

### 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

MOH registration number

Study registered at the country of origin

Type of registration

Prospective

Date of registration in national regulatory agency

**Primary sponsor** 

Cyclerion Therapeutics, Inc.

Date of registration in primary registry

27/03/2021

**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

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

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

C1701-202 STRONG SCD هو تقييم سلامة وتحمل مستويات جرعة مختلفة من أسبوعاً تقريباً لدى مرضى داء الخلايا المنجلية المستقر. تشمل الأهداف الاستكشافية تقبيم ٢ امقارنة مع الدواء الارضائي عند تناوله يومياً لمدة 1701-W الحر ائك الدو ائية (PK) وكذلك تقييم تَأثير IW-1701 على أعراض داء الخلايا المنجلية المستقر ونوعية الحياة المتعلقة بالصحة والمؤشرات الحيوية للنشاط الديناميكي الدوائي

Health conditions/problem studied: Specify

Stable sickle cell disease

#### Protocol number

C1701-202

Study registered at the country of origin: Specify

Type of registration: Justify

N/A

Primary sponsor: Country of origin

United States of America

Date of registration in national regulatory agency

Acronvm

STRONG SCD

Acronym

STRONG SCD



#### 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

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

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

Key inclusion and exclusion criteria: Gender

Key inclusion and exclusion criteria: Specify gender

Both

Key inclusion and exclusion criteria: Age minimum

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

Pharmaceutical

Type of intervention

Type of intervention: Specify type

N/A

Trial scope

Trial scope: Specify scope

Safety

N/A

Study design: Allocation Randomized controlled trial Study design: Masking Blinded (masking used)

Study design: Control

Study phase 2

Placebo

Study design: Purpose

Study design: Specify purpose



Treatment

Study design: Assignment

Parallel

IMP has market authorization

Nο

Name of IMP

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

N/A

Study model: Specify model

N/A

Time perspective

IN/A

Time perspective: Specify perspective

N/A

Target follow-up duration

Number of groups/cohorts

Biospecimen retention

Samples with DNA\*\*

Target sample size

88

N/A

Study design: Specify assignment

N/A

IMP has market authorization: Specify

Year of authorization

Month of authorization

Study model: Explain model

Time perspective: Explain time perspective

N/A

N/A

Target follow-up duration: Unit

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.

Actual enrollment target size

88



Date	of	first	enrollment:	Type
Date	vı	mot	emomment.	IVDE

Anticipated

Date of study closure: Type

Anticipated

Recruitment status

Complete

Date of completion

22/07/2020

IPD sharing statement plan

No

Date of first enrollment: Date

18/11/2019

Date of study closure: Date

20/01/2021

**Recruitment status: Specify** 

IPD sharing statement description

Not applicable

Additional data URL

https://www.clinicaltrials.gov/ct2/show/NCT03285178

Admin comments

**Trial status** 

Approved

ClinicalTrials.gov

Secondary Identifying Numbers				
Full name of issuing authority	Secondary identifying number			

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@syn eoshealth.com	Syneos Health (previsouly INC Research)
Scientific	Dr. Wissam Houhou	Ghassan Hammoud Street, Saida	Lebanon	+961 70 874 770	dr_houhou_wiss am@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	
Nini Hospital	Dr. Adlette Inati	Pediatric Hematology 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	
Nini Hospital	25/09/2019	Sarah Kharsa	sarah.kharsa@hopitalnini.com	+961 6 431 400 Ext 452	

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	