses for this end point

### Secondary: Number of patients who maintained PedACR30 response

End point title	Number of patients who maintained PedACR30 response
End point description: This is an open label treatment extension of the 2010-019094-15 study, an antecedent dose ranging trial of Givinostat ready-to-use oral suspension formulation. Eligible patients were those who had completed the previous study achieving a clinical benefit, i.e. patients achieving at least an ACR Paediatric 30 response. Only data at weeks 48, 60 and 108 are reported here.	
End point type	Secondary
End point timeframe: At quarterly controls. from the beginning of the extension study to the end of trial (EOT, week 108)	

<b>End point values</b>	Givinostat 0.75 mg/kg bid			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: Number of patients				
week 48	1			
week 60	0			
week 108	1			

### Statistical analyses

No statistical analyses for this end point

### Secondary: Number of patients who reached PedACR70 response

End point title	Number of patients who reached PedACR70 response
End point description:	
For this endpoint the number of patients who improve the quality of their response (PedACR70) at the quarterly controls was assessed.	
Only data at weeks 48, 60 and 108 are reported here.	
End point type	Secondary
End point timeframe:	
At quarterly controls	

<b>End point values</b>	Givinostat 0.75 mg/kg bid			
Subject group type	Reporting group			
Number of subjects analysed	1			
Units: Number of patients				
week 48	0			
week 60	1			
week 108	0			

### Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

At Day 1, every 12 weeks of treatment, at End of treatment.

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	16
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### Reporting groups

Reporting group title	Givinostat 0.75 mg/kg bid
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Reporting group description:

The patient was treated at the dose of 0.75 mg/kg BID from December 28th, 2011 to January 27th, 2014 when the study was terminated as a consequence of Sponsor decision to stop development of Givinostat in polyarticular course Juvenile Idiopathic Arthritis.

Serious adverse events	Givinostat 0.75 mg/kg bid		
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	Givinostat 0.75 mg/kg bid		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 1 (100.00%)		
Infections and infestations			
Influenza			
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

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### 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.

The study was terminated as a consequence of the Sponsor decision to close the Givinostat Clinical Development in Juvenile Idiopathic Arthritis.
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