

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
Registration date 05/07/2013	Overall study status Completed	☐ Statistical analysis plan ☒ Results
Last Edited 14/02/2020	Condition category Cancer	☐ Individual participant data

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

Contact name

Dr Dominic Culligan

Contact details

Cornhill Road
Aberdeen
United Kingdom
AB25 2ZN

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dominic.culligan@nhs.net

Additional identifiers

Clinical Trials Information System (CTIS)

2011-004559-38

Protocol serial number

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

Primary study design

Interventional

Study design

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

Study type(s)

Treatment

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(s)

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

Key secondary outcome(s)

Not provided at time of registration

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 trial of erythropoietic 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

Healthy volunteers allowed

No

Age group

Adult

Lower age limit

18 Years

Sex

All

Total final enrolment

13

Key exclusion criteria

1. Active treatment for MDS, including erythropoietic 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

United Kingdom

Scotland

Study participating centre

Cornhill Road

Aberdeen

United Kingdom

AB25 2ZN

Sponsor information

Organisation

University of Birmingham (UK)

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: C1CL670AGB05T

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