Clinical trial results:
Influence of pulsatile dexamethasone therapy in childhood epilepsia on the immune Systeme.
Einflüsse der pulsatilen Dexamethason-Therapie auf das Immunsystem bei der Behandlung kindlicher Epilepsien

Summary

EudraCT number	2016-002658-19
Trial protocol	AT
Global end of trial date	17 August 2021

Results information

Result version number	v1 (current)
This version publication date	27 September 2024
First version publication date	27 September 2024

Trial information

Trial identification

Sponsor protocol code	20016-01

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	Innrain 52, Innsbruck, Austria, 6020
Public contact	Department of Pediatrics I, Department of Pediatrics I, Medical University of Innsbruck, +43 51250423501, kks-regulatory@i-med.ac.at
Scientific contact	Department of Pediatrics I, Department of Pediatrics I, Medical University of Innsbruck, +43 51250423501, kks-regulatory@i-med.ac.at

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	08 November 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	17 August 2021
Global end of trial reached?	Yes
Global end of trial date	17 August 2021
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Changes within the humoral or cellular immune system (e.g. T cell and B cell subsets; specific antibody concentrations against pertussis, measles, and according IgG specific avidities; T cell receptor diversity) after pulsatile dexamethasone treatment

Protection of trial subjects:

There was no additional risk for the patients during the study. Blood was taken as part of routine care.

Background therapy:

anti-epileptic therapy following national treatment guidlines

Evidence for comparator:

There was no evidence for a comparator in this trial.

Actual start date of recruitment	02 October 2019
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: 20
Worldwide total number of subjects	20
EEA total number of subjects	20

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)	14
Adolescents (12-17 years)	6
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Children with West-Syndrome, Lennox-Gastaut-Syndrome, Continuous Spike-Waves during Slow Sleep (CSWS Syndrom) / Electrical Status epilepticus in slow Sleep (ESES) with pulsatile dexamethasone therapy and healthy age matched controls were enrolled at the Department of Paediatrics I, MUI.

Pre-assignment

Screening details:

Children with West-Syndrome, Lennox-Gastaut-Syndrome, Continuous Spike-Waves during Slow Sleep (CSWS Syndrom) / Electrical Status epilepticus in slow Sleep (ESES) with pulsatile dexamethasone therapy and healthy age matched controls were enrolled at the Department of Paediatrics I, MUI.

Pre-assignment period milestones

Number of subjects started	23[1]
Number of subjects completed	20

Pre-assignment subject non-completion reasons

Reason: Number of subjects	Physician decision: 3

Notes:

[1] - The number of subjects reported to have started the pre-assignment period are not the same as the worldwide number enrolled in the trial. It is expected that these numbers will be the same.
Justification: No blood was taken from three patients primarily enrollement. Therefore, these patients were excluded from the study.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Control

Arm description:

11 age- and sex-matched healthy volunteers who were not treated were included.
Blood sampling was done routinely.

Arm type	No intervention

No investigational medicinal product assigned in this arm

Arm title	patients

Arm description:

9 patients who recieved 5 cycles of pulsatile corticoid therapy with dexamethasone 20 mg/m2 i.v. on 3 days were enrolled.

Arm type	Experimental
Investigational medicinal product name	Dexamethasone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate and solvent for dispersion for injection
Routes of administration	Intravenous use

Dosage and administration details:

I.v. administration of dexamethasone; Charge numbers V04412A and T29343B (Dexabene® 4mg Ampullen, Ratiopharm); dosage: 20mg/m² are given in maximum 5 times with a break of minimum 4 weeks between

Number of subjects in period 1	Control	patients
Started	11	9
Completed	11	9

Baseline characteristics

Reporting groups	
Reporting group title	Control
Reporting group description:	
11 age- and sex-matched healthy volunteers who were not treated were included. Blood sampling was done routinely.	
Reporting group title	patients
Reporting group description:	
9 patients who recieved 5 cycles of pulsatile corticoid therapy with dexamethasone 20 mg/m2 i.v. on 3 days were enrolled.	

Reporting group values	Control	patients	Total
Number of subjects	11	9	20
Age categorical Units: Subjects			
Children (2-11 years)	6	6	12
Adolescents (12-17 years)	5	3	8
Age continuous Units: years			
median	11	7.2	
full range (min-max)	6.9 to 15.5	6.0 to 14.2	-
Gender categorical Units: Subjects			
Male	11	9	20

End points

End points reporting groups

Reporting group title	Control

Reporting group description:

11 age- and sex-matched healthy volunteers who were not treated were included.
Blood sampling was done routinely.

Reporting group title	patients

Reporting group description:

9 patients who recieved 5 cycles of pulsatile corticoid therapy with dexamethasone 20 mg/m2 i.v. on 3 days were enrolled.

Primary: possible influence of dexamethasone treatment on the human T and B cell pool and linked immune reactions

End point title	possible influence of dexamethasone treatment on the human T and B cell pool and linked immune reactions[1]

End point description:

Changes within the humoral or cellular immune system (e.g. T cell and B cell subsets; specific antibody concentrations against pertussis, measles, and according IgG specific avidities; T cell receptor diversity) after pulsatile dexamethasone treatment should be assessed by flow cytometric analysis, routine blood testing, ELISA techniques, T cell receptor spectratyping, from whole blood.

Due to small sample size not all tests were performed in all patients/controls.

End point type	Primary

End point timeframe:

06.05.2020 - 17.08.2021

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: Due to small sample size not all tests were performed in all patients/controls. Due to the format of our study no new data on efficacy or safety reasons were gained.

End point values	Control	patients		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	11	9		
Units: specific T and B cell subsets	0	0		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]

Timeframe for reporting adverse events:

06.05.2020 - 17.08.2021

Adverse event reporting additional description:

All patients had received the investigated drug at least several months prior to enrollment in our study population. No relevant changes on efficacy or safety were identified.

Assessment type	Systematic

Dictionary used

Dictionary name	CTCAE
Dictionary version	4.0

Reporting groups

Reporting group title	patients

Reporting group description:

All patients had received the investigated drug at least several months before inclusion into our study population. Therefore, nor relevant changes were found with regard to knowledge of the safety reasons.

Reporting group title	Controll

Reporting group description:

11 age- and gender-matched healthy subjects, which were not treated, were enrolled

Serious adverse events	patients	Controll	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 9 (0.00%)	0 / 11 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

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

Non-serious adverse events	patients	Controll	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 9 (0.00%)	0 / 11 (0.00%)	

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: No AEs were observed in this trial.

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
25 April 2019	According to the new DSGVO, written informed consent sheets had been adaapted in November 2019. An amendment was handed in the local Ethics committee and got approved on the 14th of November 2019

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.

We were unable to recruit the desired number of patients due to the small incidence of patients with intractable epileptic syndromes in our geographical area. This limits the power of the study.

Notes: