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

# Use of BETAmethasone in Ataxia Teleangectasia

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

**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:

## 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.

## Primary study design
Interventional

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

## 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/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

### 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 |