

Use of BETAmethasone in Ataxia Teleangectasia

Submission date 28/10/2008	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
Registration date 20/11/2008	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 30/12/2020	Condition category Nervous System Diseases	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr Raffaella Zannolli

Contact details
Department of Paediatrics
Policlinico le Scotte
Viale Bracci 8
Siena
Italy
I-53100

Additional identifiers

Clinical Trials Information System (CTIS)
2008-001185-91

Protocol serial number
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

Study type(s)

Treatment

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(s)

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.

Key secondary outcome(s)

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.

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/mm³ 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

Healthy volunteers allowed

No

Age group

Child

Lower age limit

3 years

Sex

All

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)

ROR

<https://ror.org/022pga911>

Funder(s)

Funder type

Research organisation

Funder Name

Fondazione Monte Paschi di Siena (Italy)

Results and Publications

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