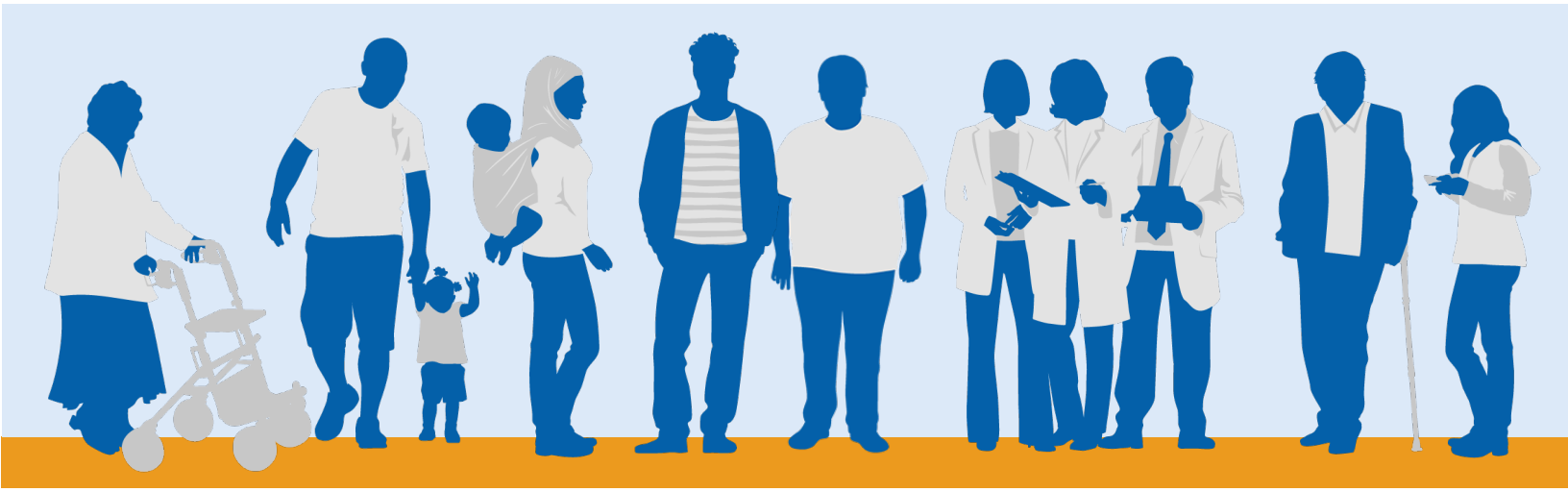
**Clinical Trial Results Summary**

NOVARTIS

# The safety of LOU064 in people with or without eczema



## Thank you!

**Thank you to the participants** who took part in the clinical trial for the trial drug **LOU064**, also known as **remibrutinib**. Every participant helped the researchers learn more about how well LOU064 works and how safe it is to take.

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:** CLOU064X2101
**Drug studied:** LOU064
**Sponsor:** Novartis

## This clinical trial at a glance

**What was the main purpose of this trial?** Read more on **page 3**

The main purpose of this trial was to learn about the safety of different dose levels of the trial drug LOU064. To do this, the clinical trial team tracked any medical problems the participants may have had during the trial. LOU064 is a trial drug designed to reduce symptoms of allergic conditions, such as eczema.

**Who was in this trial?** Read more on **page 4**

- 185 men and women
- The participants were 18 to 65 years old
- Some participants had eczema or a history of allergic reactions, but most didn't

**What trial treatments did participants take?** Read more on **page 5**

Each participant was assigned one of these treatments:

- **LOU064**
- **Placebo** – looks like the trial drug but has no trial drug in it. Using a placebo helps researchers better understand the actual effects of a trial drug.

The participants took their trial treatment as capsules.

**What were the main results of this trial?** Read more on **pages 6-7**

- Less than half of the participants without eczema had medical problems. Most of the participants with eczema had medical problems. None were considered serious.
- The most common medical problem was headache. 3 participants left this trial at the trial doctor's suggestion because their eczema got worse.

Read about other results of this trial on **page 8**

You can find **more information** about this trial by going to the websites listed on **page 9**.

# What was the main purpose of this trial?

The purpose of this trial was to learn about the safety of different dose levels of the trial drug LOU064. Researchers are looking for better ways to treat health conditions that could be caused by **overactive immune cells**. This includes some types of eczema, also called atopic dermatitis.

**What are overactive immune cells?**

Usually, immune cells protect the body from illness and infection. But if they become overactive, such as during an allergic reaction, they can attack and damage the body's own tissues.

**Eczema** is a long-term condition that causes itchy, red, and dry patches of skin.

**LOU064** is a trial drug designed to reduce symptoms of allergic conditions, such as eczema.

This 6-Part trial was the first time LOU064 was given to people. Before a drug can be approved for doctors to prescribe, researchers do many trials to find out how safe it is and how well it works.

Each of the 6 Parts focused on the safety of LOU064, as well as:

- **Parts 1, 2, and 4** – how much and how quickly LOU064 got into the blood at different dose levels
- **Part 3** – the effect of food on the levels of LOU064 in the blood
- **Part 5** – the effect of 2 capsules with small and large grain sizes on how much LOU064 got into the blood
- **Part 6** – how much LOU064 got into the blood in people with eczema

This helps researchers decide which doses to use in future clinical trials.

### The main question this trial was designed to answer:

- What medical problems did the participants have during this trial?
  Keeping track of the medical problems at different dose levels helped to learn about the safety of LOU064.

# How long was this trial?

The 6-Part trial began in August 2016 and ended in January 2020. Depending on which Part the participant was in, they could take part in the trial for up to about 3 months. A total of 4 participants did not complete this trial. 3 of the 4 left because their eczema got worse.

# Who was in this trial?

Across all 6 Parts of this trial, 185 participants took a trial treatment. There were 145 men and 40 women in this trial. The participants were 18 to 65 years old.

For Parts 1-5, all participants were considered healthy. This means they don't have certain diseases or conditions. For this trial, healthy participants didn't have eczema. Some healthy participants had a history of allergic reactions to irritants, like pollen or pet dander, but didn't need medicine for them.

For Part 6, all participants had eczema.

The table below shows the number and type of participants who were in each part of this trial. Parts 2 and 4 are combined because they had a similar design.

| | Number of participants | Participant type |
|---|---|---|
| **Part 1** | 80 | Healthy |
| **Part 2 and 4** | 64 | Healthy with a history of allergic reactions |
| **Part 3** | 12 | Healthy |
| **Part 5** | 13 | Healthy |
| **Part 6** | 16 | Had eczema |

This trial took place in Germany and the Netherlands.

Visit novctrd.com for more information about:

- Who could and could not be in this trial
- The participants in this trial, such as their age, gender, and race

Use trial number **CLOU064X2101** to find the scientific summary.

# What trial treatments did the participants take?

This trial studied a total of 13 dose levels of LOU064. Each Part of the trial studied one or more dose levels.

A computer program was used to randomly assign each participant the treatment they took as capsules:

- **LOU064**
- **Placebo:** A placebo looks like the trial drug but has no trial drug in it. Using a placebo helps researchers better understand the actual effects of a trial drug.

Using a computer program to assign the treatments helped make sure the team compared the results as fairly as possible.

The table below shows the treatment participants took and when they took it during each Part of this trial. The LOU064 dose levels were measured in milligrams (mg).

| | Focus | Treatments | How often |
|---|---|---|---|
| **Part 1** | Different dose levels | **LOU064** 0.5-600 mg **OR Placebo** | 1 time only |
| **Part 2** | Different dose levels | **LOU064** 10-600 mg **OR Placebo** | 1 time a day for 12 days |
| **Part 3** | With and without food | **LOU064** 60 mg | 1 time with food<br>1 time without food |
| **Part 4** | Different dose levels | **LOU064** 100-200 mg **OR Placebo** | 2 times a day for 12 days |
| **Part 5** | large small<br>Different grain sizes | **LOU064** 50 mg | 1 time with large grain size<br>1 time with small grain size |
| **Part 6** | People with eczema | **LOU064** 100 mg **OR Placebo** | 2 times a day for 28 days |

In most Parts of the trial, the participants and trial staff did not know what treatment each participant took. Some trials are done this way because knowing what treatment participants take can influence the results. Not knowing what treatment participants take helps make sure the results are looked at fairly.

In Part 3, everyone knew what treatment each participant took and if the participant had eaten before taking it.

In Part 6, the participants could also use skin creams for their eczema, as long as they didn't contain certain medicines, like corticosteroids.

# What were the main results of this trial?

This is a summary of the overall results of this trial. Individual results from each participant may be different and are not included in this summary.

Researchers need many trials to learn if a drug or other treatment is safe and works well. Other trials may provide new information or different results.

Always talk to a doctor before making any changes to your health care.

## What medical problems did the participants have during the trial?

Medical problems that happen during trials are called “adverse events”. Trial doctors looked for any adverse events during the visits to the trial site. The participants also reported adverse events. This section includes the adverse events that happened during and after trial treatment.

Many trials are needed to know if a drug or treatment causes an adverse event. Trial doctors keep track of all adverse events that happen in trials, even if they do not think the adverse events are related to the trial treatments.

**What is an adverse event?**

- Adverse events **may or may not be caused** by treatments in the trial.
- An **adverse event** is any unwanted sign or symptom that the participants have during a trial.
- It is considered “**serious**” when it is life-threatening, causes lasting problems, the participant needs hospital care, or results in death.

During Parts 1 – 5, less than half of the participants without eczema had adverse events. During Part 6, most of the participants with eczema had adverse events. None were considered serious.

The most common adverse event was headache. 3 participants in Part 6 left this trial at the trial doctor’s suggestion because their eczema got worse.

## What serious adverse events did participants have?

No serious adverse events were reported, including deaths.

## What other adverse events did participants have?

Most participants in this trial had adverse events that were not serious. The most common adverse events that happened to **4 or more participants** in at least one Part of the trial were:

- Headache
- Common cold (nasopharyngitis)
- Viral infection in the nose, throat, and airways (viral upper respiratory tract infection)
- Pooling of blood under the skin (hematoma)

The table below shows how many participants had other adverse events during each Part of this trial. The results for Parts 2 and 4 are combined because they had a similar design.

### Participants with other adverse events

| | LOU064 Any dose | Placebo |
|---|---|---|
| **Part 1**<br>80 participants | **28%**<br>17 of 60 | **15%**<br>3 of 20 |
| **Part 2 and 4**<br>64 participants | **50%**<br>24 of 48 | **50%**<br>8 of 16 |
| **Part 3**<br>12 participants | **58%**<br>7 of 12 | No participants took the placebo in Part 3 |
| **Part 5**<br>13 participants | **46%**<br>6 of 13 | No participants took the placebo in Part 5 |
| **Part 6**<br>16 participants | **92%**<br>11 of 12 | **100%**<br>4 of 4 |

For more information about the adverse events the participants in this trial had, visit novctrd.com. Use trial number **CLOU064X2101** to find the scientific summary.

## What other results were learned?

The trial staff took many blood samples from the participants to measure the levels of LOU064 in their blood over time. They did this for each of the 6 Parts of the trial.

The clinical trial team found that LOU064:

- Quickly reached its highest level in the participants' blood about an hour after the participants took it, regardless of dose level
- Took a little longer to get into the participants' blood if they had recently eaten
- Reached its highest level in the participants' blood if the capsule had a smaller grain size
- Reached higher levels in the participants' blood after they took it for 28 days compared to their first dose

This information will help researchers decide how much and how often someone may need to take a treatment and if they can take it with food.

# What was learned from this trial?

This was the first trial to look at the effects and safety of LOU064 in humans. The clinical trial team found no serious safety concerns for the participants who took LOU064. These results will help researchers choose which dose levels to use in other clinical trials of LOU064.

This 6-Part trial included a small number of participants in each Part. This was one of many trials a treatment must go through before it can be approved for doctors to prescribe.

The results presented here are for one trial. One trial cannot give a complete picture of the benefits and risks of a trial drug. The results of many trials are needed to find out which treatments can be used for people with eczema. This summary shows only the main results from this trial. Other trials may provide new information or different results.

# Where can I learn more about this and future clinical trials?

You can find detailed results and more information about this clinical trial on the Novartis Clinical Trial Results website:

1. Visit novctrd.com
2. Click on “Clinical Trial Results” at the top right of the page
3. Read and scroll down, then click “I accept” to agree to use the information and the website
4. Select “Study number” from the drop-down menu
5. Type **“CLOU064X2101”** in the search box and click search

If you would like to view the website in a language other than English, you can click the “Google Translate” button on the top right of the page.

? If you participated in the trial and have **questions** about the results, please speak with the trial doctors or staff at your trial site.

This trial was registered on this website:

- ClinicalTrials.gov – https://clinicaltrials.gov/
  To find this trial, type **CLOU064X2101** in the **Other terms** search box

**Full trial title:**

A 6-part first-in-human study of LOU064 consisting of a 4-part randomized, double-blind, placebo-controlled SAD and MAD study to investigate the safety and tolerability in healthy volunteers, subjects with atopic diathesis and subjects with atopic dermatitis, an open-label food effect study and a double-blind formulation effect study in healthy volunteers.

If more trials are planned, they will appear on the public websites listed above. When there, search for **LOU064** or **remibrutinib**.

## Thank you!

Novartis would like to thank all of the people who participated in this clinical trial. The participants made this clinical trial possible and helped researchers answer important health questions and learn about a possible medical treatment. Many participants and many clinical trials are needed to advance medical science.

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 (USA)
+41-61-324 1111 (EU)
www.novartisclinicaltrials