



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

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## Main Information

**Primary registry identifying number**

LBCTR2019060244

**Protocol number**

CSEG101A2301

**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**

Novartis Pharma Services Inc.

**Primary sponsor: Country of origin**

Novartis Pharmaceuticals

**Date of registration in primary registry**

16/07/2019

**Date of registration in national regulatory agency**

**Public title**

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

**Acronym**

**Scientific title**

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)

**Acronym**

**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**

دراسة مرحلة ثالثة، متعددة المراكز، عشوائية التوزيع ومزدوجة التعمية لتقييم فعالية وسلامة جرعتين من دواء كريزانليزوماب مقابل الدواء الوهمي، مع أو بدون علاج هيدروكسي يوريا / هيدروكسي كارباميد، لدى المرضى المراهقين والبالغين المصابين ببدء الكريات المنجلية مع نوبات انسداد وعائي (STAND)

**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 concentration of 10 mg/mL. One vial contains 100 mg of crizanlizumab. This is a concentrate for solution for infusion IV.

Other Name: SEG101





•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**

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  $\geq 18$  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  $\geq 1.0 \times 10^9/L$
  - Platelet count  $\geq 75 \times 10^9/L$
  - Hemoglobin: for adults (Hb)  $\geq 4.0$  g/dL and for adolescents (Hb)  $\geq 5.5$  g/dL
  - Glomerular filtration rate  $\geq 45$  mL/min/1.73 m<sup>2</sup> using CKD-EPI formula in adults, and Schwartz formula in adolescents
  - Direct (conjugated) bilirubin  $< 2.0 \times ULN$
  - Alanine transaminase (ALT)  $< 3.0 \times ULN$
7. ECOG performance status  $\leq 2.0$  for adults and Karnofsky  $\geq 50\%$  for adolescents

**Key inclusion and exclusion criteria: Gender**

Both

**Key inclusion and exclusion criteria: Specify gender**

**Key inclusion and exclusion criteria: Age minimum**

12

**Key inclusion and exclusion criteria: Age maximum**

99

**Key inclusion and exclusion criteria: Exclusion criteria**

1. History of stem cell transplant.
2. 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.
3. 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  $\geq 470$  msec at pretreatment (baseline) for both male and female or instability 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 instructions and requirements.

**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: Control**

Placebo

**Study design: Purpose**

Prevention

**Study design: Assignment**

Parallel

**IMP has market authorization**

No

**Name of IMP**

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 crizanlizumab versus placebo in addition to standard of care.

To compare the efficacy of 7.5 mg/kg versus placebo on the annualized rate of all VOCs (managed at home + leading to healthcare visit), based on documentation by health care provider following contact with participant.

To compare the efficacy of 5.0 mg/kg versus placebo on the annualized rate of all VOCs (managed at home + leading to healthcare visit)

**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 without DNA

**Biospecimen description**

All Blood samples and Urine Samples will be shipped to Covance Geneva Central Lab

**Target sample size**

10

**Actual enrollment target size****Date of first enrollment: Type**

Anticipated

**Date of first enrollment: Date**

28/08/2019

**Date of study closure: Type**

Anticipated

**Date of study closure: Date**

26/02/2021

**Recruitment status**

Pending

**Recruitment status: Specify****Date of completion**

30/06/2020

**IPD sharing statement plan**

No

**IPD sharing statement description**

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.

This trial data availability is according to the criteria and process described on [www.clinicalstudydatarequest.com](http://www.clinicalstudydatarequest.com)

**Additional data URL**

<https://clinicaltrials.gov/ct2/show/record/NCT03814746?term=cseg101a2301&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
Novartis Pharma Services Inc.



## Secondary Sponsors

Name

NA

## 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.

## Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Nini Hospital	Adlette Inati	Hematology	Approved

## Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Nini Hospital	20/05/2019	Nabil Kabbara	Nabil.kabbara@hospitalnini.com	+961 (0) 6 431 400 ext 1062

## Countries of Recruitment

Name

Lebanon

Belgium

Netherlands

United Kingdom

United States of America

Jordan



## Health Conditions or Problems Studied

Condition	Code	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	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 first journal publication of results**

**Results URL link**

**Baseline characteristics**

**Participant flow**

**Adverse events**

**Outcome measures**

**URL to protocol files**