



# 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 تقييم مقارنة مع الدواء الإرضائي عند تناوله يومياً لمدة IW-1701 الحرائك الدوائية (PK) وكذلك تقييم تأثير 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, HbS $\beta$ 0-thalassemia, or HbS $\beta$ +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  $\geq$ 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  $\geq$ 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**

**Study design: Specify assignment**

Parallel

N/A

**IMP has market authorization**

**IMP has market authorization: Specify**

No

**Name of IMP**

**Year of authorization**

**Month of authorization**

IW1701/olinciguat

**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**

**Study model: Explain model**

N/A

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**

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 effect 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

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Dr. Gino Girardi	100 Brandywine Boulevard	United States of America	+1 919 418 5164	gino.girardi@syneoshealth.com	Syneos Health (previously INC Research)
Scientific	Dr. Wissam Houhou	Ghassan Hammoud Street, Saida	Lebanon	+961 70 874 770	dr_houhou_wissam@hotmail.com	Hammoud Hospital University Medical Center
Scientific	Dr. Adlette Inati	El Maarad Street, Tripoli	Lebanon	+961 3 228 033	adlette.inati@lau.edu.lb	Nini Hospital
Scientific	Dr. Ali Taher	Cairo Street, Beirut	Lebanon	+961 3 755 669	ataher@aub.edu.lb	American University of Beirut Medical Center

## 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 Parameters	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**