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Non-randomised trial of a lipid lowering drug and a steroid for the treatment of acute myeloblastic leukaemia

Submission date 03/07/2008	**Recruitment status** No longer recruiting	[_] Prospectively registered [_] Protocol [_] Statistical analysis plan [X] Results [_] Individual participant data
Registration date 25/07/2008	**Overall study status** Completed	
Last Edited 28/09/2011	**Condition category** Cancer	

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
The use of Bezfibrate and medroxyProgesterone acetate in Acute Myeloid Leukaemia and refractory anaemia with excess of blasts (RAEB) type 2: a phase II non-randomised trial

Acronym
BaP in AML

Study objectives
That patients with acute myeloblastic leukaemia (AML) who would not otherwise receive anti-leukaemia therapy will respond to therapy with bezafibrate and medroxyprogesterone acetate.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Ethics approval received from:
1. The South Birmingham Research Ethics Committee on the 9th April 2003 (ref: 5355)
2. University Hospitals Coventry and Warwickshire Research and Development Department on the 7th July 2004 (ref: NJ02/0304/EU)
3. The Research Ethics Committee of Glasgow Royal Infirmary on the 24th July 2003 (ref: 03HA010)

Study design
Interventional multicentre non-randomised phase II study

Primary study design
Interventional

Secondary study design
Non randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Elderly and relapsed high risk acute myeloid leukaemia

Interventions
1. Bezafibrate (Bezalip-Mono) 400 mg daily
2. Medroxyprogesterone acetate (Provera) 200 mg twice daily

Patients will also be given a prophylactic vitamin supplement so that they are not deficient in vitamins A and D, multivitamin tablet containing minimum vitamin A 4000 units and vitamin D 400 units.

Treatment was for 18 weeks. All patients were followed up to death (range of follow up was 8 days to 102 weeks from trial entry); one patient is still alive.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
Bezafibrate, medroxyprogesterone acetate

Primary outcome measure
Tumour response as measured by:
1. Full blood count
2. Transfusion dependency (frequency of red blood cells/platelet transfusions)
3. Percentage blasts in bone marrow and peripheral blood pre and post BaP therapy
4. Bone marrow morphology as determined by blood smears

Response will be assessed using Southwest Oncology Group (SWOG) criteria as modified from National Cancer Institute (NCI-) sponsored workshop guidelines.
1. Complete response (CR): less than 5% blasts in a marrow of sufficient cellularity with a peripheral neutrophil count greater than 1 x 10^9/l and platelet count of greater than 100 x 10^9 /l determined by two evaluations not less than 4 weeks apart
2. Partial response (PR): as determined by two evaluations not less than 4 weeks apart:
2.1. In RAEB type 2 bone marrow should show greater than 50% decrease in myeloblasts, but not necessarily disappearance of marrow dyspoiesis. In peripheral blood, greater than 50% reduction in deficit from minimum normal levels (UHB haematology reference range) of the haemoglobin, neutrophil and platelet counts (if abnormal at baseline) with an absence of myeloblasts in the peripheral blood.
2.2. In AML bone marrow should show less than 15% myeloblasts with a decrease but not necessarily a disappearance of marrow dyspoiesis with an absence of Auer rods. Plus in peripheral blood there should be a greater than 50% reduction in deficit from minimum normal levels (UHB haematology reference range) of haemoglobin, neutrophil and platelet counts (if abnormal at baseline) with absence of myeloblasts in the peripheral blood.
3. Minor response (MR): decrease in frequency of infections or bleeding episodes and a 50% decrease in transfusion requirements, decrease of marrow dyspoiesis and improvement in peripheral counts but not enough to qualify for PR or CR nor progressive disease can be established
4. No change: neither the criteria for CR, PR, MR nor progressive disease can be established
5. Progressive disease: evidence of increased blasts in bone marrow or peripheral blood

Secondary outcome measures
No secondary outcome measures

Overall study start date
01/06/2003

Completion date
01/04/2006

Eligibility

Key inclusion criteria
1. Patient has acute myeloid leukaemia (this can be any type of de novo or secondary AML, except acute promyelocytic leukaemia), or
2. Patient has refractory anaemia with an excess of blasts (greater than 10%) RAEB type 2 World Health Organization (WHO) criteria
3. Adult patients, either sex

Participant type(s)
Patient

Age group
Adult

Sex
Both

Target number of participants
20

Key exclusion criteria
1. Patient has acute promyelocytic leukaemia
2. Intensive chemotherapy is considered a suitable option
3. Low dose cytotoxic chemotherapy is likely to be required to control a rising blast cell count in the next month
4. Patient has a concurrent active malignancy
5. Patient has uncontrolled systemic disease (e.g. hypertension, diabetes) or severe cardiovascular disease
6. Patient is pregnant or lactating, or are potentially fertile (both males and females) and have not agreed to take adequate contraceptive precautions during the trial
7. Patient aged under 18 years

Date of first enrolment
01/06/2003

Date of final enrolment
01/04/2006

Locations

Countries of recruitment
England

United Kingdom

Study participating centre
Clinical Immunology and Division of Immunity and Infection,
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Sponsor information

Organisation
University Hospital Birmingham NHS Foundation Trust (UK)

Sponsor details
Trust Headquarters
PO Box 9551
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Queen Elizabeth Medical Centre
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Sponsor type
Hospital/treatment centre

Website
http://www.uhb.nhs.uk/

ROR
https://ror.org/014ja3n03

Funder(s)

Funder type
Government

Funder Name
The University Hospital Birmingham NHS Foundation Trust (UK) - paying incidental costs

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	01/04/2010		Yes	No