

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	<input type="checkbox"/> Prospectively registered
Registration date 13/09/2010	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 22/03/2016	Condition category Haematological Disorders	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data

Plain English summary of protocol

Not provided at time of registration

Contact information

Type(s)

Scientific

Contact name

Dr Medical Monitor

Contact details

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2729 Debbie Court
San Carlos
United States of America
CA 94070

Additional identifiers

EudraCT/CTIS number

2010-019645-25

IRAS number

ClinicalTrials.gov number

NCT01186419

Secondary identifying numbers

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

Secondary study design

Randomised controlled trial

Study setting(s)

Hospital

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use contact details below to request a patient information sheet

Health condition(s) or problem(s) studied

Transfusional iron overload; hereditary and acquired anemias

Interventions

Patients will be assigned to receive 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 measure

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.

Secondary outcome measures

N/A

Overall study start date

01/09/2010

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 throughout the study

Participant type(s)

Patient

Age group

Adult

Lower age limit

18 Years

Upper age limit

60 Years

Sex

Both

Target number of participants

Approximately 40 patients, 20 patients in each treatment arm.

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/ μ L 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**

Italy

Thailand

Türkiye

United Kingdom

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)

Sponsor details
2729 Debbie Court
San Carlos
United States of America
CA 94070

Sponsor type
Industry

Website
<http://www.ferrokin.com>

ROR
<https://ror.org/03bygaq51>

Funder(s)

Funder type
Industry

Funder Name
FerroKin BioSciences Inc. (USA)

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?
Basic results				No	No
Results article	results	05/04/2012		Yes	No