Study of Dose Confirmation and Safety of Crizanlizumab in Pediatric Sickle Cell Disease Patients

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Main Information

Primary registry identifying number
LBCTR2019020198

Protocol number
SEG101B2201

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
02/10/2019

Date of registration in national regulatory agency

Public title
Study of Dose Confirmation and Safety of Crizanlizumab in Pediatric Sickle Cell Disease Patients

Acronym

Scientific title
A Phase 2,Multicenter,Open-Label Study to Assess Appropriate Dosing and to Evaluate Safety of Crizanlizumab,With or Without Hydroxyurea/Hydroxycarbamide,in Sequential,Descending Age Groups of Pediatric Sickle Cell Disease Patients With Vaso-Occlusive Crisis

Acronym

Brief summary of the study: English
The purpose of the Phase 2 CSEG101B2201 study is to confirm and to establish appropriate dosing and to evaluate the safety in pediatric patients ages 6 months to <18 years with a history of VOC with or without HU/HC, receiving crizanlizumab for 2 years. The efficacy and safety of crizanlizumab was already demonstrated in adults with sickle cell disease. The approach is to extrapolate from the PK/pharmacodynamics (PD) already established in the adult population. The study is designed as a Phase II, multicenter, open-label study.

Brief summary of the study: Arabic
دراسة مفتوحة اللصاقة، متعددة المراكز، في المرحلة الثانية لتقييم الجرعات المناسبة وسلامة دواء كريزانليزوماب، مع أو بدون هيدروكسي يوريا / هيدروكسيكاربامايد، لدى مرضى مصابين بداء الكريات المنجليّة لدى الأطفال مع نوبة انسداد وعائيّ من فئات عمريّة تسلسليّة وتنازليّة

Health conditions/problem studied: Specify
Sickle Cell Disease

Interventions: Specify
Drug: Crizanlizumab
Crizanlizumab (SEG101) is a concentrate for solution for infusion, i.v. use. Supplied in single use 10 mL vials at a concentration of 10 mg/mL.

One vial contains 100 mg of crizanlizumab.

Other Name: SEG101

Key inclusion and exclusion criteria: Inclusion criteria

•Male or female patients aged 2 to <18 years (Group 3 will be expanded to allow enrolment of patients aged 6 to <24 months (and at least 6 kg) in Part B once the appropriate dose is confirmed in 2 to <6 year old patients)
•Confirmed diagnosis of sickle cell disease (SCD) (e.g. any genotype including HbSS, HbSC, HbSβ0-thalassemia, HbSβ+-thalassemia, and others) by hemoglobin electrophoresis or high-performance liquid chromatography (HPLC) performed locally.

•Experienced at least 1 VOC within the preceding 12 months, as determined by medical history. Prior VOC must have resolved at least 7 days prior to the first dose in the study and should include all the following:
1.the occurrence of appropriate symptoms (see VOC definition in protocol Section 7.2.1.1)
2.either a visit to a medical facility or healthcare professional,
3.receipt of oral/parenteral opioid or other non-opioid parenteral analgesia.

•If receiving HU/HC or erythropoietin stimulating agent, must have been receiving the drug for at least 6 months prior to Screening and plan to continue taking at the same dose and schedule during the trial. Dose alterations of HU/HC during Part A are not allowed, and if this occurs, the patient will enter directly to the Part B.
•Received standard age-appropriate care for SCD, including penicillin prophylaxis, pneumococcal immunization, and parental education
•Transcranial Doppler (TCD) considered low risk within the past 6 months (for 2 to 16 years).

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

1

Key inclusion and exclusion criteria: Age maximum

18

Key inclusion and exclusion criteria: Exclusion criteria

•History of stem cell transplant.
•Received any blood products within 30 days of Day 1 dosing.
•Participating in a chronic transfusion program (preplanned series of transfusions for prophylactic purposes).
•Patients with bleeding disorders
•Planning on undergoing an exchange transfusion during the duration of the study. Patients requiring episodic transfusion in response to worsened anemia or VOC are permitted.
•Contraindication or hypersensitivity to any drug from similar class as study drug or to any excipients of the study drug formulation.
•Received a monoclonal antibody or immunoglobulin-based therapy within 6 months of Screening, or has documented immunogenicity to a prior monoclonal antibody.
•Received active treatment on another investigational trial within 30 days (or 5 half lives of that agent, whichever is greater) prior to Screening or plans to participate in another investigational drug trial.
•Pregnant females or females who have given birth within the past 90 days or who are breastfeeding.
•Any documented history of a stroke or intracranial hemorrhage, or an uninvestigated neurologic finding within the past 12 months
•Any conditional TCD within the past 12 months
•Use of therapeutic anticoagulation (prophylactic doses permitted) or antiplatelet therapy (other than aspirin) within the 10 days prior to Week 1 Day 1 dosing
•Hospitalized at Screening
•Planning to undergo a major surgical procedure during the duration of the study
•Planning to initiate or terminate HU/HC while on study, other than for safety reasons
•Patient with active HIV infection (detectable viral load)
•Patients with known active Hepatitis B infection.
•Patients with known Hepatitis C history.
•Significant active infection or immune deficiency (including chronic use of immunosuppressive drugs) in the opinion of the investigator.
•Malignant disease. Exceptions to this exclusion include the following: malignancies that were treated curatively and have not recurred within 2 years prior to study treatment; any completely resected carcinoma in situ.
•Has a serious mental or physical illness, which, in the opinion of the Investigator would compromise participation in the study.
•Resting QTcF ≥450 msec at pretreatment (baseline) for patients under 12 years of age and ≥450 msec for males and ≥460 msec for female patients 12 years and older.
•Cardiac or cardiac repolarization abnormality
•Long QT syndrome, family history of idiopathic sudden death or congenital long QT syndrome
•Sexually active females who are unwilling to comply with reliable method of birth control until 15 weeks following last dose of study drug.

•Current drug or alcohol abuse:
1.Has a positive qualitative urine drug test at Screening for cocaine, phencyclidine (PCP), or amphetamines (opioids are permitted).
2.Consumes >12 (for males) or >8 (for females) standard alcoholic beverages per week.

•Not able to understand and to comply with study instructions and requirements.
•Subjects, who are an employee of the sponsor or investigator or otherwise dependent on them.
•Subjects, who are committed to an institution by virtue of an order issued either by the judicial or the administrative authorities.

Type of study

Interventional

Type of intervention
Pharmaceutical

Type of intervention: Specify type
N/A

Trial scope
Other

Trial scope: Specify scope

Study design: Allocation
N/A: Single arm study

Study design: Masking
Open (masking not used)

Study design: Control
N/A

Study phase
2

Study design: Purpose
Prevention

Study design: Specify purpose
N/A

Study design: Assignment
Single

Study design: Specify assignment
N/A

IMP has market authorization
No

IMP has market authorization: Specify

Name of IMP
SEG101 - Crizanlizumab

Year of authorization

Month of authorization

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
1.Number of Vaso Occusive Crisis (VOC) events leading to healthcare visit in clinic/ER/hospital
2.Number of Vaso Occusive Crisis (VOC) events treated at home
3.Number of each subcategory of VOC event (uncomplicated pain crisis, acute chest syndrome, hepatic sequestration, splenic sequestration, priapism)

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

5

Actual enrollment target size

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

21/10/2019

Date of study closure: Type

Anticipated

Date of study closure: Date

20/07/2022

Recruitment status

Recruiting

Recruitment status: Specify

Date of completion

31/07/2020

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 expert 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 is currently available according to the process described on www.clinicalstudydatarequest.com.

Additional data URL

https://clinicaltrials.gov/ct2/show/NCT03474965?term=seg101&rank=2

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
Clinical Trials. gov	NCT03474965

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	009613228033	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	Dr Adlette Inati	Hematology	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Nini Hospital	17/12/2018	Nabil Kabbara	Nabil.kabbara@hopitalnini.com	+961 (0) 6 431 400 ext 1062

Countries of Recruitment

Name
Lebanon
Belgium
Germany
India
United States of America
Canada
Colombia
France
Italy
Oman
Spain
Switzerland
Turkey
United Kingdom

Health Conditions or Problems Studied

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

Interventions

Intervention	Description	Keyword
Please refer to table 7- 1 in the attached Protocol V00, page 51Consenting process, Physical Exam, Vital Signs, Medical History, Lab assessments, efficacy assessments and Biomarker assessments, pharmacokinetics and immunogenicity	Consenting process, Physical Exam, Vital Signs, Medical History, Lab assessments, efficacy assessments and Biomarker assessments, pharmacokinetics and immunogenicity	ICF, IMP, Lab

Primary Outcomes

Name	Time Points	Measure
PK (AUCd15) after 1st dose, Confirm appropriate dosing of crizanlizumab in patients aged 2 to < 18 years	15 days	15 days
•PK (AUCtau) after 5th dose	week 15	week 15
•Frequency of any adverse events (AEs) as a measure of safety and tolerability	6 months , 2 years	6 months , 2 years

Key Secondary Outcomes

Name	Time Points	Measure
•Number of Vaso Occusive Crisis (VOC) events leading to healthcare visit in clinic/ER/hospital	6 months, 2 years	6 months, 2 years
•Number of Vaso Occusive Crisis (VOC) events treated at home (based on documentation by health care provider following phone contact with the patient)	6 months, 2 years	6 months, 2 years
•Number of hospitalizations and ER visits (both overall and VOC-related)	6 months, 2 years	6 months, 2 years
•Absolute change from baseline in hemoglobin	Baseline, 6 months, 2 years	Baseline, 6 months, 2 years

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