Open-label, Multicenter, Single Arm, Phase II Study Assessing Treatment Patient Preference for New Deferasirox Formulation (Film-coated Tablet) Compared to the Reference Deferasirox Dispersible Tablet Formulation

22/11/2024 16:08:17

Main Information

Primary registry identifying number
LBCTR2019020189

Protocol number
CICL670FIC05

MOH registration number
29858/2018

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 already initiated, original file was previously submitted by paper

Date of registration in national regulatory agency
13/07/2018

Primary sponsor
Novartis Pharma Services Inc.

Primary sponsor: Country of origin
Novartis Pharmaceuticals

Date of registration in primary registry
18/06/2021

Date of registration in national regulatory agency
13/07/2018

Public title
Open-label, Multicenter, Single Arm, Phase II Study Assessing Treatment Patient Preference for New Deferasirox Formulation (Film-coated Tablet) Compared to the Reference Deferasirox Dispersible Tablet Formulation

Acronym
JUPITER

Scientific title
Open-label, Multicenter, Single Arm, Phase II Study Assessing Treatment Patient Preference for New Deferasirox Formulation (Film-coated Tablet) Compared to the Reference Deferasirox Dispersible Tablet Formulation

Acronym

Brief summary of the study: English
Study to evaluate patient preference of deferasirox FCT or deferasirox DT in patient with transfusion - dependent thalassemia or non-transfusion -dependent thalassemia as measured by preference questionnaire at Week 48

Brief summary of the study: Arabic
دراسة مفتوحة اللصاقة، متعددة المراكز، وحيدة المجموعة، في المرحلة الثانية لتقييم الأفضليّة العلاجيّة للمريض لصيغة ديفيرازيروكس الجديدة (قرص مغلف بطبقة رقيقة) مقارنة بصيغة ديفيرازيروكس المرجعيّة من قرص قابل للتفتت

Health conditions/problem studied: Specify
•Transfusion-dependent Thalassemia
•Non-transfusion-dependent Thalassemia

Interventions: Specify
Deferasirox (Tablet & Dispersible)

Key inclusion and exclusion criteria: Inclusion criteria

1. Prior to any screening procedures are performed, written informed consent/assent must be provided.
2. Male and female patient aged ≥ 2 years
3. Exjade naïve patient or chelated naive patient or treated by other chelators for at least 6 months, such as: a. Deferiprone/ DFP b. Deferoxamine /DFO c. Combination (DFO + DFP)
4. Subject is willing to discontinue current iron chelation therapy at least 7 days prior to the first day and for the duration of the study
5. Patients with transfusion-dependent thalassemia (independent of underlying condition) with transfusional iron overload as shown by: -a serum ferritin level of > 1000 ng/ml at screening and if available, LIC > 3 mg Fe/g dw until 6 months prior to screening
6. Patients with non-transfusion-dependent thalassemia with iron overload as shown by: -a serum ferritin level of ≥ 800 ng/ml at screening and if available, LIC ≥ 5 mg Fe/g dw until 6 months prior to screening

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

99

Key inclusion and exclusion criteria: Exclusion criteria

1. Male and female patient aged < 2 years
2. Written consent/assent from patients/parents/legal representative is not obtained
3. Creatinine clearance below the contraindication limit in the locally approved prescribing information.
4. Serum creatinine level > 1.5 x ULN (upper limit of normal)
5. AST (SGOT) /ALT (SGPT) > 5 x ULN, unless if LIC confirmed as <10 mg Fe/dw within 6 months prior to screening visit.
6. Significant proteinuria as indicated by a urinary protein/creatinine ratio > 0.5 mg/mg in a non-first void urine sample.
7. Patients with significant impaired gastrointestinal (GI) function or GI disease that may significantly alter the absorption of oral deferasirox (e.g. ulcerative diseases, uncontrolled nausea, vomiting, diarrhea, malabsorption syndrome, or small bowel resection).
8. Clinical or laboratory evidence of active Hepatitis B or Hepatitis C (HBsAg in the absence of HBsAb OR HCV Ab positive with HCV RNA positive).
9. Patients with psychiatric or addictive disorders which prevent them from giving their informed consent or undergoing any of the treatment options or patients unwilling or unable to comply with the protocol (including use of electronic devices for ePRO).
10. Patients with a known history of HIV seropositivity (Elisa or Western blot).
11. 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.
12. Patients participating in another clinical trial or receiving an investigational drug.
13. History of hypersensitivity to any of the study drug or excipients.
14. Significant medical condition interfering with the ability to partake in this study (e.g. systemic uncontrolled hypertension, unstable cardiac disease not controlled by standard medical therapy, systemic disease (cardiovascular, renal, hepatic, etc.).
15. Women of child-bearing potential, defined as all women physiologically capable of becoming pregnant, unless they are using effective methods of contraception during dosing of study treatment
16. Women are considered post-menopausal and not of child bearing potential if they have had 12 months of natural (spontaneous) amenorrhea with an appropriate clinical profile (e.g. age appropriate, history of vasomotor symptoms) or have had surgical bilateral oophorectomy (with or without hysterectomy) or tubal ligation at least six weeks ago. In the case of oophorectomy alone, only when the reproductive status of the woman has been confirmed by follow up hormone level assessment is she considered not of child bearing potential.
17. Sexually active males unless they use a condom during intercourse while taking drug and for 28 days after stopping study medication and should not father a child in this period. A condom is required to be used also by vasectomized men in order to prevent delivery of the drug via seminal fluid.

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

Active

Study phase

2

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Other

Study design: Specify assignment

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IMP has market authorization

Yes, Worldwide

IMP has market authorization: Specify

Albania, Argentina, Canada, United states, United Arab Emirates, Ukraine, Turkey, Switzerland, Saudi Arabia, Oman, Mexico , Malasia

Name of IMP

Deferasirox (ICL670)

Year of authorization

2017

Month of authorization

10

Type of IMP

Others

Pharmaceutical class

Non-chiral, Tridentate ligand iron chelator

Therapeutic indication

Male or female with transfusion-dependent thalassemia or non-transfusion-dependent thalassemia requiring chelation therapy due to iron overload will be included in this study.

Therapeutic benefit

Symptomatic treatment of Thalassemia

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 samples are done at local lab, no samples are retained or shipped outside Lebanon.

Target sample size

10

Actual enrollment target size

5

Date of first enrollment: Type
Actual

Date of first enrollment: Date
18/10/2018

Date of study closure: Type
Actual

Date of study closure: Date
01/04/2021

Recruitment status
Complete

Recruitment status: Specify

Date of completion
01/04/2021

IPD sharing statement plan
Yes

IPD sharing statement description
There is a plan to share IPD , however not mentioned yet on clinical trials.gov

Additional data URL
https://clinicaltrials.gov/ct2/show/record/NCT02993224?id=CICL670FIC05&rank=1

Admin comments

Trial status
Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
National Institute of Health (clinicaltrials.gov)	NCT02993224

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	Baabda	Lebanon	009613755669	ataher@aub.edu.lb	Chronic Care Center
Scientific	Hind Khairallah	Beirut	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	15/05/2018	Michele Abi saad	cccmas@chroniccare.org.lb	+961 3 664 310
American University of Beirut Medical Center	07/11/2017	Fuad Ziyadeh	fz05@aub.edu.lb	+9611350000#5445

Countries of Recruitment

Name
Egypt
Lebanon
Saudi Arabia
Thailand
Turkey
Viet Nam
Algeria
Morocco

Health Conditions or Problems Studied

Condition	Code	Keyword
Thalassemia	Thalassaemia, unspecified (D56.9)	Thalassemia

Interventions

Intervention	Description	Keyword
Audiometry, ECG, Chest X ray, Lab test	Audiometry, ECG, Chest X ray, Lab test	ICF, Lab, Audiometry, IMP administration

Primary Outcomes

Name	Time Points	Measure
Percentage of patient preference for deferasirox FCT vs deferasirox DT	Week 48	week 48

Key Secondary Outcomes

Name	Time Points	Measure
Percentage of patient preference for deferasirox FCT vs deferasirox DT vs previous previous iron chelation	Week 28	Week 28
Percentage of patient preference for deferasirox DT vs previous iron chelation	Week 4 and week 24	Week 4 and week 24
Percentage of reasons for preference of deferasirox FCT vs. deferasirox DT	Week 28 and week 48	Week 28 and week 48
Pill counts to assess drug compliance for deferasirox DT vs FCT	Baseline to wk 24, wk 25 to wk 48	Baseline to wk 24, wk 25 to wk 48

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