NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn more about the effects of SEG101 in men with painful erections due to sickle cell disease

Thank you!

Thank you to the participants who took part in the clinical trial for **unwanted painful erections** due to **sickle cell disease**. 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: CSEG101AUS05

Drug studied: SEG101, also known as **crizanlizumab**

Sponsor: Novartis

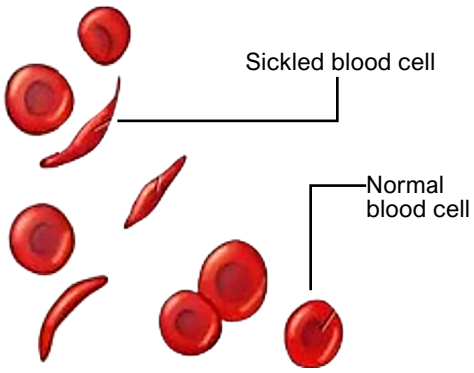
If you were a participant and have any questions about the results, please talk to the doctor or staff at the trial site.

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

What was the main purpose of this trial?

The purpose of this trial was to learn about the effects of **SEG101**, also known as **crizanlizumab**, in men with **sickle cell disease (SCD)**, causing **painful erections (priapism)**. To find this out, researchers gave participants **SEG101** to learn whether their **unwanted painful erections** decreased.

Priapism is a condition where a man gets long-lasting and **painful erections** without any sexual activity.



Priapism can happen to men with **SCD**, which is a genetic disease that can pass from parents to children.

SCD causes red blood cells to change shape (to become sickle-shaped) and stick together which blocks small blood vessels. These blocked vessels can stop blood flow out from the penis, which can lead to **painful erections**.

There was a need for medicines to specifically treat **unwanted painful erections** in men with **SCD**.

SEG101 attaches to a protein that causes the sickle blood cells to stick together and block blood flow. Thus, by blocking these proteins, **SEG101** may help to prevent blocked blood flow out of the penis in men with **SCD**.

SEG101 is approved for the treatment of **SCD** in the United States.

Trial drug
SEG101,
also called
crizanlizumab
Pronounced as
Kriz-an-LIZ-ue-mab

The trial's purpose was to answer these main questions:

- How much did the unwanted painful erections decrease after 6 months of treatment with **SEG101**?
- What adverse events did the participants have during this trial?
  - ↳ An **adverse event** is any sign or symptom that participants have during a trial.

How long was this trial?

The trial began in October 2019 and ended in November 2023. It was planned for the participants to be in the trial for about 1 year and 3 months after receiving the trial treatment.

Who was in this trial?

36 male participants with **SCD** experiencing **unwanted painful erections** received treatment in this trial.

Participants' ages ranged from 14 to 58 years. Their average age was 32 years. All the participants were from the United States. All the participants were Black or African American.

The participants could take part in this trial if they:

- were men or adolescents aged 12 years or older,
- had **SCD**, and
- had unwanted erections at least 4 times in the 3 and a half months before taking part in the trial.

What treatments did the participants receive?

All participants received **SEG101**.

SEG101 was given as an infusion into a vein of the participants.

- It was given at a dose of 5 milligrams (mg) for every kilogram (kg) of body weight.
- It was given once during Week 1 and Week 3, and then once every month.

Participants and clinical trial team knew what treatment each participant was getting.

Participants could take other medicines if the trial doctor said it was okay. Participants could also take **rescue medications**.

Rescue medications are medicines to relieve participants if their painful erections don't go away with the trial drug.

What happened during this trial?

Before treatment — Up to 3 months

Trial doctors checked the participants' health and their medical records to make sure they could be in this clinical trial.

During treatment — Up to 1 year

- 36 participants received **SEG101** on Day 1 of Week 1 and Week 3, and then every 4 weeks.
- **SEG101** was given as an infusion into a vein at a dose of 5 mg/kg.
- Participants received treatment into a vein for up to 1 year, or until they experienced an unacceptable adverse effect, could no longer be contacted, or chose to leave the trial early.

After treatment — Up to 3 and a half months

Participants returned to their trial site once after receiving their last dose of treatment for a follow-up visit to get their overall health checked.

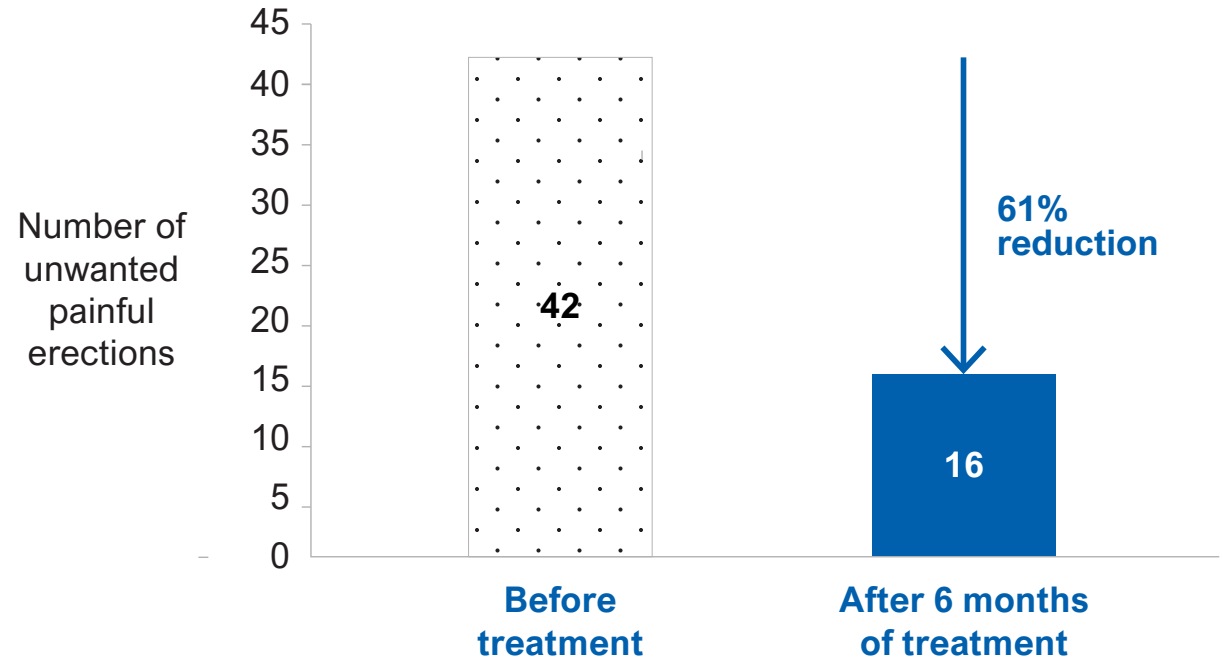
What was the main result of this trial?

How much did the unwanted painful erections decrease after 6 months of treatment with SEG101?

The number of **unwanted painful erections** decreased by 61% after receiving 6 months of **SEG101** treatment compared to before treatment.

The researchers compared the number of **unwanted painful erections** the participants had before treatment with the number of events after 6 months of treatment.

Change in the number of
unwanted painful erections



What adverse events did the participants have?

Trial doctors keep track of all **adverse events** that happen in trials, 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 up to 3 and a half months after the last 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.

7 of 36 participants (19%) had adverse events that were considered serious. 26 of 36 participants (72%) had other adverse events. None of the participants died. 1 of 36 participants (3%) left the trial treatment due to an adverse event.

The researchers concluded there were no new safety concerns for **SEG101** in this trial.

How many participants had adverse events?

Participants who:	**SEG101** 36 participants
Had at least 1 serious adverse event	**7 of 36** 19%
Had at least 1 other adverse event	**26 of 36** 72%
Left the trial treatment due to an adverse event	**1 of 36** 3%

What serious adverse events did the participants have?

7 participants had serious adverse events. No participants died in this study.

The table below shows the serious adverse events that happened in participants.

	SEG101 36 participants
Lung infection Pneumonia	**2 of 36** 6%
Blood clot in a vein between the head and the heart Jugular vein thrombosis	**1 of 36** 3%
Blood clots in the blood vessels of the lung Pulmonary embolism	**1 of 36** 3%
Low levels of oxygen in the blood Hypoxia	**1 of 36** 3%
Pain in the hands and feet Pain in extremity	**1 of 36** 3%
Skin infection around the eyes Periorbital cellulitis	**1 of 36** 3%
Swelling in the hands and feet Peripheral swelling	**1 of 36** 3%

What other adverse events did the participants have?

26 participants had other adverse events.

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

	SEG101 36 participants
Headache	**7 of 36** 19%
Fever Pyrexia	**6 of 36** 17%
COVID-19	**5 of 36** 14%
Diarrhea	**4 of 36** 11%
Back pain	**3 of 36** 8%
Cough	**3 of 36** 8%
Extreme tiredness Fatigue	**3 of 36** 8%
High level of fats (triglycerides) in the blood Hypertriglyceridaemia	**3 of 36** 8%
Increase in the number of white blood cells called lymphocytes Lymphocyte count increased	**3 of 36** 8%

What were the adverse events related to the study drug infusion that the participants have?

9 out of 36 participants (25%) had adverse events related to the study drug infusion. The table below shows the adverse events related to the study drug infusion that happened in participants.

	SEG101 36 participants
Headache	**3 of 36** 8%
Body's reaction during or shortly after receiving a treatment infusion Infusion related reaction	**2 of 36** 6%
Back pain	**1 of 36** 3%
Chest pain not caused by heart disease Non-cardiac chest pain	**1 of 36** 3%
Itchy skin Pruritis	**1 of 36** 3%
Joint pain Arthralgia	**1 of 36** 3%
Neck pain	**1 of 36** 3%
Pain and discomfort in armpit area Axillary pain	**1 of 36** 3%
Raised red bumps on the skin Rash maculo-papular	**1 of 36** 3%

What was learned from this trial?

Researchers learned about the effects of **SEG101** in men with **sickle cell disease** experiencing **unwanted painful erections**.

- The researchers found that **SEG101** treatment reduced the **unwanted painful erections** by 61%.
- Researchers found no new safety concerns for **SEG101**.

At the time this report was created, there were ongoing trials with **SEG101**.

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 **NCT03938454**

If more trials are planned, they will appear on the public website above. When there, search for **SEG101**, or **crizanlizumab**.

Full clinical trial title: A Prospective Phase 2, Open-Label, Single-arm, Multicenter Study to Assess Efficacy and Safety of SEG101 (Crizanlizumab), in Sickle Cell Disease Patients with Priapism (SPARTAN)

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