NOVARTIS

## Clinical Trial Results Summary

# A clinical trial in India to learn more about the safety of crizanlizumab in people with sickle cell disease who also had painful episodes (vaso-occlusive crises)

# Thank you!

Thank you to the participants who took part in the clinical trial for **sickle cell disease with painful episodes**. Every participant helped the researchers learn more about the trial drug **SEG101**, also called **crizanlizumab**.

Novartis sponsored this trial and believes it is important to share what was learned from the results of this trial with the participants and the public. We hope this helps the participants understand their important role in medical research.

## Trial information

**Trial number:** CSEG101A2403

**Novartis drug studied:** **SEG101**, also called **crizanlizumab**

**Sponsor:** Novartis

If you were a participant and have any questions about the results, please talk to the doctor or staff at the trial site.

This summary only shows the results of a single clinical trial. Other clinical trials may have different results.

# What was the main purpose of this trial?

The purpose of this trial was to learn about the safety of **crizanlizumab** in people with **sickle cell disease** in India who also had **painful episodes** requiring a hospital visit. This trial was done to fulfill the regulatory requirements to confirm the safety of **crizanlizumab**, specifically in the Indian population with this condition.

Regular red blood cells are flexible and round so that they can travel through small blood vessels to deliver oxygen to various parts of the body. **Sickle cell disease** is a genetic condition, that is passed down from parents, causing red blood cells to become stiff and shaped like a crescent or sickle. This makes it difficult for the blood cells to pass through small blood vessels. Eventually, blood cells stick to the blood vessels and block them, causing severe painful episodes, which are also called **vaso-occlusive crises (VOCs)**.

**Crizanlizumab** may help prevent **VOCs** in people with **sickle cell disease**. It works by blocking a protein, called **P-selectin**, which is present on blood cells and blood vessels and causes them to stick together. **Crizanlizumab** is approved in some countries, including India, to reduce the number of **VOCs** in adults and adolescents aged 16 years and older with **sickle cell disease**.

**Trial drug**
**SEG101** also called crizanlizumab
**Pronounced as**
criz-AN-liz-U-mab

## The trial's purpose was to answer this main question:

- What medical problems, also called adverse events, happened during this trial?
  - An **adverse event** is any sign or symptom that participants have during a trial. Adverse events **may** or **may not** be caused by treatments in the trial.

## How long was this trial?

The trial began in July 2021 and ended in February 2024. Individual participants were in this trial for around 1 year and 4 months including one year of treatment with the study drug and about 4 months of follow-up.

The researchers completed this trial as planned. When the trial ended, the researchers collected information from participants and created a report of the trial results. This summary is based on that report.

## Who was in this trial?

140 participants with **sickle cell disease who had VOCs** received treatment in this trial – 75 men and 65 women. Participants' ages ranged from 16 to 97 years, including 23 participants between 16 and less than 18 years of age. The average age of participants was 26 years.

All the participants were from India and were Asian.

The participants could take part in this trial if they:

- were at least 16 years of age
- had a confirmed diagnosis of **sickle cell disease** and at least 1 **VOC** for which they had to visit a hospital
- were at least able to do self-care activities

## What treatment did the participants receive?

The treatment in this trial was **crizanlizumab**.

All participants received 5 milligrams of **crizanlizumab** per kilogram of their body weight as an infusion into a vein. Participants received the treatment on the first day of weeks 1 and 3 followed by once every 4 weeks for up to a year or until they stopped participating in the trial for any reason.

The participants, researchers, and trial staff knew what treatment the participants were receiving.

# What happened during this trial?

**Before treatment** **Up to 1 month**

The trial staff checked to make sure the participants could be in this trial.

**During treatment** **Up to 1 year**

140 participants received **crizanlizumab** for up to a year or until they stopped participating in the trial for any reason.

**After treatment** **Up to 15 weeks**

Trial staff checked participants' general health and for any medical problems for up to 15 weeks after the participants' last dose of the trial treatment.

# What was the main result of this trial?

## What medical problems, also called adverse events, happened during this trial?

Trial doctors keep track of all medical problems, also called **adverse events**, that happen in trials. They track adverse events even if they think the adverse events are not related to the trial treatments.

Many trials are needed to know if a drug or treatment causes an adverse event.

This section is a summary of the adverse events that happened from the start of treatment until 15 weeks after the last dose of the trial treatment.

An **adverse event** is:

- Any **sign or symptom** that the participants have during a trial
- Considered **serious** when it is life-threatening, causes lasting problems, the participant needs hospital care, or results in death

Adverse events **may** or **may not** be caused by treatments in the trial.

53 of 140 participants had adverse events. 3 participants had adverse events that were considered serious that led to their deaths. These serious adverse events were not considered related to the trial treatment by the trial doctors. The researchers concluded there were no new safety concerns for **crizanlizumab** in this trial.

## How many participants had adverse events?

| | **Crizanlizumab** 140 participants |
|---|---|
| Had at least 1 **adverse event** | **53 of 140** 38% |
| Had at least 1 **serious adverse event** | **3 of 140** 2% |
| **Died** | **3 of 140** 2% |

## What serious adverse events did the participants have?

3 out of 140 (2%) participants, had serious adverse events and died as a result of these events during the trial. None of these events were considered to be related to the use of **crizanlizumab**.

The table below shows the serious adverse events that happened during the trial.

| | **Crizanlizumab** 140 participants |
|---|---|
| **Death due to sudden stopping of the heart** Sudden cardiac death | **1 of 140** Less than 1% |
| **Road traffic accident** | **1 of 140** Less than 1% |
| **Blood clot in a blood vessel in the lungs** Pulmonary embolism | **1 of 140** Less than 1% |

## What other adverse events did the participants have?

The table below shows the other adverse events that happened in **3% or more** participants. Additional adverse events happened in fewer participants.

| | **Crizanlizumab** 140 participants |
|---|---|
| **Pain** | **10 of 140** 7% |
| **Fever** Pyrexia | **8 of 140** 6% |
| **Joint Pain** Arthralgia | **6 of 140** 4% |
| **Low number of red blood cells** Anemia | **5 of 140** 4% |

# What was the other result of this trial?

## What adverse events of special interest did the participants have?

To learn this, researchers tracked how many participants had **adverse events of special interest** after receiving **crizanlizumab**.

18 out of 140 (13%) participants had at least one adverse event of special interest during the trial.

**Adverse events of special interest** are medical problems that have been identified as events that should be monitored more closely for a drug.

The table below shows all the adverse events of special interest that happened during the trial. A participant may have more than one event during the trial.

| | Crizanlizumab 140 participants |
|---|---|
| **Serious and rare medical event** (Death due to sudden stopping of the heart) | **1 of 140** Less than 1% |
| **Infections** (Infection of the nose and throat, eyes, gums, chest, part of the throat, liver; chickenpox) | **10 of 140** 7% |
| **Pain events** (Stomach pain, left eye pain) | **2 of 140** 1% |
| **Infusion-related reactions, standard search*** (Stomach pain, hives) | **2 of 140** 1% |
| **Infusion-related reactions, new combined search*** (Stomach pain, left eye pain, hives) | **3 of 140** 2% |

*Researchers used special search methods to categorize these events out of all the adverse events that happened during the trial. The standard search included the most common medical problems participants experienced on the day of the trial drug infusion. The combined search also included these adverse events as well as other more severe medical problems that happened any time after the trial drug infusion.

## What was learned from this trial?

Researchers learned more about the safety of **crizanlizumab** in people with **sickle cell disease who had vaso-occlusive crises (VOCs)** in India.

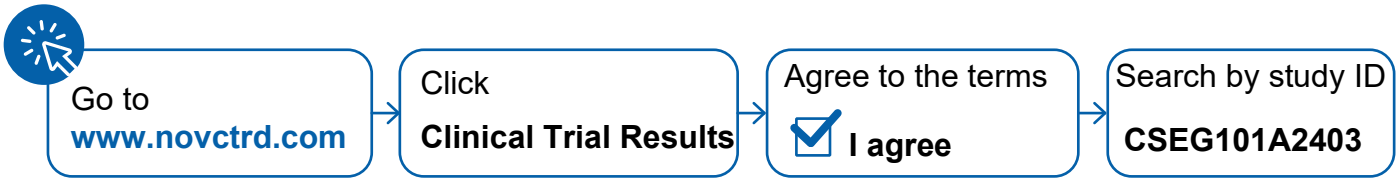
The researchers concluded that there were no new safety concerns with the use of **crizanlizumab** in this trial.

When this summary was written, several studies were ongoing for **crizanlizumab**.

# Where can I learn more about this trial?

More information about the results and adverse events in this trial can be found in the scientific summary of the results available on the Novartis Clinical Trial Results website, **www.novctrd.com**.

Follow these steps to find the scientific summary:




For more information about this trial, go to this website:

- **clinicaltrials.gov**– search using the number **NCT04662931**

Other trials of **crizanlizumab** may appear on the public website above. When there, search for **SEG101** or **crizanlizumab**.

**Full clinical trial title:** An Indian multi-centric phase IV study to assess the safety of crizanlizumab with or without hydroxyurea therapy in sickle cell disease patients with vaso-occlusive crises.

NOVARTIS

Novartis is a global healthcare company based in Switzerland that provides solutions to address the evolving needs of patients worldwide.

1-888-669-6682 (US) | +41-61-324 1111 (EU)
**www.novartis.com/clinicaltrials**