A Randomized, Placebo-controlled, Phase 2 Study to Evaluate the Safety and Pharmacodynamics of Once-daily Oral IW-1701 in Patients with Stable Sickle Cell Disease

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Main Information

Primary registry identifying number
LBCTR2019091283

Protocol number
C1701-202

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

Primary sponsor
Cyclerion Therapeutics, Inc.

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
23/10/2019

Date of registration in national regulatory agency

Public title
A Randomized, Placebo-controlled, Phase 2 Study to Evaluate the Safety and Pharmacodynamics of Once-daily Oral IW-1701 in Patients with Stable Sickle Cell Disease

Acronym
STRONG SCD

Scientific title
A Randomized, Placebo-controlled, Phase 2 Study to Evaluate the Safety and Pharmacodynamics of Once-daily Oral IW-1701 in Patients with Stable Sickle Cell Disease

Acronym
STRONG SCD

Brief summary of the study: English

The primary objective of the C1701-202 STRONG SCD study is to evaluate the safety and tolerability of different dose levels of IW-1701 compared with placebo when administered daily for approximately 12 weeks to patients with stable sickle cell disease (SCD). Exploratory objectives include evaluation of pharmacokinetic (PK) as well as evaluation of the effect of IW-1701 on symptoms of SCD, health-related quality of life, and biomarkers of pharmacodynamic (PD) activity.

Brief summary of the study: Arabic

الهدف الأساسي من دراسة
C1701-202 STRONG SCD هو تقييم سلامة وتحمل مستويات جرعة مختلفة من
أسبوعاً تقريباً لدى مرضى داء الخلايا المنجلية المستقر. تشمل الأهداف الاستكشافية تقييم١٢مقارنة مع الدواء الارضائي عند تناوله يومياً لمدة
IW-1701 الحرانك الدوائية
وكذلك تقييم تأثير (PK)
IW-1701 على أعراض داء الخلايا المنجلية المستقر ونوعية الحياة المتعلقة بالصحة والمؤشرات الحيوية للنشاط الديناميكي الدوائي
.(PD)

Health conditions/problem studied: Specify
Stable sickle cell disease

Interventions: Specify

Eligible patients will be stratified by hydroxyurea (HU) use (yes or no) and randomly assigned in a 3:1 ratio to receive IW-1701 once daily or placebo.
Arm 1: IW-1701 (Olinciguat) -uptitration possible for patients who meet the conditions to begin taking the applicable higher dose.
Arm 2: placebo.

Key inclusion and exclusion criteria: Inclusion criteria

1. Patient is ambulatory male or female 16 to 70 years of age at the Screening Visit.

2. Patient has SCD, including HbSS, HbSC, HbSβ0-thalassemia, or HbSβ+-thalassemia, documented in their medical history

3. If patient is on medication(s) for SCD, such as hydroxyurea (HU), are on a stable regimen.

4. Per medical history and/or patient recall, patient has had at least 1 and no more than 10 sickle cell-related pain crises in the 12 months before the Screening Visit and none occurring in the 4 weeks before the Randomization Visit.

5. Women of childbearing potential must have a negative pregnancy test prior to randomization and must agree to use protocol-specified contraception from the Screening Visit through 90 days after the final dose of study drug.

6. Male patients must be surgically sterile by vasectomy (conducted ≥60 days before the Screening Visit or confirmed via sperm analysis) or must
agree to use protocol-specified contraception and agree to refrain from sperm donation from the Screening Visit through 90 days after the final dose of study drug.

7. Patient completes daily eDiary entries for at least 10 days during the last 14 days of the Run in Period as assessed at the Randomization Visit.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

16

Key inclusion and exclusion criteria: Age maximum

70

Key inclusion and exclusion criteria: Exclusion criteria

1. Patient requires a program of prescheduled, regularly administered chronic blood transfusion therapy.

2. Patient has been hospitalized for an SCD-related complication in the 4 weeks before the Randomization Visit.

3. Patient has taken opioid(s) >200 morphine mg equivalent/day within the 4 weeks before the Randomization Visit.

4. Patient is taking aspirin ≥325 mg daily, P2Y12 inhibitors, any anticoagulant medication, specific inhibitors of phosphodiesterase 5 (PDE5), nonspecific inhibitors of PDE5, moderate or strong cytochrome P450 3A (CYP3A) inhibitors, any supplements for the treatment of erectile dysfunction, riociguat, or nitrates or nitric oxide donors in any form.

5. Patient has major concurrent illness or medical condition that in the opinion of the Investigator would preclude participation in a clinical study.

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Safety

Trial scope: Specify scope

N/A

Study design: Allocation

Randomized controlled trial

Study design: Masking

Blinded (masking used)

Study design: Control

Placebo

Study phase

2

Study design: Purpose

Study design: Specify purpose

Treatment

N/A

Study design: Assignment

Parallel

Study design: Specify assignment

N/A

IMP has market authorization

No

IMP has market authorization: Specify

Name of IMP

IW1701/olinciguat

Year of authorization

Month of authorization

Type of IMP

Cell therapy

Pharmaceutical class

soluble guanylate cyclase (sgc) stimulator

Therapeutic indication

Stable sickle cell disease

Therapeutic benefit

There remains considerable unmet medical need in SCD, not only for treatments that prevent painful crises and other acute complications, but also for treatments that address the daily symptoms of the disease, including chronic pain.

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

Optional genotyping testing. If patient agrees, a blood sample of 4 mL will be collected and stored. The test may help to better understand how the disease and related diseases work, the effet of IW-1701 and/or other medications on the body, how IW-1701 is processed by the body, who might benefit from IW-1701 and why some people have side effects from taking the drug but other people don't.

Target sample size

88

Actual enrollment target size

88

Date of first enrollment: Type
Anticipated

Date of first enrollment: Date
18/11/2019

Date of study closure: Type
Anticipated

Date of study closure: Date
31/07/2020

Recruitment status
Pending

Recruitment status: Specify

Date of completion

IPD sharing statement plan
No

IPD sharing statement description
Not applicable

Additional data URL
https://www.clinicaltrials.gov/ct2/show/NCT03285178

Admin comments

Trial status
Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
ClinicalTrials.gov	NCT03285178

Sources of Monetary or Material Support

Name
Cyclerion Therapeutics, Inc.

Secondary Sponsors

Name
None

Contact for Public/Scientific Queries

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Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Hammoud Hospital University Medical Center	Dr. Wissam Houhou	Hematology and Oncology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Hammoud Hospital University Medical Center	16/09/2019	Ghada Aoun	medical@hammoudhospital.org	+961 7 723 111 Ext 1956

Countries of Recruitment

Name
Lebanon
United Kingdom
United States of America

Health Conditions or Problems Studied

Condition	Code	Keyword
sickle cell disease	Sickle-cell disorders (D57)	Sickle Cell Disease (SCD)

Interventions

Intervention	Description	Keyword
Arm 1	Placebo	Placebo
Arm 2	IW-1701	olinciguat

Primary Outcomes

Name	Time Points	Measure
Safety and tolerability	12 weeks	Incidence, frequency, and severity of TEAEs and study drug-related TEAEs

Key Secondary Outcomes

Name	Time Points	Measure
Hemodynamic Parameters	12 weeks	blood pressure and pulse
Pain Crisis Paramaters	12 weeks	Time to first pain crisis, proportion and frequency of pain crisis
Biomarkers	12 weeks	Biomarker concentration changes
Pharmacokinetic	12 weeks	Plasma concentrations
Patient-reported Outcomes	12 weeks	Patient Questionnaires

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