

## Summary of Clinical Trial Results

### A study looking at the safety of baloxavir marboxil in children with influenza who are younger than 1 year (miniSTONE-1)

See the end of the summary for the full title of the study.

#### About this summary

This is a summary of the results of a clinical trial (called a 'study' in this document) – written for:

- members of the public and
- people who took part in the study

This summary is based on information known at the time of writing.

The study started in January 2019 and finished in July 2023. This summary was written after the study had ended.

No single study can tell us everything about the risks and benefits of a medicine. It takes lots of people in many studies to find out everything we need to know. The results from this study may be different from other studies with the same medicine.

- **This means that you should not make decisions based on this one summary – always speak to your doctor before making any treatment decisions for your child**

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#### Thank you to the people who took part in this study

The people who took part have helped researchers to answer important questions about influenza and the medicine studied (baloxavir marboxil), which could have benefits for the general public.

## Key information about this study

- This study was done to see if baloxavir marboxil is safe for use in children younger than 1 year
- This study included 48 children in 7 countries (Costa Rica, Finland, Mexico, Poland, South Africa, Spain and the USA)
- The main finding was that baloxavir marboxil was as safe for children aged younger than 1 year to take as it is for older children and adults
- None of the children who took part had serious side effects that were related to baloxavir marboxil

## 1. General information about this study

### Why was this study done?

Influenza, also called flu, is a common disease caused by infection with the flu virus. Common symptoms include fever, cough, sore throat, body aches and fatigue.

Most people recover from flu within a week without needing to see a doctor. Sometimes, flu can lead to more serious illnesses (complications) such as swelling/inflammation of the lungs (pneumonia) or blood poisoning (sepsis). Flu often affects children, and they are more likely than adults to have complications.

There are many over-the-counter medications that can be used to treat the symptoms of flu, but these do not treat the cause – the viral infection. Antiviral treatments attack the flu virus directly. These can be prescribed by a doctor or pharmacist. Each year, millions of people worldwide need treatment for flu.

Baloxavir marboxil is an antiviral treatment for influenza that works differently to other antivirals. It has been shown to be effective for treating flu in adults and children who are at least 1 year old. This study looked at how safe baloxavir marboxil is in children who are younger than 1 year.

### What was the medicine being studied?

A medicine called ‘baloxavir marboxil’ was the focus of this study

- You say this as ‘ba-lox-av-ear mar-box-il’
- Baloxavir marboxil works by stopping the flu virus from making copies of itself in the body
- This may mean that the flu is less serious and children recover more quickly when they are treated with baloxavir marboxil

### What did researchers want to find out?

**The main question that researchers wanted to answer was:**

How many children had side effects (medical problems that happen during the study) due to baloxavir marboxil treatment?

## What kind of study was this?

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This study was a 'Phase 3' study. This means that it expanded on previous studies to find out about the side effects of baloxavir marboxil in children who are younger than 1 year. It can then be decided whether the treatment can be approved for doctors to give to very young children.

This was an 'open label' study. This means that both the people taking part in the study and the study doctors knew the study medicine that the children were being given.

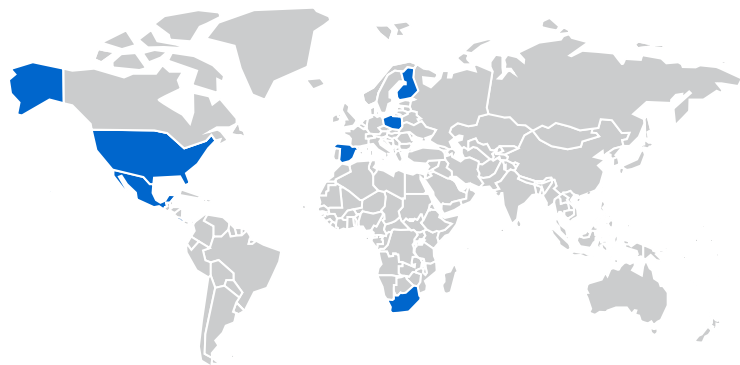
## When and where did the study take place?

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The study started in January 2019 and finished in July 2023. This summary was written after the study had ended.

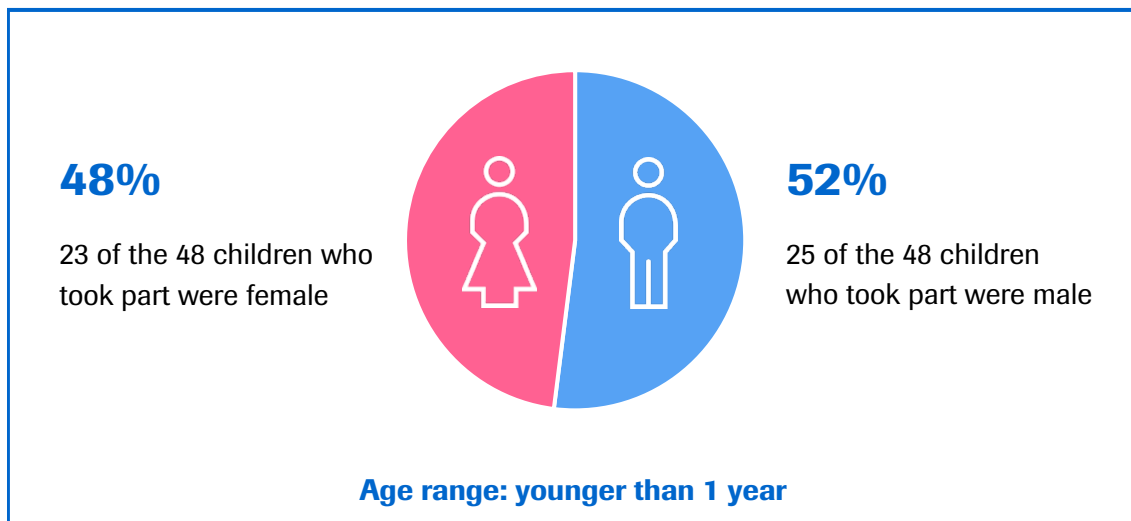
The study took place at 15 study centres in the 7 countries shown in the following map:

- Costa Rica
- Finland
- Mexico
- Poland
- South Africa
- Spain
- USA



## 2. Who took part in this study?

In this study, 48 children younger than 1 year with a flu or flu-like infection took part.



Children could take part in the study if:

- They were aged less than 1 year
- Their parents/caregivers agreed they could take part in the study
- They were diagnosed with flu based on a doctor's judgement of the symptoms and a positive flu test result
- They had a negative COVID-19 test result within 2 days of seeing a doctor
- They saw a doctor within 4 days of having flu symptoms

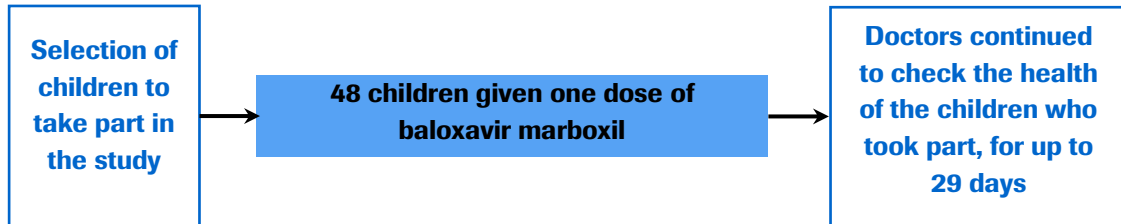
Children could not take part in the study if:

- They needed treatment for flu (or another illness) in a hospital
- They had been given another treatment that could affect how baloxavir marboxil works
- They were born prematurely, or they weighed less than 2.5kg

### 3. What happened during the study?

During the study, all children who took part were given a single dose of baloxavir marboxil as granules mixed with water taken by mouth. The amount they were given depended on their body weight.

When the study finished, the parents or caregivers of the participating children were asked to come back to their study centre for more visits – to check the overall health of these children. Look below to see more information about what happened in the study.



### 4. What were the results of the study?

**The main question:** How many children had side effects due to baloxavir marboxil treatment?

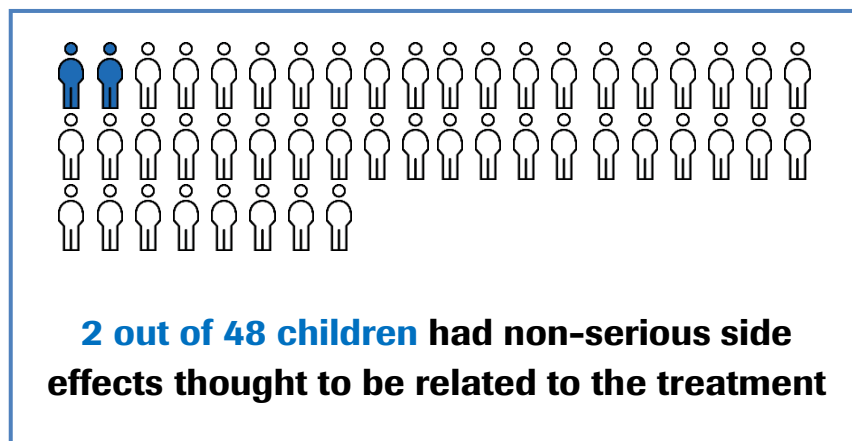
Side effects are medical problems that happen during the study.

Non-serious side effects are medical problems (such as feeling dizzy) during the study. Serious side effects are those that cause unplanned hospitalisation (or hospitalisation for longer than planned) or are life-threatening, cause long-term disability or loss of life. Only side effects that doctors thought were related to the study medicine are shown.

Not all of the children in this study had a side effect.

None of the children who took part in this study had serious side effects that were related to the study treatment.

Out of the 48 children in this study, 2 (4%) had non-serious side effects that were thought to be related to the treatment. These side effects were both diarrhoea.



During the study, none of the children treated with baloxavir marboxil died due to side effects that may have been related to the study medicine.

None of the children treated with baloxavir marboxil stopped being given the study medicine because of side effects.

This section only shows the key results from this study. You can find more information about the study results on the websites at the end of this summary (see Section 7).

## 5. How has this study helped research?

The information presented here is from a single study of 48 children younger than 1 year with flu. These results helped researchers learn more about flu and baloxavir marboxil, which could lead to better treatments for the general public.

The key findings of the study are that baloxavir marboxil is generally safe in children younger than 1 year and there were no serious side effects related to the treatment. This is in line with the known safety of baloxavir marboxil in older children, adolescents and adults.

No single study can tell us everything about the risks and benefits of a medicine. It takes lots of people in many studies to find out everything we need to know. The results from this study may be different from other studies with the same medicine.

- **This means that you should not make decisions based on this one summary – always speak to your doctor before making any treatment decisions for your child.**

## 6. Are there plans for other studies in children?

Studies with baloxavir marboxil are still happening.

CENTERSTONE is a study looking at the effect of baloxavir marboxil in stopping the spread of flu in people aged 5 to 64 years.

PEBBLESTONE is a study of baloxavir marboxil in children aged 1 to 11 years, looking at influenza virus resistance, which is when changes happen inside a virus that can make antiviral treatment less effective.

More information about these studies can be found at:

- <https://clinicaltrials.gov/study/NCT03969212>
- <https://clinicaltrials.gov/ct2/show/NCT06094010>

## 7. Where can I find more information?

You can find more information about this study (miniSTONE-1) on the websites listed below:

- <https://clinicaltrials.gov/ct2/show/results/NCT03653364>
- <https://www.clinicaltrialsregister.eu/ctr-search/trial/2018-002154-70/FI>
- <https://forpatients.roche.com/en/trials/infectious-diseases/influenza/study-to-assess-the-safety--pharmacokinetics--and-effic-62594.html>

### Who can I contact if I have questions about this study?

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If you have any further questions after reading this summary:

- Visit the ForPatients platform and fill out the contact form – <https://forpatients.roche.com/en/trials/infectious-diseases/influenza/study-to-assess-the-safety--pharmacokinetics--and-effic-62594.html>
- Contact a representative at your local Roche office

If your child has taken part in this study and you have any questions about the results:

- Speak with your study doctor

If you have questions about the treatment of your child:

- Speak to the doctor in charge of your child's treatment

### Who organised and paid for this study?

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This study was organised and paid for by F. Hoffmann-La Roche Ltd, which has its headquarters in Basel, Switzerland.

### Full title of the study and other identifying information

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The full title of this study is: "Study to Assess the Safety, Pharmacokinetics, and Efficacy of Baloxavir Marboxil in Healthy Pediatric Participants From Birth to <1 Year With Influenza-Like Symptoms".

The study is known as 'miniSTONE-1'.

- The protocol number for this study is: CP40559
- The ClinicalTrials.gov identifier for this study is: NCT03653364
- The EudraCT number for this study is: 2018-002154-70