Safety Study of Crushed Deferasirox Film Coated Tablets in Pediatric Patients With Transfusional Hemosiderosis (MIMAS)

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Main Information

Primary registry identifying number
LBCTR2019030206

Protocol number
CICL670F2429

MOH registration number
32772/2018

Study registered at the country of origin
Yes

Study registered at the country of origin: Specify

Type of registration
Retrospective

Type of registration: Justify
LCTR was recently initiated, original file was previously submitted by Paper

Date of registration in national regulatory agency
01/08/2018

Primary sponsor
Novartis Pharma Services Inc.

Primary sponsor: Country of origin
Novartis Pharmaceuticals

Date of registration in primary registry
17/12/2019

Date of registration in national regulatory agency
01/08/2018

Public title
Safety Study of Crushed Deferasirox Film Coated Tablets in Pediatric Patients With Transfusional Hemosiderosis (MIMAS)

Acronym

Scientific title
A Single-arm Interventional Phase IV, Post-authorisation Study Evaluating the Safety of Pediatric Patients With Transfusional Hemosiderosis Treated With Deferasirox Crushed Film Coated Tablets

Acronym

Brief summary of the study: English
The study employs an interventional, prospective, single arm, open label, global, multicenter,non-randomized trial design to monitor and assess the safety profile of the crushed deferasirox FCT in pediatric patients between age ≥2 to <6 with transfusional hemosiderosis over 24 weeks. This study will aim to enroll at least 40 patients.

Brief summary of the study: Arabic
دراسة تدخلية وحيدة المجموعة في المرحلة الرابعة بعد الترخيص لتقييم سلامة المرضى الاطفال المصابين بالحدد الدموي (الهيموسيدروسز) ذي الصلة بنقل الدم والمعالجين بأقراص ديفيرازيروكس المسحوقة المغلفة بطبقة رقيقة

Health conditions/problem studied: Specify
Transfusional Hemosiderosis

Interventions: Specify
Drug: Deferasirox
Deferosirox is provided in tablet forms of 90, 180 and 360mg. Tablets must be crushed.

Other Name: ICL670

Key inclusion and exclusion criteria: Inclusion criteria
1.Patients ≥2 to <6 years old diagnosed with transfusional hemosiderosis

2.Documented history of red blood cell transfusions
3.Written informed consent/assent before any study-specific procedures. The consent will be obtained from caregiver(s) or patient's legal representative. Investigators will also obtain assent of patients according to local, regional, or national regulations.
4.For patients on prior DFX: Serum ferritin (SF) >500 ng/mL, measured at screening visit 1 and requiring a DFX daily dose equivalent to FCT ≥ 7mg/kg/day.
5.For patients on a prior chelator other than DFX (e.g. deferiprone or deferoxamine) or chelation naive: Serum ferritin (SF) >1000 ng/mL measured at screening visits 1 and 2.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

2

Key inclusion and exclusion criteria: Age maximum

6

Key inclusion and exclusion criteria: Exclusion criteria

1.Patients that receive more than one iron chelator at the same time as current iron chelation treatment. (Patients who have received combination therapy in their medical history but are currently being treated with a single ICT agent are eligible.)

2.Patients continuing on deferoxamine or deferiprone in addition to study treatment.

(Patients switching to or continuing on deferasirox are eligible).

3.Unresolved adverse events if the patient was previously treated with deferiprone or deferoxamine or deferasirox.
4.Significant proteinuria as indicated by a urinary protein/creatinine ratio > 0.5 mg/mg in a non-first void sample urine measured at screening visit 1.
5.Serum creatinine > age adjusted ULN measured at any screening visit
6.Creatinine clearance below 90 mL/minute measured at any screening visit. Creatinine clearance using the Schwartz formula will be estimated from serum creatinine measured at each respective visit.
7.ALT and/or AST > 2.5 x ULN measured at screening visit 1.
8.Total bilirubin (TBIL) >1.5 x ULN measured at screening visit 1.
9.Patients with significant impaired GI function or GI disease that may significantly alter the absorption of oral deferasirox FCT (e.g. ulcerative diseases, uncontrolled nausea, vomiting, diarrhea, malabsorption syndrome, or small bowel resection).
10.History of and/or laboratory evidence of active Hepatitis B or Hepatitis C (HBsAg in the absence of HBsAb OR HCV Ab positive with HCV RNA positive.
11.Liver disease with severity of Child-Pugh Class B or C.
12.History of hypersensitivity to any of the study drug or excipients.
13.Patients participating in another clinical trial or receiving an investigational drug.
14.Patients with a known history of HIV seropositivity.
15.Patients unwilling or unable to comply with the protocol.
16.History of malignancy of any organ system, treated or untreated, within the past 5 years whether or not there is evidence of local recurrence or metastases, with the exception of localized basal cell carcinoma of the skin.

17.Significant medical condition interfering with the ability to partake in this study (e.g.

uncontrolled hypertension, unstable cardiac disease not controlled by standard medical therapy, systemic disease: cardiovascular, renal, hepatic, etc.).

18.Female patients who reach menarche and they or their caregivers refuse pregnancy testing and/or if there is a positive pregnancy test result.

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Therapy

Trial scope: Specify scope

N/A

Study design: Allocation

N/A: Single arm study

Study design: Masking

Open (masking not used)

Study design: Control

N/A

Study phase

4

Study design: Purpose

Study design: Specify purpose

Treatment

N/A

Study design: Assignment

Single

Study design: Specify assignment

N/A

IMP has market authorization

Yes, Lebanon and Worldwide

IMP has market authorization: Specify

USA, UK, France, Germany, Netherlands, Switzerland, Sweden, Italy...

Name of IMP

Deferasirox Film Coated Tablets "Jadenu"

Year of authorization

2017

Month of authorization

10

Type of IMP

Others

Pharmaceutical class

Non-chiral, Tridentate ligand iron chelator

Therapeutic indication

Iron Over Load

Therapeutic benefit

Treatment of Iron Overload symptoms

Study model

N/A

Study model: Explain model

N/A

Study model: Specify model

N/A

Time perspective

N/A

Time perspective: Explain time perspective

N/A

Time perspective: Specify perspective

N/A

Target follow-up duration

Target follow-up duration: Unit

Number of groups/cohorts

Biospecimen retention

None retained

Biospecimen description

Local Lab tests at site

Target sample size

3

Actual enrollment target size

3

Date of first enrollment: Type

Date of first enrollment: Date

Actual

10/10/2018

Date of study closure: Type

Actual

Date of study closure: Date

11/03/2020

Recruitment status

Complete

Recruitment status: Specify

Date of completion

17/05/2019

IPD sharing statement plan

No

IPD sharing statement description

undecided

Additional data URL

https://clinicaltrials.gov/ct2/show/record/NCT03372083?id=CICL670F2429&rank=1&view=record

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinical Trials. gov	NCT03372083

Sources of Monetary or Material Support

Name
Novartis Pharma Services Inc.

Secondary Sponsors

Name
NA

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Ali Taher	Beirut	Lebanon	009613755669	ataher@aub.edu.lb	Chronic Care Center
Scientific	Hind Khairallah	Sin El Fil	Lebanon	+961 1 512002 Ext. 271	Hind.Khairallah@fattal.com.lb	Khalil Fattal et Fils s.a.l.

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Chronic Care Center	Dr Ali Taher	Hematology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Chronic Care Center	26/07/2018	Michele Abi saad	cccmas@chroniccare.org.lb	+961 3 664 310
American University of Beirut Medical Center	05/07/2018	Fuad Ziyadeh	fz05@aub.edu.lb	+961 (0) 1 350 000 ext:5445

Countries of Recruitment

Name
Egypt
Lebanon
Italy
Oman
Russian Federation
Saudi Arabia
Thailand
United Arab Emirates

Health Conditions or Problems Studied

Condition	Code	Keyword
Transfusional Hemosiderosis	Thalassaemia, unspecified (D56.9)	Transfusional Hemosiderosis

Interventions

Intervention	Description	Keyword
Physical examination, Vitals, Height, Weight, Hematology, Chemistry, urinalisis, ECG, Ocular assessment, Auditory assessment	Physical examination, Vitals, Height, Weight, Hematology, Chemistry, urinalisis, ECG, Ocular assessment, Auditory assessment	ICF, IMP, Lab tests , diary completion

Primary Outcomes

Name	Time Points	Measure
Percentage of patients with selected gastrointestinal disorders	24 weeks	24 wks
To assess the safety of crushed deferasirox FCT with respect to selected gastrointestinal (GI) disorders	through out the study	through out the study

Key Secondary Outcomes

Name	Time Points	Measure
•Percentage of patients who experienced AEs suspected to be related to study drug	24 weeks	24 wks
•Change from baseline ECGs up	24 weeks	24 weeks
•Change from baseline serum ferritin (SF)	24 weeks	24 weeks
•Absolute change for serum creatinine	24 weeks	24 weeks
•Absolute change for creatinine clearance UPCR	24 weeks	24 weeks
•Palatability Questionnaire Score	24 weeks	24 weeks

Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files