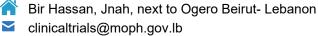
**REPUBLIC OF LEBANON MINISTRY OF PUBLIC HEALTH** Lebanon Clinical Trials Registry

#### Study of Two Doses of Crizanlizumab Versus Placebo in Adolescent and Adult Sickle Cell Disease Patients (STAND)

12/08/2025 01:04:48

Primary registry identifying number	Protocol number
BCTR2019060244	CSEG101A2301
NOH registration number	
24334/2019	
Study registered at the country of origin	Study registered at the country of origin: Specify
Yes	
Type of registration	Type of registration: Justify
Prospective	N/A
Date of registration in national regulatory agency	
Primary sponsor	Primary sponsor: Country of origin
Novartis Pharma Services Inc.	Novartis Pharmaceuticals
Date of registration in primary registry	Date of registration in national regulatory agency
22/12/2022	
Public title	Acronym
Study of Two Doses of Crizanlizumab Versus Placebo in Adolescent and Adult Sickle Cell Disease Patients (STAND)	
Scientific title	Acronym
A Phase III, Multicenter, Randomized, Double-blind Study to Assess Efficacy and Safety of Two Doses of Crizanlizumab Versus Placebo, With or Without Hydroxyurea/ Hydroxycarbamide Therapy, in Adolescent and Adult Sickle Cell Disease Patients With Vaso- Occlusive Crises (STAND)	
Brief summary of the study: English	
The purpose of this study is to compare the efficacy and safety of 2 doses of crizanlizumab (5.0 mg/kg and 7.5 mg/kg) versus placebo in adolescent and adult sickle cell disease (SCD) patients with history of vaso-occlusive crisis (VOC) leading to healthcare visit.	
Brief summary of the study: Arabic	
، عشوانيَّة التوزيع ومزدوجة التعمية لتقييم فعاليَّة وسلامة جرعتين من دواء كريز انليزوماب مقابل الدواء سي يوريا / هيدروكسي كاربامايد، لدى المرضى المراهقين والبالغين المصابين بداء الكريات المنجليَّة مع (STAND) نوبات انسداد وعانيً	دراسة مرحلة ثالثة، متعددة المراكز الوهمي، مع أو بدون علاج هيدروك
Health conditions/problem studied: Specify	
Sickle Cell Disease	
Interventions: Specify	
•Drug: Crizanlizumab (SEG101) Crizanlizumab will be supplied in single use 10 mL glass vials at a concen is a concentrate for solution for infusion IV.	tration of 10 mg/mL. One vial contains 100 mg of crizanlizumab. This



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Key inclusion and exclusion criteria: Specify gender

•Drug: Placebo

Placebo will be supplied in single use 10 mL glass vials at a concentration of 10 mg/mL. One vial contains 100 mg of placebo. This is a concentrate for solution for infusion IV.

#### Key inclusion and exclusion criteria: Inclusion criteria

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1.Written informed consent must be obtained prior to any screening procedures

2.Male or female patients aged 12 years and older on the day of signing informed consent. Adolescent include patients aged 12 to 17 years old and adults ≥ 18 years and older

3.Confirmed diagnosis of SCD by hemoglobin electrophoresis or high performance liquid chromatography (HPLC) [performed locally]. All SCD genotypes are eligible, genotyping is not required for study entry

4.Experienced at least 2 VOCs leading to healthcare visit within the 12 months prior to screening visit as determined by medical history. Prior VOC leading to healthcare visit must include:

a.Pain crisis defined as an acute onset of pain for which there is no other medically determined explanation other than vaso- occlusion - b.a visit to a medical facility and/or healthcare professional,

c.and receipt of oral/parenteral opioids or parenteral nonsteroidal anti-inflammatory drug (NSAID) analgesia As well as other complicated crises, such as acute chest syndrome, priapism, and hepatic or splenic sequestration

5.If receiving HU/HC or erythropoietin stimulating agent or L-glutamine, must have been receiving the drug for at least 6 months prior to Screening visit and plan to continue taking at the same dose and schedule until the subject has reached one year of study treatment

6.Patients must meet the following central laboratory values at the screening visit:

∘Absolute Neutrophil Count ≥1.0 x 109/L

∘Platelet count ≥75 x 109/L

∘Hemoglobin: for adults (Hb) ≥4.0 g/dL and for adolescents (Hb) ≥5.5 g/dL

∘Glomerular filtration rate ≥ 45 mL/min/1.73 m2 using CKD-EPI formula in adults, and Shwartz formula in adolescents

∘Direct (conjugated) bilirubin < 2.0 x ULN

∘Alanine transaminase (ALT) < 3.0 x ULN

7.ECOG performance status ≤2.0 for adults and Karnofsky ≥ 50% for adolescents

#### Key inclusion and exclusion criteria: Gender

 Both
 Key inclusion and exclusion criteria: Age minimum
 Key inclusion and exclusion criteria: Age maximum

 12
 99

#### Key inclusion and exclusion criteria: Exclusion criteria

1. History of stem cell transplant.

 Participating in a chronic transfusion program (pre-planned series of transfusions for prophylactic purposes) and/or planning on undergoing an exchange transfusion during the duration of the study; episodic transfusion in response to worsened anemia or VOC is permitted.
 Contraindication or hypersensitivity to any drug or metabolites from similar class as study drug or to any excipients of the study drug formulation. History of severe hypersensitivity reaction to other monoclonal antibodies, which in the opinion of the investigator may pose an increased risk of serious infusion reaction.

4. Received active treatment on another investigational trial within 30 days (or 5 half-lives of that agent, whichever is greater) prior to Screening visit or plans to participate in another investigational drug trial.

5.Women of child-bearing potential, defined as all women physiologically capable of becoming pregnant unless they are using highly effective methods of contraception during dosing and for 15 weeks after stopping treatment.

6.Concurrent severe and/or uncontrolled medical conditions which, in the opinion of the Investigator, could cause unacceptable safety risks or compromise participation in the study.

7. History or current diagnosis of ECG abnormalities indicating significant risk of safety such as:

∘Resting QTcF ≥470 msec at pretreatment (baseline) for both male and female or inestability to determine QTc

•Concomitant clinically significant cardiac arrhythmias (e.g ventricular tachycardia), and clinically significant second or third degree AV block without a pacemaker

•History of familial long QT syndrome or know family history of Torsades de Pointes

8.Not able to understand and to comply with study intructions and requirements.

#### Type of study Interventional Type of intervention Pharmaceutical Trial scope

Safety

Type of intervention: Specify type N/A

Trial scope: Specify scope N/A

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Study design: Allocation	Study design: Masking	
Randomized controlled trial	Blinded (masking used)	
Study design: Control	Study phase	
Placebo	3	
Study design: Purpose	Study design: Specify purpose	)
Prevention	N/A	
Study design: Assignment	Study design: Specify assignm	nent
Parallel	N/A	
IMP has market authorization	IMP has market authorization:	Specify
No		
Name of IMP	Year of authorization	Month of authorization
SEG101 - Crizanlizumab		
Type of IMP		
Immunological		
Pharmaceutical class		
anti-human P-selectin antibody G1		
Therapeutic indication		
prevention of vaso-occlusive crises (VOCs) in patients of all genotypes with	sickle cell disease (SCD)	
Therapeutic benefit		
To compare the efficacy of 5.0 mg/kg versus placebo and 7.5 mg/kg of crize	anlizumab versus placebo	
in addition to standard of care. To compare the efficacy of 7.5 mg/kg versus placebo on the annualized ra	te of all VOCs (managed at	
home + leading to healthcare visit), based on documentation by health care with participant.		
To compare the efficacy of 5.0 mg/kg versus placebo on the annualized rate home + leading to healthcare visit)	e of all VOCs (managed at	
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	1
Number of groups/cohorts		

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Biospecimen retention Samples without DNA	<b>Biospecimen description</b> All Blood samples and Urine Samples will be shipped to Covance Geneva Central Lab
Target sample size	Actual enrollment target size 11
Date of first enrollment: Type Actual	Date of first enrollment: Date 07/08/2019
Date of study closure: Type Actual	Date of study closure: Date 29/07/2027
Recruitment status Complete	Recruitment status: Specify
Date of completion 24/06/2021	
IPD sharing statement plan	IPD sharing statement description
No	Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations.
Additional data URL	This trial data availability is according to the criteria and process described on www.clinicalstudydatarequest.com
https://clinicaltrials.gov/ct2/show/record/NCT03814746?term=cseg101a230	)1&rank=1
Admin comments	
Trial status	

Approved

Secondary Identifying Numbers		
Full name of issuing authority	Secondary identifying number	
Clinicaltrials.gov	NCT03814746	

#### **Sources of Monetary or Material Support**

#### Name

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Novartis Pharma Services Inc.



#### **Secondary Sponsors**

Name

NA

Contac	Contact for Public/Scientific Queries					
Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Adlette Inati	Tripoli	Lebanon	03228033	adlette.inati@lau. edu.lb	Nini Hospital
Scientific	Hind Khairallah	Sin El Fil	Lebanon	+961 1 512002 Ext. 271	Hind.Khairallah@ fattal.com.lb	Khalil Fattal et Fils s.a.l.
Public	Miguel Abboud	Beirut	Lebanon	03534213	ma56@aub.edu.l b	American University of Beirut Medical Center

Centers/Hospitals Involved in the Study			
Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Nini Hospital	Adlette Inati	Hematology	Approved
AUBMC	Miguel Abboud	Hematology	Approved

Ethics Review				
Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Nini Hospital	20/05/2019	Nabil Kabbara	Nabil.kabbara@hopitalnini.com	+961 (0) 6 431 400 ext 1062
American University of Beirut Medical Center	30/12/2019	Fuad Ziyadeh	fz05@aub.edu.lb	+961 (0) 1 350 000 ext:5445



Countries of Recruitment
Name
Lebanon
Belgium
Netherlands
United Kingdom
United States of America
Jordan

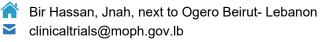
Health Conditions or Problems Studied		
Condition Code Keyword		Keyword
Sickle Cell Disease	Sickle-cell disorders (D57)	SCD

Interventions		
Intervention	Description	Keyword
ICF, Lab tests, IMP , Questionnaires	ICF, Lab tests, IMP , Questionnaires	ICF, Lab tests, IMP , Questionnaires

#### **Primary Outcomes**

Name	Time Points	Measure	
Rate of vaso-occlusive crisis (VOC) events leading to	1 year	1 year	
To compare the efficacy of 5.0 mg/kg versus placebo and 7.5 mg/kg of crizanlizumab versus placebo in addition to standard of care		1 year	

## Key Secondary Outcomes Name Time Points Measure •Rate of all VOCs leading to healthcare visit and treated at home 1 year, 5 years 1 year, 5 years •Number of days with VOC leading to healthcare visit 1 year 1 year





# Trial Results Summary results Study results globally Date of posting of results summaries 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