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Assessing the safety, tolerability, and pharmacodynamics of FBS0701 in the treatment of chronic iron overload requiring chelation therapy

Submission date
02/09/2010

Recruitment status
No longer recruiting

Registration date
13/09/2010

Overall study status
Completed

Last Edited
22/03/2016

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

Contact name
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Additional identifiers

Clinical Trials Information System (CTIS)
2010-019645-25

ClinicalTrials.gov (NCT)
NCT01186419

Protocol serial number
FBS0701-CTP-04

Study information

Scientific Title
A phase 2, 24-week, randomized, open label, multi-center study to assess the safety, tolerability, and pharmacodynamics of FBS0701 in the treatment of chronic iron overload requiring chelation therapy

Acronym
NAV

Study objectives
FBS0701 is a safe and tolerable orally available iron chelator when administered chronically daily to patients with transfusional iron overload.

FBS0701 is an oral iron chelator designed to treat iron overload associated with chronic transfusion.

Ethics approval required
Old ethics approval format

Ethics approval(s)
The Essex 1 Research Ethics Committee (REC), July 2010, ref: 10/H031/37

Study design
Multicentre phase II open-label randomized trial

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Transfusional iron overload; hereditary and acquired anemias

Interventions
Patients will be assigned to recieve either 16 mg/kg/day or 32 mg/kg/day of FBS0701 capsules orally once daily. There is no comparator or placebo arm. Screening procedures are carried out over 45 days. Duration of treatment is 24 weeks and the duration of follow-up is for a further 4 weeks beyond the end of the intervention period.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
FBS0701

Primary outcome(s)
1. To evaluate the safety and tolerability based on clinical assessments of two doses of FBS0701 when administered daily to patients with transfusional iron overload, as assessed by:
1.1. Adverse event occurrence
1.2. Changes in vital signs
1.3. 12-lead ECG
1.4. Physical examination
1.5. Clinical laboratory assessments
The assessments above will be carried out at intervals throughout the screening, treatment and follow-up phase.
2. To identify a differential response between dose groups in liver iron content determined by magnetic resonance imaging (MRI). MRI assessments will be performed at baseline, week 12 and week 24.

Key secondary outcome(s))
N/A

Completion date
31/07/2011

Eligibility

Key inclusion criteria
1. Age: 18-60 years old at screening
2. Transfusional iron overload due to:
2.1. Hereditary anemias such as sickle cell disease, β-thalassemia and Blackfan-Diamond anemia
2.2. Acquired anemias such as Myelodysplastic Syndrome and other forms of bone marrow failure
3. Patients must also be transfusion-dependent (8 or more transfusions annually) and require chronic treatment with deferoxamine, deferasirox, and/or deferiprone
4. Willing to discontinue all existing iron chelation therapies throughout the study period
5. Serum ferritin >500 ng/mL at screening
6. Baseline liver iron concentration (LIC) and cardiac T2* MRI per protocol requirements
7. Mean of the previous three pre-transfusion haemoglobin concentrations ≥ 7.5 g/dL
8. Agrees to use an approved method of contraception througout the study

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Upper age limit
60 years

Sex
All

Key exclusion criteria
1. As a result of medical review, physical examination or screening investigations, the Principal Investigator considers the patient unfit for the study
2. Non-elective hospitalisation within the 30 days prior to baseline testing
3. Evidence of clinically relevant oral, cardiovascular, gastrointestinal, hepatic, renal, endocrine, pulmonary, neurologic, psychiatric, immunologic, bone marrow or skin disorder as determined by the investigator
4. Evidence of significant renal insufficiency
5. Cardiac left ventricular ejection fraction outside of protocol requirements
6. Known sensitivity to magnesium stearate, croscarmellose sodium or FBS0701
7. Platelet count below 150,000/µL and/or absolute neutrophil count less than 1500/mm3 at screening
8. Alkaline phosphatase, Aspartate Aminotransferase (AST) or Alanine Aminotransferase (ALT) outside of protocol requirements
9. Use of any investigational agent within the 30 days prior to the baseline testing

Date of first enrolment
01/09/2010

Date of final enrolment
31/07/2011

Locations

Countries of recruitment
United Kingdom

Italy

Thailand

Türkiye

United States of America

Study participating centre
FerroKin BioSciences, Inc.
San Carlos
United States of America
CA 94070

Sponsor information

Organisation
FerroKin BioSciences Inc. (USA)

ROR
https://ror.org/03bygaq51

Funder(s)

Funder type
Industry

Funder Name
FerroKin BioSciences Inc. (USA)

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	05/04/2012		Yes	No
Basic results				No	No
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes