



# 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  $\geq 2$  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 الأطفال المصابين بالحديد الدموي (الهيموسيدروس) ذي الصلة بنقل الدم والمعالجين بأقراص ديفيرازيروكس المسحوقة المغلفة بطبقة رقيقة

**Health conditions/problem studied: Specify**

Transfusional Hemosiderosis

**Interventions: Specify**

Drug: Deferasirox  
Deferasirox 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  $\geq 2$  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  $\geq$  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**

**Study design: Specify assignment**

Single

N/A

**IMP has market authorization**

**IMP has market authorization: Specify**

Yes, Lebanon and Worldwide

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

**Name of IMP**

**Year of authorization**

**Month of authorization**

Deferasirox Film Coated Tablets "Jadenu"

2017

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**

**Study model: Explain model**

N/A

N/A

**Study model: Specify model**

N/A

**Time perspective**

**Time perspective: Explain time perspective**

N/A

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                               |
| <b>Date of study closure: Type</b>  | <b>Date of study closure: Date</b>       |
| Actual  | 11/03/2020                               |
| <b>Recruitment status</b>   | <b>Recruitment status: Specify</b>       |
| Complete  |  |
| <b>Date of completion</b>   |  |
| 17/05/2019  |  |
| <b>IPD sharing statement plan</b>   | <b>IPD sharing statement description</b> |
| No  | undecided                                |
| <b>Additional data URL</b>  |  |
| <a href="https://clinicaltrials.gov/ct2/show/record/NCT03372083?id=CICL670F2429&amp;rank=1&amp;view=record">https://clinicaltrials.gov/ct2/show/record/NCT03372083?id=CICL670F2429&amp;rank=1&amp;view=record</a> |  |
| <b>Admin comments</b>   |  |
| <b>Trial status</b>   |  |
| 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 | 009613755<br>669             | ataher@aub.edu.<br>lb             | Chronic<br>Care<br>Center          |
| Scientific   | Hind Khairallah   | Sin El Fil | Lebanon | +961 1<br>512002<br>Ext. 271 | Hind.Khairallah@<br>fattal.com.lb | Khalil<br>Fattal et<br>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 | cccmass@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<br>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, urinalysis, ECG, Ocular assessment, Auditory assessment | Physical examination, Vitals, Height, Weight, Hematology, Chemistry, urinalysis, 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**