An Open-label Extension Study to Evaluate the Long-term Safety and Efficacy of Maralixibat in the Treatment of Subjects With Progressive Familial Intrahepatic Cholestasis (PFIC)

04/04/2025 12:34:30

Main Information

Primary registry identifying number
LBCTR2021034759

Protocol number
MRX-503

MOH registration number
NCT04185363

Study registered at the country of origin
No

Study registered at the country of origin: Specify
Study registered in clinicaltrials.gov

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency
04/12/2019

Primary sponsor
Mirum Pharmaceuticals Inc

Primary sponsor: Country of origin
California

Date of registration in primary registry
21/03/2021

Date of registration in national regulatory agency
04/12/2019

Public title
An Open-label Extension Study to Evaluate the Long-term Safety and Efficacy of Maralixibat in the Treatment of Subjects With Progressive Familial Intrahepatic Cholestasis (PFIC)

Acronym

Scientific title
An Extension Study of Maralixibat in Patients With Progressive Familial Intrahepatic Cholestasis (PFIC)

Acronym

Brief summary of the study: English
This is an open-label, multicenter, Phase 3 study to evaluate the long-term safety and efficacy of maralixibat in the treatment of pediatric subjects with PFIC.

Brief summary of the study: Arabic
هذه دراسة مفتوحة ، متعددة المراكز ، المرحلة 3 وفعالية maralixibat لتقييم السلامة على المدى الطويل في علاج الأطفال الذين يعانون من PFIC.

Health conditions/problem studied: Specify
Progressive Familial Intrahepatic Cholestasis (PFIC)

Interventions: Specify
Drug: Maralixibat
Dose, route, frequency: Subjects will receive maralixibat oral solution based on their individual body weight, up to 600 µg/kg BID.

Key inclusion and exclusion criteria: Inclusion criteria
1. Provide informed consent and assent (as applicable) per Institutional Review Board/Ethics Committee.
2. Completion of study MRX-502; treatment interruption between MRX-502 and MRX-503 should be avoided. Subjects who do not complete the study MRX-503 Baseline Visit (Day 0) on the same day as the study MRX-502 EOT Visit will be considered for participation in study MRX-503 only after discussion with the Medical Monitor.

3. Males and females of non-childbearing potential. Males and non-pregnant, non-lactating females of childbearing potential who are sexually active must agree to use acceptable contraception during the study through 30 days after the last dose of maralixibat.
4. Females of childbearing potential must have a negative urine pregnancy test at the Baseline Visit (Day 0).
5. Access to email or telephone for scheduled remote visits.
6. Ability to read and understand the questionnaires (both caregivers and subjects above the age of assent).
7. Access to consistent caregiver(s) during the study.
8. Subject and caregiver willingness to comply with all study visits and requirements.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

1

Key inclusion and exclusion criteria: Age maximum

18

Key inclusion and exclusion criteria: Exclusion criteria

1.Any female who is pregnant or lactating or who is planning to become pregnant.
2. Administration of prohibited medication between the MRX-502 EOT visit and the MRX-503 Baseline Visit (Day 0).
3. History of non-compliance in study MRX-502, non-adherence to medical regimens, unreliability, mental instability, or incompetence that could compromise the validity of informed consent or lead to non-adherence with the study protocol based on Investigator judgment.
4. Experienced an adverse event (AE) or serious adverse event (SAE) related to maralixibat during the MRX-502 study that led to permanent discontinuation of the subject from maralixibat.
5. Any other conditions or laboratory abnormalities that, in the opinion of the Investigator or Sponsor Medical Monitor, may compromise the safety of the subject, or interfere with the subject participating in or completing the study.
6. Cognitive impairment of the subject or caregiver that would, in the opinion of the Investigator, preclude appropriate understanding of study information and compliance with study procedures.

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

N/A

Study design: Masking

Open (masking not used)

Study design: Control

N/A

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

No

IMP has market authorization: Specify

Name of IMP

Maralixbat

Year of authorization

Month of authorization

Type of IMP

Others

Pharmaceutical class

Maralixibat is an inhibitor of the apical sodium-dependent bile acid transporter/ileal bile acid transporter/solute carrier family 10 (sodium/bile acid cotransporter family) member 2 (ASBT/IBAT/SLC10A2), a transmembrane protein localized on the luminal surface of ileal enterocytes.

Therapeutic indication

Progressive familial intrahepatic cholestasis (PFIC)

Therapeutic benefit

The overall safety, tolerability, and preliminary efficacy of maralixibat in ongoing and completed studies indicate that there is a positive benefit-to-risk profile for the treatment of rare pediatric cholestatic liver diseases.
Given the clinical outcomes associated with PFIC, including the negative impact on patients' and caregivers' quality of life, and the fact that there are currently no approved treatments, there is a high unmet medical need for a novel treatment for this disease.

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

Blood samples

Target sample size

90

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

01/05/2021

Date of study closure: Type

Anticipated

Date of study closure: Date

30/12/2022

Recruitment status

Other

Recruitment status: Specify

Enrolling by invitation

Date of completion

IPD sharing statement plan

No

IPD sharing statement description

The Sponsor and/or its representatives accessing the records and data will take all reasonable precautions in accordance with applicable laws, regulations, and guidelines to maintain the confidentiality of subjects' identities. Subjects are assigned a unique identifying number; however, their initials and date of birth may also be collected, if permitted under local laws governing privacy

Additional data URL

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
US NCT	NCT04185363

Sources of Monetary or Material Support

Name
Mirum Pharmaceuticals Inc. 950 Tower LaneFoster City, CA 94404

Secondary Sponsors

Name
N/A

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Hanen Hamid	City: Aley, Town: Bchamoun, Street: Yanar Street, Building 33, Ground Floor	Lebanon	+96181021910	Hanen.hamid@clinart.net	Clinart
Scientific	Adib Moukarzel	HDF	Lebanon	009613516060	adib.moukarzel@usj.edu.lb	Hotel Dieu du France

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hotel Dieu De France	Adib Moukarzel	Gastroenterology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hotel Dieu de France	30/03/2020	Nancy Choucair Alam	nancy.alam@usj.edu.lb	961 1 421 000 ext 2335

Countries of Recruitment

Name
Lebanon
Argentina
Colombia
United Kingdom
United States of America
Austria
Belgium
Brazil
Canada
France
Germany
Italy
Mexico
Poland
Singapore
Turkey
Hungary

Health Conditions or Problems Studied

Condition	Code	Keyword
Progressive Familial Intrahepatic Cholestasis	2-Propanol (T51.2)	(PFIC)

Interventions

Intervention	Description	Keyword
Maralixibat Chloride	Inhibitor of the apical sodium-dependent bile acid transporter/ileal bile acid transporter/solute carrier family 10 (sodium/bile acid cotransporter family) member 2 (ASBT/IBAT/SLC10A2)	Maralixibat

Primary Outcomes

Name	Time Points	Measure
Incidence of Treatment Emergent Adverse Events (TEAEs) during the study	changes from non-serious to serious	Severity of AE

Key Secondary Outcomes

Name	Time Points	Measure
Mean change from baseline over time in serum bile acid	Normalisation of sBA	sBA levels

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