Study to Evaluate Treatment Compliance, Efficacy and Safety of an Improved Deferasirox Formulation (Granules) in Pediatric Patients (2-<18 Years Old) With Iron Overload (CALYPSO)

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Main Information

Primary registry identifying number
LBCTR2019020197

Protocol number
ICL670F2202

MOH registration number
6428/ص

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
15/07/2015

Primary sponsor
Novartis Pharma Services Inc.

Primary sponsor: Country of origin
Novartis Pharmaceuticals

Date of registration in primary registry
23/09/2019

Date of registration in national regulatory agency
15/07/2015

Public title
Study to Evaluate Treatment Compliance, Efficacy and Safety of an Improved Deferasirox Formulation (Granules) in Pediatric Patients (2-<18 Years Old) With Iron Overload (CALYPSO)

Acronym
CALYPSO

Scientific title
A randomized, open-label, multicenter, two arm, phase II study to evaluate treatment compliance, efficacy and safety of an improved deferasirox formulation (granules) in pediatric patients with iron overload

Acronym

Brief summary of the study: English
This is a randomized, open-label, multicenter, two arm, phase II study to evaluate treatment compliance and change in serum ferritin of a deferasirox granule formulation and a deferasirox DT formulation in children and adolescents aged ≥ 2 and < 18 years at enrollment with any transfusion-dependent anemia requiring chelation therapy due to iron overload, to demonstrate the effect of improved compliance on iron burden.

Randomization will be stratified by age groups (2 to <10 years, 10 to <18 years) and prior iron chelation therapy (Yes/ No). There will be two study phases which include a 1 year core phase where patients will be randomized to a 48 week treatment period to either Deferasirox DT or granules, and an optional extension phase where all patients will receive the granules up to 5 years. Patients who demonstrated benefit to granules or DT in the core phase, and/or express the wish to continue in the optional extension phase on granules, will be offered this possibility until there is local access to the new formulation (granules or FCT) or up to 5 years, whichever occurs first.

Brief summary of the study: Arabic

دراسة عشوائيّة التوزيع، مفتوحة اللصاقة، متعددة المراكز، ذات مجموعتين، في المرحلة الثانية لتقييم الامتثال للعلاج بصيغة ديفيرازيروكس محسّنة (حُبيبات) وفعاليّتها وسلامتها لدى الأطفال المرضى الذين يعانون من الحديد الزائد

Health conditions/problem studied: Specify

Pediatric Patients (2-<18 Years Old) With Iron Overload

Interventions: Specify

•Drug: Deferasirox granule formulation
Deferasirox granules will be provided as stick packs containing 90 mg, 180 mg and 360 mg granules for oral use.

Other Name: ICL670

•Drug: Deferasirox DT formulation
Deferasirox DT will be provided as 125 mg, 250 mg and 500 mg dispersible tablets for oral use

Other Name: ICL670

Key inclusion and exclusion criteria: Inclusion criteria

•Written informed consent/assent before any study-specific procedures. Consent will be obtained from parent(s) or legal guardians. Investigators will also obtain assent of patients according to local guidelines.
•Male and female children and adolescents aged ≥ 2 and < 18 years. [France: Male and female children and adolescent aged ≥ 2 and < 18 years old, however children aged ≥ 2 and ≤ 6years can be enrolled only when deferoxamine treatment is contraindicated or inadequate in these patients as per investigator decision. Applicable to core phase only. Once in the core phase patients can turn 18 years and still be considered eligible, also for participation in the optional extension phase.
•Any transfusion-dependent anemia associated with iron overload requiring iron chelation therapy and with a history of transfusion of approximately 20 PRBC units and a treatment goal to reduce iron burden (300mL PRBC = 1 unit in adults whereas 4 ml/kg PRBC is considered 1 unit for children).
•Serum ferritin > 1000 ng/mL, measured at screening Visit 1 and screening Visit 2 (the mean value will be used for eligibility criteria).
•Patient has to have participated and completed the 48 weeks core phase treatment as per protocol (For optional extension phase eligibility only).

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

18

Key inclusion and exclusion criteria: Exclusion criteria

•Creatinine clearance below the contraindication limit in the locally approved prescribing information (using Schwartz formula) at screening visit 1 or screening visit 2.
•Serum creatinine > 1.5 xULN at screening measured at screening Visit 1 and or screening Visit 2
•ALT and/or AST > 3.0 x ULN at screening visit 1 or screening visit 2.
•(Criterion no longer applicable, removed as part of Amendment 1): Prior iron chelation therapy.
•Liver disease with severity of Child-Pugh class B or C.
•Significant proteinuria as indicated by a urinary protein/creatinine ratio > 0.5 mg/mg in a second morning urine sample at screening Visit 1 or screening Visit 2.
•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).

Other protocol-defined Inclusion/Exclusion may apply.

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

Randomized controlled trial

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
Single

Study design: Specify assignment
N/A

IMP has market authorization
Yes, Lebanon and Worldwide

IMP has market authorization: Specify
Worldwide

Name of IMP
Jadenu (ICL670) / Deferasirox

Year of authorization
2017

Month of authorization
10

Type of IMP
Others

Pharmaceutical class
Deferasirox is an N-substituted bis-hydroxyphenyl-triazole, a class of tridentate iron chelators.

Therapeutic indication
Patients with Iron Overload/ Transfusion Dependent Anemia

Therapeutic benefit
- Change in serum ferritin in ICT naïve patients.
-The comparison of means between the two treatment arms of change from baseline to week 24 of treatment in serum ferritin in pediatric ICT naïve patients with iron overload.

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
Samples with DNA**

Biospecimen description

MCHC, MCV, Platelets, Red blood cells, White blood cells(WBC) count with differential, RBC Morphology with Differential (Basophils, Eosinophils, Lymphocytes, Monocytes, Neutrophils) Biochemistry Albumin, Alkaline phosphatase, ALT, AST, Bicarbonate, Calcium, Chloride, Creatinine, Creatine kinase, Direct (conjugated) Bilirubin, Indirect Bilirubin, Total Bilirubin, Total Cholesterol, LDL, HDL, Lactate Dehydrogenase (LDH), Total Protein, Triglycerides, Blood Urea Nitrogen (BUN) or Urea, Uric Acid, C Reactive Protein (CRP), Urinalysis Microscopic Panel: Red Blood Cells, White Blood Cells, Casts, Crystals, Bacteria, Epithelial cells
Macroscopic Panel (Dipstick): Color, Bilirubin, Blood, Glucose, Ketones, Leukocytes esterase, Nitrite, pH, Protein, Specific Gravity, Urobilinogen
Hepatitis markers HbsAg, HbsAb, HbcAb, HCV RNA, Anti-HCV
Additional tests Serum ferritin, creatinine clearance, urine protein/creatinine ratio, serum pregnancy test

Target sample size

23

Actual enrollment target size

23

Date of first enrollment: Type

Actual

Date of first enrollment: Date

13/10/2016

Date of study closure: Type

Actual

Date of study closure: Date

31/12/2019

Recruitment status

Complete

Recruitment status: Specify

Date of completion

21/12/2017

IPD sharing statement plan

No

IPD sharing statement description

Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations.

This trial data availability is according to the criteria and process described on www.clinicalstudydatarequest.com

Additional data URL

https://clinicaltrials.gov/ct2/show/NCT02435212?term=2013-004739-55&rank=1

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinical Trials. gov	NCT02435212

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	01-350000 ext 5392	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
American University of Beirut Medical Center	15/06/2016	Fouad Ziyadeh	fz05@aub.edu.lb	+961 (0) 1 350 000 ext:5445
Chronic Care Center	11/07/2016	Michele Abi saad	cccmas@chroniccare.org.lb	+961 3 664 310

Countries of Recruitment

Name
Lebanon
Belgium
Bulgaria
Egypt
Oman
United States of America
India
Italy
France
Tunisia
Turkey

Health Conditions or Problems Studied

Condition	Code	Keyword
Patients with Iron Overload/ Transfusion Dependent Anemia	Anaemia, unspecified (D64.9)	Transfusion Dependent Anemia

Interventions

Intervention	Description	Keyword
Physical examination, height, weight, Hematology, Chemistry, Ferritin, Creatinine, Cleatinine Clearance, Hepatitis, Pregnancy Test, Urine Dipstick, Microscopic Urinalysis, Proteinuria, Urine Pregnancy Test, Liver function test, Ocular exam, audiometry, ECG, Electrocardiogram, PK sampling, vital signs, Growth and development	ICF, IMP, Lab tests and ECG , diary completion	ICF, IMP, Lab tests and ECG , diary completion

Primary Outcomes

Name	Time Points	Measure
•Compliance (using stick/pack tablet count).	24 weeks	24 wks
•Change in serum ferritin in ICT naive patients	baseline, 24 wks	baseline, 24 wks

Key Secondary Outcomes

Name	Time Points	Measure
•Compliance (using stick/pack tablet count)	48 weeks	48 wks
•Change in serum ferritin in ICT naive patients	baseline, 24 wks, 48 wks	baseline, 24 wks, 48 wks
•Overall safety, as measured by frequency and severity of adverse	from baseline to 48 wks	from baseline to 48 wks

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