ISRCTN62162141 https://doi.org/10.1186/ISRCTN62162141

A phase 2 study of the efficacy and safety of Deferasirox administered at early iron loading in patients with transfusion-dependent myelodysplastic syndromes

Submission date 05/07/2013	**Recruitment status** No longer recruiting	[_] Prospectively registered [_] Protocol [_] Statistical analysis plan [X] Results [_] Individual participant data
Registration date 05/07/2013	**Overall study status** Completed	
Last Edited 14/02/2020	**Condition category** Cancer	

Plain English summary of protocol
http://www.cancerresearchuk.org/cancer-help/trials/a-study-looking-drug-deferasirox-people-myelodysplastic-syndromes-de-iron

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
2011-004559-38

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
13706

Study information

Scientific Title
A phase 2 study of the efficacy and safety of Deferasirox administered at early iron loading in patients with transfusion-dependent myelodysplastic syndromes

Acronym
Deferasirox for early iron loading in transfusion-dependant MDS

Study objectives
Myelodysplastic Syndromes (MDS) cause a failure of the bone marrow, which does not produce enough blood cells (red cells, white cells and platelets). This is because the bone marrow contains too many abnormal cells (dysplastic cells) which function poorly.

Many patients with MDS do not produce enough red blood cells, which leads to anaemia. This means that they receive regular blood transfusions to treat the anaemia and alleviate symptoms. However, blood is rich in iron and repeated transfusions may cause a build-up of excess iron. Although iron is an essential part of the blood, an excess of iron may affect the way in which the organs in the body function. This includes the liver and heart. This situation is called iron overload.

The aim of this study is to test how effective, safe and tolerable a drug called Deferasirox (also called Exjade®) is when used to treat rising iron levels in patients with MDS. The study treatment will aim to control the iron levels in the blood, which steadily increase after receiving regular blood transfusions. It is not intended to treat MDS. Normally doctors will wait until the level of iron in the blood significantly increases before considering starting treatment for iron overload, but in this study Deferasirox treatment is given early rather than waiting for the iron levels to rise until a high level is reached and organ damage begins. In summary, this study is looking at the feasibility of starting treatment early, before overload begins.

Ethics approval required
Old ethics approval format

Ethics approval(s)
12/NE/0220

Study design
Non-randomised; Interventional; Design type: Process of Care, Treatment

Primary study design
Interventional

Secondary study design
Non randomised study

Study setting(s)
Hospital

Study type(s)
Treatment

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

Health condition(s) or problem(s) studied
Topic: National Cancer Research Network, Blood; Subtopic: Haematological Oncology, Blood (all Subtopics); Disease: Unknown Primary Site, Non-malignant haematology

Interventions
Deferasirox, oral deferasirox for patients with transfusion dependant, low risk MDS and early iron loading; Study entry: registration only

Intervention Type
Drug

Phase
Phase II

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

Primary outcome measure
Time to reach a ferritin of 1500 ug/l; Timepoint(s): Treatment is for 12 months and follow up for 24 months

Secondary outcome measures
Not provided at time of registration

Overall study start date
25/01/2013

Completion date
25/01/2015

Eligibility

Key inclusion criteria
1. At least 18 years old
2. Written informed consent
3. MDS with:
3.1. Baseline haemoglobin concentration < 11 g/dl and clinically requiring red cell transfusion with a frequency of at least 2 units every 6 weeks for the receding 12 week period.
3.2. Serum ferritin > 300 ug/l but < 1000 ug/l in absence of ongoing inflammation (CRP < 3 x ULN)
3.3. Serum creatinine < 1.2 x ULN and/or creatinine clearance > 40 ml/min
3.4. Alanine aminotransferase (ALT) or aspartate aminotransferase (AST) < 2.5 ULN
3.5. International Prognostic Scoring System (IPSS) Low/INT-1 previously untreated or having

failed a therapeutic trail of erythropoetic stimulating agents (ESA) or other active MDS drug therapy, or alternatively lost their response to such therapy
3.6. IPSS INT-2 with <10% blasts and lacking a complex karyotype or monosomy 7 (and with stable blood counts from diagnosis to study entry)
Target gender: male & female

Participant type(s)
Patient

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
Planned Sample Size: 54; UK Sample Size: 54

Total final enrolment
13

Key exclusion criteria
1. Active treatment for MDS, including erythropoetic stimulating agents (ESA), 5-azacitidine, antilymphocyte globulin and low dose chemotherapy such as cytarabine during the trial and within the last 8 weeks
2. Life expectancy of less than 1 year
3. Known HIV positive
4. Active infection
5. Use of prior investigational agents within 6 weeks
6. Pregnancy or lactation
7. Other severe concurrent medical illness that limit the patients prognosis to <1 year, or psychiatric disorders
8. Concurrent active or previous malignancy, within the last 3 years, except controlled, localised prostate cancer on hormone therapy or basal cell carcinoma or cervical carcinoma in situ or completely resected colonic polyps carcinoma in situ
9. Ongoing inflammation as measured by C-reactive protein (CRP) > 3 x ULN
10. Serum creatinine > 1.2 x ULN and/or creatinine clearance <40 mls/min
11. ALT or AST >2.5 ULN
12. History of drug/alcohol abuse or non-compliance

Date of first enrolment
25/01/2013

Date of final enrolment
25/01/2015

Locations

Countries of recruitment
Scotland

United Kingdom

Study participating centre
Cornhill Road
Aberdeen
United Kingdom
AB25 2ZN

Sponsor information

Organisation
University of Birmingham (UK)

Sponsor details
Institute for Cancer studies
Edgbaston
Birmingham
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B15 2TT

Sponsor type
University/education

ROR
https://ror.org/03angcq70

Funder(s)

Funder type
Charity

Funder Name
Leukaemia and Lymphoma Research (Grant Codes: 11019)

Alternative Name(s)

Funding Body Type
Private sector organisation

Funding Body Subtype
Other non-profit organizations

Location
United Kingdom

Funder Name
Novartis Oncology (Switzerland); Grant Codes: CICL670AGB05T

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?
Plain English results				No	Yes
Basic results			21/06/2019	No	No
HRA research summary			28/06/2023	No	No