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A Randomized Controlled Study in Newly Diagnosed Severe Aplastic Anemia Patients Receiving Antithymocyte Globulin (ATG), Cyclosporin A, with or without Granulocyte Colony Stimulating Factor (G-CSF)

Submission date
21/09/2005

Registration date
21/10/2005

Last Edited
18/01/2011

Recruitment status
No longer recruiting

Overall study status
Completed

Condition category
Haematological Disorders

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[X] Results
[ ] Individual participant data

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

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
N/A

Study information

Scientific Title

Acronym
SAA-G-CSF

Study objectives
In patients with severe/very severe aplastic anaemia (who are not eligible for bone marrow transplantation), this study aims to evaluate the effect of G-CSF on failure free survival and mortality in patients also receiving ATG and Cyclosporin A

Ethics approval required
Old ethics approval format

Ethics approval(s)
Not provided at time of registration

Study design
Randomised controlled trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Not specified

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Severe or very severe aplastic anaemia

Interventions
Open label, randomized, controlled study of G-CSF, ATG and Cyclosporin A, versus ATG and Cyclosporin A. Subjects will be evaluated for hematologic response through day 240. Subjects who do not demonstrate a partial or complete remission by day 120 will be randomized to receive either a second course of ATG or continue their current regimen. Subjects who do demonstrate a partial or complete remission will continue their current regimen through day 240 or maintenance of a complete remission for 30 days. The last day of study treatment will be day 240.

Intervention Type
Drug

Phase
Not Specified

Drug/device/biological/vaccine name(s)
G-CSF, ATG and Cyclosporin A

Primary outcome measure
To evaluate the effect of G-CSF on failure free survival and mortality in study subjects also receiving ATG and Cyclosporin A. Also time to hematologic response (failure defined as death, non-response or requirement of further treatment).

Secondary outcome measures
1. The proportion of subjects who achieve a hematologic response
2. The incidence of severe infections
3. The benefit due to the addition of G-CSF on death rate, days of hospitalization, and duration of antibiotic treatment
4. Time to achieving a complete remission within 120 days
5. Proportion of subjects who achieve a complete remission within 120 days
6. The relapse rate among responders
7. Median blood counts among subjects who achieve transfusion independence
8. The proportion of subjects who have a change in severity of disease (e.g. improvement from very severe to severe aplastic anemia)
9. Proportion of subjects who respond to re-treatment with ATG
10. The safety of G-CSF in subjects treated with G-CSF, ATG and Cyclosporin A, compared to subjects who receive ATG and Cyclosporin A

Overall study start date
26/02/2001

Completion date
31/12/2007

Eligibility

Key inclusion criteria
1. Severe or very severe aplastic anaemia
2. Less than 6 months from diagnosis of severe aplastic anaemia by bone marrow biopsy
3. For patients in the UK and in Germany there are minimum age restrictions: 16 and 18 years respectively

Participant type(s)
Patient

Age group
Adult

Lower age limit

18 Years

Sex
Both

Target number of participants
340

Key exclusion criteria
1. Eligibility for a human leukocyte antigen (HLA) matched sibling donor transplant
2. Prior therapy with ATG
3. Cyclosporin A <4 weeks before enrollment
4. Treatment with G-CSF <2 weeks before enrollment
5. Other growth factors <4 weeks before enrollment
6. Diagnosis of Fanconi anaemia, dyskeratosis congenita or congenital bone marrow failure syndrome
7. Evidence of myelodysplastic disease
8. Diagnosis or previous history of carcinoma (except local cervical, basal cell, squamous cells, or melanoma)
9. Subjects who have infection, hepatic, renal cardiac, metabolic or other concurrent diseases of such severity that death is imminent
10. Pregnant or breast feeding females

Date of first enrolment
26/02/2001

Date of final enrolment
31/12/2007

Locations

Countries of recruitment
Czech Republic

France

Germany

Greece

Italy

Netherlands

Switzerland

United Kingdom

Study participating centre

Hematology
Basel
Switzerland
4031

Sponsor information

Organisation
European Group Blood and Marrow Transplantation

Sponsor details
P Debyelaan 25
Haematologie
Maastricht
Netherlands
6299HX

Sponsor type
Other

ROR
https://ror.org/014wq8057

Funder(s)

Funder type
Industry

Funder Name
Chugai-Aventis

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	28/04/2011		Yes	No