A Phase 3, Open-label, Uncontrolled Study to Evaluate the Activity, Safety, Pharmacokinetics and Pharmacodynamics of Roxadustat for the Treatment of Anemia in Pediatric Participants with Chronic Kidney Disease

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Main Information

Primary registry identifying number
LBCTR2024015495

Protocol number
1517-CL-1003

MOH registration number
-

Study registered at the country of origin
No

Study registered at the country of origin: Specify
-

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency
30/01/2024

Primary sponsor
Astellas

Primary sponsor: Country of origin
USA

Date of registration in primary registry
07/03/2024

Date of registration in national regulatory agency
30/01/2024

Public title
A Phase 3, Open-label, Uncontrolled Study to Evaluate the Activity, Safety, Pharmacokinetics and Pharmacodynamics of Roxadustat for the Treatment of Anemia in Pediatric Participants with Chronic Kidney Disease

Acronym
-

Scientific title
A Phase 3, Open-label, Uncontrolled Study to Evaluate the Activity, Safety, Pharmacokinetics and Pharmacodynamics of Roxadustat for the Treatment of Anemia in Pediatric Participants with Chronic Kidney Disease

Acronym
-

Brief summary of the study: English
Roxadustat has been approved for the treatment of adult patients with symptomatic anemia associated with CKD. This study is part of a program to develop Roxadustat in the treatment of anemia in pediatric CKD patients. Since no data in pediatrics is currently available, per the Committee for Medicinal Products for Human Use scientific advice, a study duration of 52 weeks allows for adequate characterization of the safety and activity of Roxadustat.
In the EU, this study is in accordance with the PIP approved by the PDCO.

Brief summary of the study: Arabic
تمت الموافقة على Roxadustat لعلاج المرضى البالغين الذين يعانون من فقر الدم العرضي المرتبط بمرض الكلى المزمن. هذه الدراسة هي جزء من برنامج لتطوير Roxadustat في علاج فقر الدم لدى مرضى CKD من الأطفال. نظرا لعدم توفر بيانات في طب الأطفال حاليا ، أسبوعا تسمح بالتوصيف المناسب لسلامة ونشاط52وفقا للمشورة العلمية للجنة المنتجات الطبية للاستخدام البشري ، فإن مدة الدراسة البالغة Roxadustat.
تتوافق هذه الدراسة مع PIP المعتمدة من قبل PDCO في الاتحاد الأوروبي.

Health conditions/problem studied: Specify

Anemia in Pediatric Participants with Chronic Kidney Disease.

Interventions: Specify

Roxadustat will be administered 3 times a week according to weight-based pediatric dosing.
A suitable, age-appropriate, roxadustat azo dye-free mini-tablet or tablet formulation will be utilized by participants who are able to swallow tablets and is to be diluted with purified water and administered via syringe orally or via a gastric tube as an aqueous dispersion for participants unable to swallow tablets.
For ESA-treated participants, the appropriate starting dose of roxadustat will be based on prior ESA dose and the participant's body weight. Titrations to the roxadustat starting dose will be based on current Hb level and change in Hb over the previous 4 weeks.

Key inclusion and exclusion criteria: Inclusion criteria

1. IRB/IEC approved written informed consent and privacy language as per national regulations (e.g., Health Insurance Portability and Accountability Act authorization for US study sites) must be obtained from the participant or participant's parent or legal guardian, and if required, child assent, prior to any study-related procedures (including withdrawal of prohibited medication, if applicable).
2. Participant is aged 2 to < 18 years old at the screening/first study visit.
3. Participant has a diagnosis of anemia in CKD Kidney Disease Outcomes Quality Initiative stages 3 or 4 or 5. This can include participants not on dialysis or DD participants (including hemodialysis, peritoneal dialysis and hemodiafiltration participants).
4. Participants not on dialysis must have an estimated glomerular filtration rate (Schwartz formula) of < 60 mL/min per 1.73 m2.
5. ESA-treated participants should have a screening Hb level, assessed via HemoCue, between 10.0 and 12.0 g/dL; ESA-naïve participants can have a Hb level ≤ 11 g/dL.
6. Participant has a ferritin level > 100 ng/mL or a TSAT value > 20%.
7. Participant has an ALT and AST ≤ 2 x ULN and TBL ≤ 1.5 x ULN at enrollment visit.
8. Participant is treated with an ESA or is ESA-naïve, where ESA status is defined as:
a. ESA-treated: Participant is taking a stable dose of an ESA for at least 4 weeks prior to screening.
b. ESA-naïve: Participant has no prior ESA exposure OR participant's total prior ESA exposure ≤ 3 weeks within the preceding 4 weeks from screening OR participant was previously treated with and discontinued an ESA ≥ 8 weeks prior to screening.
9. Female participant is not pregnant (see [Appendix 2]) and at least 1 of the following conditions apply:
c. Not a WOCBP (see [Appendix 2])
d. WOCBP who agrees to follow the contraceptive guidance (see [Appendix 2]) from the time of informed consent through at least 4 weeks after final study intervention administration.
10. Female participant must agree not to breastfeed starting at screening and throughout the study and for 4 weeks post-last roxadustat dose.
11. Female participant must not donate ova starting at first administration of roxadustat and throughout the study period and for 4 weeks post-last roxadustat dose.
12. Male participants with female partner(s) of childbearing potential (including breastfeeding partner) must agree to use contraception (see [Appendix 2]) throughout the treatment period and for 4 weeks post-last roxadustat dose.
13. Male participants must not donate sperm during the treatment period and for 4 weeks post-last roxadustat dose.
14. Male participants with pregnant partner(s) must agree to remain abstinent or use a condom for the duration of the pregnancy throughout the study period and for 4 weeks post-last roxadustat dose.
15. Participant and/or participant's parent or legal guardian agrees for the participant not to participate in another interventional study while participating in the present study.

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

1. Participant has received any investigational therapy within 28 days or 5 half-lives, whichever is longer, prior to screening.
2. Participant has any medical condition, including active, systemic or clinically significant infection, which in the opinion of the investigator or medical monitor, may pose a safety risk to a participant in this study, which may confound the safety or activity assessment or may interfere with study participation making the participant unsuitable for study.
3. Participant has a known or suspected hypersensitivity to roxadustat, related HIF-PHI, or any components of the formulation used.
4. Participant has uncontrolled hypertension as judged by the principal investigator in the 2 weeks prior to screening.
5. Participant has a known hematologic disease other than anemia secondary to renal disease, (e.g., history of sickle cell disease, sickle cell anemia, hemoglobin sickle cell disease, or hemoglobin sickle cell beta thalassemia).
6. Participant has untreated hypothyroidism.
7. Participant has severe hyperparathyroidism defined as serum PTH levels above 1000 pg/mL intact PTH within 4 weeks of screening.
8. Participant has a functioning kidney allograft.
9. Participant has a folate or B12 or carnitine deficiency. Acceptable if treated to normal values within 4 weeks of screening.
10. Participant has a known active malignancy or malignancy within 18 months before the screening visit. Radiation or chemotherapy must be completed at least 12 months before the screening visit.
11. Participant has a scheduled living donor organ transplantation date within 12 weeks of screening.
12. Participant has a whole blood or packed RBC transfusion during the 8 weeks prior to screening.
13. Participant has any current condition leading to active significant blood loss in the past 4 weeks.
14. Participant has a diagnosis of hemolytic uremic syndrome within 12 weeks prior to screening.
a. Participant who has a previous diagnosis of atypical hemolytic syndrome must be relapse-free (stable Hb, normal platelet count, normal serum lactate dehydrogenase, and normal haptoglobin level) for more than 12 weeks prior to screening.

15. Participant has a history of chronic liver disease, including comorbidity with autosomal recessive polycystic kidney disease, cystinosis, and primary hyperoxaluria.
16. Participant had an episode of peritonitis within 30 days of screening.
17. Participant has active inflammation such as glomerulonephritis flare (i.e., lupus nephritis, IgA nephritis, rapidly progressive glomerulonephritis, membranoproliferative glomerulonephritis, antineutrophil cytoplasmic antibodies vasculitis) requiring pulse corticosteroid treatment or induction treatment with an immunosuppressive agent (i.e., cyclophosphamide, rituximab, or another monoclonal antibody) within 6 weeks of screening visit. Receipt of monoclonal antibody or biologic for maintenance treatment of underlying condition is acceptable.
18. Participant has a known history of human immunodeficiency virus infection.

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

Single Arm Study

Study design: Masking

Open (masking not used)

Study design: Control

Uncontrolled

Study phase

3

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, Worldwide

IMP has market authorization: Specify

China, Japan, Europe, Great Britan

Name of IMP	Year of authorization	Month of authorization
Roxadustat	2018	12

Type of IMP

Others

Pharmaceutical class

Hypoxia-inducible factor, prolyl hydroxylase inhibitor (HIF-PHI).

Therapeutic indication

Symptomatic anemia associated with CKD.

Therapeutic benefit

In adult CKD participants with anemia not on dialysis, there was a statistically significant increase in Hb from baseline to weeks 28 through 36 in participants treated with roxadustat, showing superiority of roxadustat over placebo. The proportion of participants who achieved Hb response during the first 24 weeks without rescue therapy was higher in the roxadustat group compared with placebo. This difference was statistically significant and showed superiority of roxadustat over placebo. In addition, roxadustat was shown to be non-inferior to an ESA in adult CKD participants not on dialysis.
In adult CKD participants with anemia on dialysis, the change from baseline in Hb without use of rescue therapy up to weeks 28 through 36 was similar between groups and noninferiority of roxadustat to ESA was confirmed. Similarly, the proportion of participants who achieved Hb response without use of rescue therapy up to weeks 28 through 36 was similar between groups and noninferiority of roxadustat to ESA was confirmed.

Study model

N/A

Study model: Explain model

Study model: Specify model
N/A

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
NA

Target sample size
100

Actual enrollment target size

Date of first enrollment: Type
Anticipated

Date of first enrollment: Date
01/06/2024

Date of study closure: Type
Anticipated

Date of study closure: Date
31/12/2030

Recruitment status
Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan
No

IPD sharing statement description
NA

Additional data URL
-

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
NA	NA

Sources of Monetary or Material Support

Name
Astellas

Secondary Sponsors

Name
NA

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Aziz Zoghbi	MCT Lebanon s.a.r.l.	Lebanon	01--612500 ext2040	Zog_Az@Mctcro.com	CRO
Scientific	Chebl Mourani	Hotel Dieu de France Hospital	Lebanon	03 290 090	cheblmourani@gmail.com	PI

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu de France Hospital	Dr Chebl Mourani	Pediatrician	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	02/10/2023	Virginia El khoury	Virginia.elkhoury@usj.edu.lb	+961 1 421229

Countries of Recruitment

Name
Lebanon

Health Conditions or Problems Studied

Condition	Code	Keyword
Anemia in pediatrics with CKD	Anaemia in chronic diseases classified elsewhere (D63)	Anemia in Chronic Kidney Disease

Interventions

Intervention	Description	Keyword
Roxadustat	Hemopoiesis Stimulating Agent	Treatment

Primary Outcomes

Name	Time Points	Measure
The Primary Objective is to Evaluate the activity of Roxadustat for the treatment of anemia in adolescents and children with CKD.	Weeks 20 to 24	Change in Hb level between baseline (before start of dosing) and average Hb level over treatment weeks 20 to 24. The 24-week treatment period is defined as 4 weeks of fixed dose treatment followed by 20 weeks of dose titration(s)

Key Secondary Outcomes

Name	Time Points	Measure
Evaluate the PK and PD of roxadustat for the treatment of anemia in adolescents and children with CKD	-	PK parameters: Cmax, AUC, CL/F, Tmax ● PD assessments: Hb level and dose titration history at all timepoints
Evaluate the safety of roxadustat for the treatment of anemia in adolescents and children with CKD, including cardiovascular and thrombotic risks	-	● Number and percent of TEAEs ● Number and percent of treatment-emergent cardiovascular and thrombotic AEs ● Tabulations of safety assessments (e.g., clinical laboratory tests, vital signs, growth parameters and physical examinations)

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