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A randomised, comparative, open label phase III trial on efficacy and safety of long-term treatment with ICL670 (5 to 40 mg/kg/day) in comparison with deferoxamine (DFO) (20 to 60 mg/kg/day) in β-thalassaemia patients with transfusional haemosiderosis

Submission date
23/07/2003

Registration date
05/09/2003

Last Edited
23/05/2022

Recruitment status
No longer recruiting

Overall study status
Completed

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

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Additional identifiers

ClinicalTrials.gov (NCT)
NCT00061750

Protocol serial number
CICL670 0107

Study information

Scientific Title
A randomised, comparative, open label phase III trial on efficacy and safety of long-term treatment with ICL670 (5 to 40 mg/kg/day) in comparison with deferoxamine (DFO) (20 to 60 mg /kg/day) in β-thalassaemia patients with transfusional haemosiderosis

Acronym
ICL107

Study objectives
This study was undertaken to investigate the hypothesis that deferasirox (ICL670) was noninferior to deferoxamine (DFO).

Ethics approval required
Old ethics approval format

Ethics approval(s)
This trial was conducted in accordance with good clinical practices. Institutional review board or ethics committee approval was obtained at each participating institution and written informed consent was obtained from all patients or their legal guardians prior to participation in any study procedures.

Study design
Randomised controlled trial

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
β-thalassaemia

Interventions
Patients meeting the eligibility requirements were randomised to receive deferasirox or deferoxamine. Randomisation was stratified by age groups:
1. 2 to younger than 12 years
2. 12 to younger than 18 years
3. 18 years or older

After randomisation, patients were assigned by the investigator to a dose dependent on their baseline liver iron concentrations (LIC). Once-daily treatment with deferasirox at the assigned dose was administered as a suspension in water half an hour prior to breakfast 7 days a week.

Deferoxamine was administered as a slow subcutaneous infusion using electronic Microject Chrono infusion pumps (Cane Medical Technology, Torino, Italy) over 8 to 12 hours, 5 days a week.

Treatment with either therapy was continued for 1 year.

Intervention Type
Drug

Phase
Phase III

Drug/device/biological/vaccine name(s)
Deferasirox (ICL670), Deferoxamine (DFO)

Primary outcome(s)
Maintenance or reduction of LIC.

Key secondary outcome(s))
1. Safety and tolerability
2. Change in serum ferritin level
3. Net body iron balance

Completion date
01/11/2003

Eligibility

Key inclusion criteria
1. β-thalassaemia outpatients 2 years old or greater
2. Transfusional haemosiderosis
3. Previously treated with DFO, or never treated with any iron chelator
4. Without any contra-indications to either trial medication

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Not Specified

Sex
All

Key exclusion criteria
1. Alanine aminotransferase (ALT) level greater than 250 U/L during the year prior to enrolment
2. Chronic hepatitis B infection
3. Active hepatitis C infection

4. A history of a positive human immunodeficiency virus (HIV) test
5. Serum creatinine above the upper limit of normal (ULN)
6. A urinary protein-creatinine ratio of greater than 0.5 mg/mg
7. Nephrotic syndrome
8. Uncontrolled systemic hypertension
9. A prolonged corrected QT interval
10. Systemic infection within the 10 days prior to entry
11. Gastrointestinal conditions preventing absorption of an oral medication
12. Concomitant conditions preventing therapy with deferasirox or deferoxamine
13. A history of ocular toxicity related to iron chelation therapy
14. A poor response to deferoxamine
15. Noncompliance with prescribed therapy

Date of first enrolment
01/03/2003

Date of final enrolment
01/11/2003

Locations

Countries of recruitment
United Kingdom

Argentina

Belgium

Brazil

Canada

France

Germany

Greece

Italy

Tunisia

Türkiye

United States of America

Study participating centre

Children's Hospital & Research Center at Oakland
Oakland
United States of America
94609-1809

Sponsor information

Organisation
Novartis Pharmaceuticals Corporation (USA)

ROR
https://ror.org/028fhxy95

Funder(s)

Funder type
Industry

Funder Name
Novartis Pharmaceuticals Corporation (USA)

Results and Publications

Individual participant data (IPD) sharing plan
Not provided at time of registration

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		15/01/2008	23/05/2022	Yes	No
Other publications	A phase 3 study of deferasirox (ICL670), a once-daily oral iron chelator, in patients with beta-thalassemia	01/05/2006		Yes	No
Other publications	Inflammation and oxidant-stress in beta-thalassemia patients treated with iron chelators deferasirox (ICL670) or deferoxamine: an ancillary study of the Novartis CICL670A0107 trial	01/06/2008		Yes	No