Patiromer for treatment of hyperkalaemia in children from birth to <6 years of age

13/08/2025 02:41:16

Main Information

Primary registry identifying number
LBCTR2023055223

Protocol number
RLY5016-208p

MOH registration number

Study registered at the country of origin
Yes

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
14/12/2022

Primary sponsor
Vifor Pharma, Inc.

Primary sponsor: Country of origin
USA

Date of registration in primary registry
13/06/2023

Date of registration in national regulatory agency
14/12/2022

Public title
Patiromer for treatment of hyperkalaemia in children from birth to <6 years of age

Acronym

Scientific title
A 2-Part, Open-Label, Phase 2, Multiple Dose Study to Evaluate the Pharmacodynamic Effects, Safety, and Tolerability of Patiromer in Children under 6 Years of Age with Hyperkalaemia (EMERALD 2)

Acronym

Brief summary of the study: English

This is a 2-part, multicentre, open-label, Phase 2, multiple dose, 2-age cohort (0 to <2 and 2 to <6 years of age) study, consisting of a 4-week pharmacodynamic (PD)/dose-ranging period followed by an optional 52-week safety-extension period to assess change in potassium levels from baseline to Day 28 following administration of different doses of patiromer administered in children 0 to <6 years of age with hyperkalaemia and To assess the safety and tolerability of patiromer in children 0 to <6 years of age with hyperkalaemia.The maximum study duration for a participant will be up to 58 weeks, including an up to 2 weeks screening period, a 4-week PD/dose-ranging period (Part 1), and a 52-week optional safety-extension period (Part 2).The study will include 2 cohorts: Cohort A (2 to <6 years of age) and Cohort B (0 to <2 years of age).

Brief summary of the study: Arabic

هذه دراسة مكونة من جزئين ، متعددة المراكز ، مفتوحة التسمية ، المرحلة 2 ، جرعة متعددة ، فئة عمرية 2 إلى أقل من 0 (من 2 إلى أقل2 و 0 بعد إعطاء جرعات مختلفة من باتيروماير المعطى للأطفال من سن 28أسبوعًا لتقييم التغيير في مستويات البوتاسيوم من خط الأساس إلى اليوم 28 ، وتتألف من 6من أسابيع من الديناميكيات الدوائية4 سنوات من العمر) / (PD) فترة الجرعة متبوعة بفترة تمديد أمان اختيارية مدتها 52فترة نطاق / PD أسابيع4 أسبوعًا ، بما في ذلك فترة فحص تصل إلى أسبوعين ، و 58الدم ، وستصل مدة الدراسة القصوى للمشارك إلى 6 إلى أقل من 2). ستشمل الدراسة مجموعتين: الفوج أ (من 2 أسبوعًا (الجزء 52الجرعة (الجزء الأول) ، وفترة تمديد أمان اختيارية مدتها إلى أقل من سنتين من العمر0سنوات من العمر) والفوج ب (من). سنوات مع فرط بوتاسيوم6 إلى أقل من 0 سنوات مع فرط بوتاسيوم الدم ولتقييم السلامة وتحمل البيروماير لدى الأطفال من سن 6إلى أقل من

Health conditions/problem studied: Specify

Hyperkalaemia

Interventions: Specify

The study will include 2 parts: 1) a 4-week PD/dose-ranging period in an interventional clinical trial setting, followed by 2) an optional 52-week safety-extension period. In the optional safety-extension period, the frequency and procedures of visits will adhere to the standard of care for paediatric subjects of the specific study site; however, at least every 3 months. Potassium values, patiromer dosing and compliance, and adverse events (AEs) will be reported.
During this study, potassium values can be measured as serum, plasma, whole blood, or capillary blood potassium. However, any of the above methods need to be kept constant throughout the study, from screening visit to end of study.

Key inclusion and exclusion criteria: Inclusion criteria

1. Paediatric subjects (<6 years of age) with hyperkalaemia at screening.
2. Subject's age should not reach 6 years during the 28 days of the PD/ dose-ranging period.
3. Subject is able to receive regular external feeding and medication, including via tubes, e.g., percutaneous endoscopic gastrostomy (PEG).
4. At screening/baseline, 2 potassium values need to be above the age-appropriate upper limit of normal (ULN) of the local laboratory. However, the average of the 2 potassium values from 2 separate sample collections (1 sample from screening/baseline and 1 sample not older than 30 days) needs to be above the age-appropriate ULN of the local laboratory plus 0.5 mEq/l (equivalent to 0.5 mmol/l).
5. In the opinion of the Investigator, the subject is expected to require treatment for hyperkalaemia for at least 28 days upon enrolment in the study.
6. If taking any renin-angiotensin aldosterone system inhibitors (RAASi), beta blockers, fludrocortisone, or diuretic medications, must be on a stable dose for at least 14 days prior to screening.
7. Parent(s) or legally acceptable representative(s) has provided the appropriate written informed consent, in accordance with local regulations. The assent of the child should also be obtained when appropriate or if requested by the Institutional Review Board (IRB)/Ethics Committee (EC)/Independent Ethics Committee (IEC). The written informed consent must be provided before any study-specific procedures are performed including screening procedures.
8. Parent(s) or legally authorised representative(s) or another appropriate person delegated by the legally authorised representatives must be available to help the study-site personnel ensure follow-up; accompany the participant to the study site on each assessment day according to the Schedule of Events (Table 1, Table 2) (e.g., able to comply with scheduled visits, treatment plan, laboratory tests and other study procedures); accurately and reliably dispense investigational product as directed.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

0

Key inclusion and exclusion criteria: Age maximum

5

Key inclusion and exclusion criteria: Exclusion criteria

1. Preterm birth infants with <37 weeks of gestation cannot be included in Cohort B.
2. Participants who due to their general condition, e.g., anaemia or low body weight are not suitable to have blood volume withdrawn as specified in the Schedule of Events (Table 1, Table 2).
3. Subjects with pseudo-hyperkalaemia due to haemolysis or to abnormally high numbers of platelets (above ULN), leukocytes (above ULN), or erythrocytes (above ULN) at screening based on results obtained from the local laboratory.
4. Any subject with evidence of potential potassium-related 12-lead electrocardiogram (ECG) changes (i.e., changes consistent with hyper- or hypokalaemia) at screening.
5. Any subject with serum magnesium <1.4 mg/dl at screening/baseline.
6. Any of the following renal conditions: maintenance haemodialysis or peritoneal dialysis, renal artery stenosis, and acute kidney injury (defined by 2012 Kidney Disease Improving Global Outcomes) or a history of acute renal insufficiency in the past 3 months. Note: CKD is not excluded.
7. A history of or current diagnosis of a severe gastrointestinal (GI) diagnosis or surgery that could affect GI transit of the drug (delayed gastric emptying), such as a severe swallowing disorder, severe gastroesophageal reflux, uncorrected pyloric stenosis, intussusception, any other intestinal obstruction (e.g., Hirschsprung disease, chronic intestinal pseudo-obstruction, clinically significant postsurgical abdominal adhesions) or any gut-shortening surgical procedure prior to screening. Pre-gastric above-mentioned pathologies may be disregarded in case of existence of a PEG tube, as the PEG tube will serve for nutrition and investigational product administration.
8. Liver enzymes (alanine aminotransferase, aspartate aminotransferase) more than 3 times the ULN at screening, based on the local laboratory, as well as subject's respective age.
9. Active cancer, currently on cancer treatment, or history of cancer in the past 2 years (except for non-melanoma skin cancer).
10. Recipient of any organ transplant requiring treatment with immunosuppressive therapy or scheduled for kidney transplant procedure during the first 28 days after Day 1.
11. History of sudden infant death in a sibling.
12. Has severe hypoxaemia, respiratory acidosis, asphyxia, or hypotension 3 months before screening based on assessment of the Investigator.
13. Subjects treated with sodium polystyrene sulphonate, calcium polystyrene sulphonate, or sodium zirconium cyclosilicate within the last 48 hours prior to fulfilling the baseline potassium assessments requested in Inclusion Criterion 4.
14. Use of the following medications if doses have not been stable for at least 14 days prior to screening or if doses are anticipated to change during the 4-week PD/dose-ranging period: digoxin, bronchodilators, theophylline, heparins (including low molecular heparins), tacrolimus, mycophenolate mofetil, cyclosporine, trimethoprim, or cotrimoxazole.
15. Use of any investigational product for an unapproved indication within 30 days prior to screening or within 5 half-lives, whichever is longer.
16. Known hypersensitivity to patiromer or its components.
17. In the opinion of the Investigator, parent(s) or legal representative(s) inability to comply with the protocol.

18. In the opinion of the Investigator, any medical condition, uncontrolled systemic disease, or serious intercurrent illness that would significantly decrease study compliance or jeopardise the safety of the subject or potentially affect the quality of the data such as: hyperkalaemia at screening that requires emergency intervention; cardiovascular event or intervention within 3 months prior to screening; a haemodynamically unstable arrhythmia; hospitalisation for heart failure within the past 3 months; poorly controlled blood pressure; poorly controlled diabetes mellitus or frequent need for adjustment in insulin prescription or recent hospitalisation for treatment of hyper or hypoglycaemia.
19. If the child is being breastfed:
a) There is suspicion of current alcohol or substance misuse/abuse in breastfeeding mother
b) The breastfeeding mother is taking potassium supplements.

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Other

Trial scope: Specify scope

Study design: Allocation

Non-randomized controlled trial

Study design: Masking

Open (masking not used)

Study design: Control

N/A

Study phase

2

Study design: Purpose

Other

Study design: Specify purpose

Pharmacodynamic, Efficacy, Safety, Dose response

Study design: Assignment

Other

Study design: Specify assignment

cohort design

IMP has market authorization

Yes, Lebanon and Worldwide

IMP has market authorization: Specify

USA, EU, Norway, Iceland, Lichtenstein, Switzerland and Australia, Eurasian Economic Union (EAEU; Russia)

Name of IMP

Patiromer

Year of authorization

2015

Month of authorization

10

Type of IMP

Others

Pharmaceutical class

Potassium binder

Therapeutic indication

treatment of hyperkalaemia in Pediatric population

Therapeutic benefit

This study will assess safety and dosing in this population and represents at the same time a potential treatment for hyperkalaemic paediatric subjects.

Study model

N/A

Study model: Explain model

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

NA

Target sample size

21

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

17/04/2023

Date of study closure: Type

Anticipated

Date of study closure: Date

30/04/2026

Recruitment status

Other

Recruitment status: Specify

Not Initiated

Date of completion

IPD sharing statement plan

Yes

IPD sharing statement description

The sponsor assures that the key design elements of this protocol will be posted in a publicly accessible database such as ClinicalTrials.gov. The clinical study report will be submitted to the regulatory authorities in Lebanon within one year of the end of the study (worldwide). The detailed obligations regarding the publication of any data, material results, or other information generated or created in relation to the study shall be set out in the agreement between each investigator and the sponsor.

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

No Numbers

Sources of Monetary or Material Support

No Sources

Secondary Sponsors

No Sponsors

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Aziz Zoghbi	MCT-CRO,Berytech Technology and Health,5th Floor Damascus Road,Beirut,Lebanon	Lebanon	009611612500	zog_az@mctcro.com	Regional Manager
Scientific	Chebl Mourani	Hotel Dieu de France, Alfred Naccache Avenue, Achrafieh, Beirut, Lebanon	Lebanon	009613185632	cheblmourani@gmail.com	Principal Investigator
Scientific	Katia El Taoum	AUBMC	Lebanon	+9618822363	ke27@aub.edu.lb	Principal Investigator

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel dieu de france	Chebl Mourani	Pediatric nephrology	Approved
American University of Beirut Medical Center	Katia El Taoum	Pediatric	Pending

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	31/01/2023	Nancy Alam	nancy.alam@usj.edu.lb	+961 1 421 000 ext 2335

Countries of Recruitment

Name
United States of America

Health Conditions or Problems Studied

Condition	Code	Keyword
hyperkalaemia	Hyperkalaemia (E87.5)	hyperkalaemia

Interventions

Intervention	Description	Keyword
Patiromer for Cohort A (2 to <6 years of age)	The patiromer starting dose is based on Body Weight (Based on Outcomes and DSMB Recommendation).Titration during the study will depend on the potassium response of the subject.	Patiromer
Patiromer for Cohort B (0 to <2 years of age)	To ensure safety in Cohort B, dosing will begin with Cohort A (2 to <6 years of age). After 3 subjects from Cohort A have completed 4 weeks of treatment, the Data Safety and Monitoring Board (DSMB) will evaluate safety in these 3 subjects. If the safety profile is acceptable, enrolment will then proceed in Cohort B (0 to <2 years of age). The patiromer starting dose in Cohort B is based on body weight and DSMB recommendations, and it will be adapted during the study depending on the potassium response of the subject.	Patiromer

Primary Outcomes

Name	Time Points	Measure
change in potassium levels	from baseline to Day 28	blood potassium

Key Secondary Outcomes

Name	Time Points	Measure
change in potassium levels	from baseline to Day 3, Day 7, and Day 14 and end of the study Part 1 and during the optional safety-extension period.	blood potassium
treatment-emergent adverse events (TEAEs) and serious adverse events (SAEs)	Part 1: Day 1 up to end of treatment (Day 28 ±3 Days); Part 2: Day 1 up to follow-up visit (Up to 54 weeks)]	NA
vital signs, 12-lead ECG, and clinical safety laboratory evaluations	From baseline to Day 3, Day 7, Day 14, Day 28 and during Part 2: up to 52 weeks	Change in resting heart rate (beats per minute) in systolic blood pressure (mmHg) in diastolic blood pressure (mmHg) in body temperature (Celsius) Normal and abnormal 12-lead electrocardiogram (ECG) findings
occurrence of blood potassium	From baseline to Day 3, Day 7, Day 14, Day 28 and during Part 2: up to 52 weeks	blood potassium
Occurrence of blood magnesium	From baseline to Day 3, Day 7, Day 14, Day 28 and during Part 2: up to 52 weeks	blood magnesium
Occurrence of serum calcium, phosphate, fluoride, creatinine, bicarbonate, and blood urea nitrogen levels	From baseline to Day 3, Day 7, Day 14, Day 28 and during Part 2: up to 52 weeks	blood test

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