ISRCTN08774933 https://doi.org/10.1186/ISRCTN08774933

Use of BETAmethasone in Ataxia Teleangectasia

Submission date 28/10/2008	**Recruitment status** No longer recruiting	[_] Prospectively registered [_] Protocol [_] Statistical analysis plan [X] Results [_] Individual participant data
Registration date 20/11/2008	**Overall study status** Completed	
Last Edited 30/12/2020	**Condition category** Nervous System Diseases	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
2008-001185-91

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
EUDRA-CT: 2008-001185-91

Study information

Scientific Title
Central randomised, double-blind, crossover, multicentre clinical trial of betamethasone and placebo in children with ataxia teleangectasia

Acronym
BETA-AT clinical trial

Study objectives
1. Betamethasone determines a larger decrease of neurological symptoms than placebo
2. Betamethasone determines a larger increase of health-related quality of life than placebo

2006 case study results in http://www.ncbi.nlm.nih.gov/pubmed/17030666.

As of 29/09/2009 this record was updated to include amended anticipated start and end dates; the initial dates at the time of registration were as follows:
Initial anticipated start date: 15/07/2008
Initial anticipated end date: 30/04/2009

Ethics approval required
Old ethics approval format

Ethics approval(s)
The local ethics committee (Azienda Ospedaliera Universitaria Senese Comitato Etico Locale - Sperimentazione Clinica Medicinali) gave approval on the 4th June 2008.

Study design
Phase II, central randomised, double-blind, crossover, multicentre clinical trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Not specified

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details below to request a patient information sheet

Health condition(s) or problem(s) studied
Ataxia telangiectasia (AT)

Interventions
Central randomised, double-blind, crossover study of betamethasone versus placebo, at the dose of 0.05 mg/kg every 12 hours. The substances (betamethasone or placebo) will be administered orally for 30 days (each branch). Between the day 11 and 20, they will slightly

tapered for 10 days; then again administered at a full dosage for 10 days. Each branch of the trial will be followed by a washout period of 30 days. Between the day 1 and 10 of each wash out period, the substances will be slightly tapered.

Added 01/10/2009:
Plasma levels of betamethasone were tested 1 day before entrance into the study and at the 31st day of each arm of the trial.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
Betamethasone

Primary outcome measure
Check of the neurological symptoms (Ataxia International Cooperative Ataxia Rating Scale) before and after the drug or placebo. Test schedule: 1 day before the entrance and at the 31st day within each branch of the trial.

Secondary outcome measures
Check of the general health status and quality of life before and after the drug or placebo. Test schedule: 1 day before the entrance and at the 31st day within each branch of the trial.

Overall study start date
11/11/2008

Completion date
01/07/2009

Eligibility

Key inclusion criteria
1. Proven molecular diagnosis of A-T (alpha-fetoprotein [AFP] level more than twice the upper limit of normal and demonstration of ATM protein deficiency by Western blot)
2. Evident neurological signs of ataxia (uncoordination of head and eyes in lateral gaze deflection, gait ataxia associated with an inappropriately narrow base)
3. Aged greater than or equal to 3 years, either sex
4. Plasma CD4+ lymphocytes/mm^3 greater than or equal to 500 (3 - 6 years) or greater than or equal to 200 (greater than 6 years)
5. Written informed consent to participate from the parents and verbal consent to participate from the patient, if able to understand the main concepts and aims of the study

Participant type(s)
Patient

Age group
Child

Lower age limit
3 Years

Sex
Both

Target number of participants
25

Total final enrolment
13

Key exclusion criteria
1. Confinement to a wheelchair (i.e. inability to walk)
2. Current or previous neoplastic disease
3. History of severe impairment of the immunological system (i.e. history of serious infectious disease)
4. Presence of other chronic conditions (i.e. diabetes, mental delay, osteoporosis, etc) representing a contraindication to the use of a steroid drug
5. Noncompliance with the aims and methods of the study

Date of first enrolment
11/11/2008

Date of final enrolment
01/07/2009

Locations

Countries of recruitment
Italy

Study participating centre
Department of Paediatrics
Siena
Italy
I-53100

Sponsor information

Organisation
Fondazione Monte Paschi di Siena (Italy)

Sponsor details

Via Banchi di Sotto, 34
Siena
Italy
I-53100

Sponsor type
Research organisation

Website
http://www.fondazionemps.it

ROR
https://ror.org/022pga911

Funder(s)

Funder type
Research organisation

Funder Name
Fondazione Monte Paschi di Siena (Italy)

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	01/09/2012	30/12/2020	Yes	No