Summary of Clinical Trial Results

A study to look at whether basmisanil worked in children with Dup15q syndrome – and how safe this medicine was

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 December 2022 and recruitment was stopped early – in January 2024. The sponsor made the difficult decision to end the study. This was due to a need to focus the organisation and research resources on where it could have the most impact for the widest number of people across therapy areas.

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 decisions about your treatment.

Contents of the summary

Thank you to the people who took part in this study

The people who took part have helped researchers to answer important questions about Dup15q syndrome and the medicine studied – 'basmisanil'.

Key information about this study

Why was this study done?

- This study was done to look at how well a potential new treatment for Dup15q syndrome worked, how safe this medicine was for children with Dup15q, and the effect it had on the body.
- In this study, people were given either the medicine being studied (called 'basmisanil') or a non-active 'placebo' – it was decided by chance which treatment each person was given.
- This study included 7 people in 3 countries.

What were the results?

- The researchers found that after 2 weeks of treatment, basmisanil reduced a specific brain signal (qEEG beta-band signal) that is higher in people with Dup15q syndrome. However, these results come from a small group of participants (5) and should be viewed carefully.
- As the study stopped early, the researchers could not reach any further conclusions about the effects of basmisanil treatment on the symptoms of Dup15q syndrome.
- No one taking basmisanil or placebo had serious unwanted effects.
- This study was stopped early, as part of a portfolio evaluation, because the sponsor's research organisation continuously assesses its priorities and opportunities in line with the company's strategy.

1. General information about this study

Why was this study done?

Dup15q syndrome is a rare disorder that affects how the brain and nerves develop. It is caused by having extra copies of a piece of DNA from chromosome 15. A chromosome is a thread-like structure in a cell that contains genes, which are segments of DNA carrying genetic information.

The symptoms can differ a lot from one child to another, but can include:

1. Low muscle tone (hypotonia) where muscles are looser and more relaxed than usual
2. Seizures, which are sudden, uncontrolled electrical disturbances in the brain that can cause changes in behaviour, movements, feelings, or levels of consciousness
3. Delays in thinking, learning, and remembering
4. Slower development of motor skills, like walking or using hands

5. Trouble with communication and understanding social cues, often linked to autism

People with Dup15q syndrome may have extra proteins called GABA receptors. These help control how messages move through the brain and how the body responds to messages. Because of the extra GABA receptors, their brains and bodies may react differently to these signals compared to people who do not have Dup15q syndrome.

In this study, researchers aimed to find out what effects a potential new treatment for children with Dup15q syndrome has on language, social skills, and their ability to do everyday tasks (such as eating and dressing).

What was the medicine being studied?

A medicine called 'basmisanil' was the focus of this study.

- You say this as 'bazz-MISS-uh-nill'.
- Basmisanil may work by dampening the effect of the extra GABA receptors.
- This may mean that it lowers the overactive signals in the brain and could be helpful to manage symptoms of Dup15q syndrome.

Basmisanil was compared to a 'placebo'.

- You say this as 'plah – see – bo'
- The placebo looked the same as basmisanil but did not contain any real medicine. This means it had no medicine-related effect on the body.
- Researchers compared basmisanil to a placebo so they could show which benefits or unwanted effects are actually caused by the medicine.

What did researchers want to find out?

- Researchers did this study to compare basmisanil with a placebo – to see how well basmisanil worked and the effects it had on the brain (see Section 4 'What were the results of the study?').
- They also wanted to find out how safe the medicine was – by checking how many people had unwanted effects and seeing how serious they were, when taking each of the medicines during this study (see Section 5 'What were the unwanted effects?').

The main question that researchers wanted to answer was:

- What effects did basmisanil have on language, social skills and ability to carry out everyday tasks?

Another question that researchers wanted to answer was:

- What effects did basmisanil have on the brain?

What kind of study was this?

This study was a 'Phase 2' study. This means that basmisanil had been tested in a number of people, including children, before this study. In this study, children with Dup15q syndrome either took basmisanil or a placebo. This was to find out if basmisanil worked to improve their language, social skills, and their ability to do everyday tasks.

The study was 'randomised'. This means that it was decided by chance which of the medicines people in the study would have – like tossing a coin. Randomly choosing which medicine people take makes it more likely that the types of people in both groups (for example, age, race) will be a similar mix. Apart from the exact medicines being tested in each group, all other aspects of care were the same between the groups.

The first part of this was 'double-blind'. This means that nobody knows which treatment was given – not the people in the study or the team running it. This is done to make sure that the results of the treatment are not affected by what people expected from the received treatment. After the study is finished, the people in the study can ask to find out which treatment they got.

The second part of the study was 'open-label'. This means everyone involved, including the participant and the study doctor, knew which study treatment the participant was given.

When and where did the study take place?

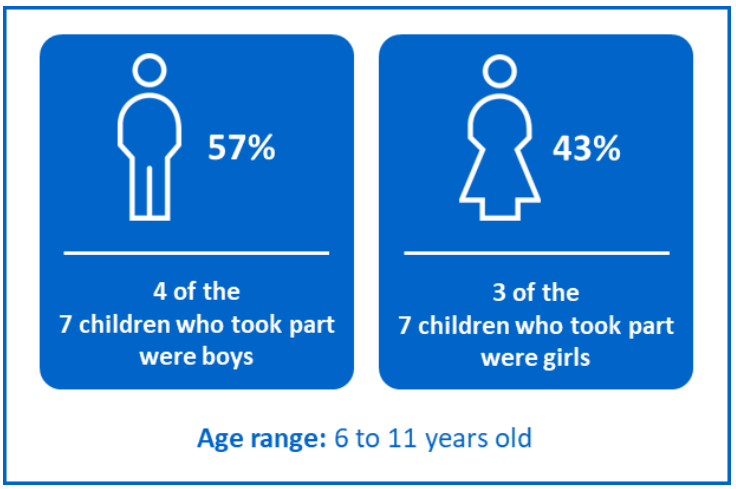
The study started in December 2022 and recruitment was stopped in January 2024 – as part of a portfolio evaluation, because the sponsor's research organisation continuously assesses its priorities and opportunities in line with the company's strategy. This summary presents the results of the study until it was stopped.

Participants were enrolled at 5 study centres – across 3 countries: Spain, the United Kingdom, and the United States.

2. Who took part in this study?

In this study, 7 children with Dup15q syndrome took part.

Children who took part in the study were between 6 and 11 years of age. 4 of the 7 children (57%) were boys and 3 of the 7 children (43%) were girls.



Children could take part in the study if:

- They were between 2 and 14 years old
- They were diagnosed with Dup15q syndrome and had symptoms that affected their daily lives

Children could not take part in the study if:

- They had epilepsy that was not controlled
- They had blood cancer or other certain cancers within the last 5 years

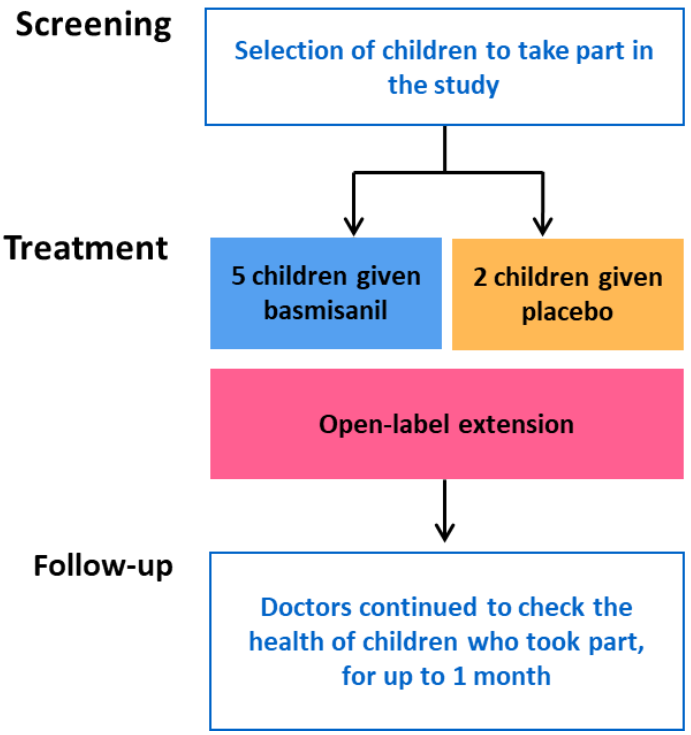
3. What happened during the study?

During the study, children were selected by chance to get 1 of 2 treatments. The treatments were selected at random – by a computer.

The treatment groups were:

- Basmisanil (the medicine being studied) OR
- Placebo

Treatment was given as granules (packaged in stick packs), to be swallowed with soft food twice on Day 1, then 3 times daily from Day 2 for 1 year.



4. What were the results of the study?

Question 1: What effects did basmisanil have on language, social skills and ability to carry out everyday tasks?

Researchers used a tool called the 'Vineland-3 Adaptive Behaviour Scales' to assess the children's language, social skills, and ability to do day-to-day activities. The children were interviewed before starting treatment and researchers planned to do follow-up interviews at 6 months and 1 year to check for any changes.

All 7 children were interviewed before treatment. However, only 3 children were interviewed again at 6 months or 1 year.

As only a small number of children were involved in the study, the researchers could not reach any conclusion on how basmisanil treatment affected the symptoms of Dup15q syndrome.

Question 2: What effects did basmisanil have on the brain?

Another piece of information that researchers collected was how treatment affected electrical activity in the brain. They measured signals in the brain with an electroencephalography (EEG) machine at the start of the study and at other visits. The researchers found that after 2 weeks of treatment, basmisanil reduced a specific brain signal (qEEG beta-band signal) that is higher in people with Dup15q syndrome:

- All 5 children in the basmisanil group had a lower level of electrical activity in the brain after 2 weeks of treatment.
- Of the 2 children in the placebo group, 1 had a higher level of activity and 1 had a lower level of activity after 2 weeks of treatment.
- However, these results come from a small group of participants (5) and should be viewed carefully.

This section only shows the key results from this study. You can find information about all other results on the websites at the end of this summary (see Section 8).

5. What were the unwanted effects?

Unwanted effects are medical problems (such as feeling dizzy) that happen during the study.

- They are described in this summary because the study doctor believes the unwanted effects were related to the treatments in the study.
- Not all of the people in this study had all of the unwanted effects.
- Unwanted effects may be mild to very serious and can be different from person to person.
- It is important to be aware that the unwanted effects reported here are from this single study. Therefore, the unwanted effects shown here may be different from those seen in other studies.
- Serious and common unwanted effects are listed in the following sections.

No safety concerns were found for basmisanil in children aged 6 to 11 years with Dup15q syndrome during the study.

Serious unwanted effects

An unwanted effect is considered ‘serious’ if it is life-threatening, needs hospital care, or causes lasting problems.

During this study, no participants died due to unwanted effects that may have been related to the study medicine.

During the study, no one decided to stop taking their medicine because of unwanted effects.

Most common unwanted effects

During this study, no one had an unwanted effect related to basmisanil.

Other unwanted effects

You can find information about other unwanted effects (not shown in the sections above) on the websites listed at the end of this summary – see Section 8.

6. How has this study helped research?

The information presented here is from a single study of 7 people with Dup15q syndrome. These results helped researchers learn more about the role of GABA receptors Dup15q syndrome and basmisanil.

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.

7. Are there plans for other studies?

No further studies are planned with basmisanil.

8. Where can I find more information?

You can find more information about this study on the websites listed below:

- https://clinicaltrials.gov/ct2/show/results/NCT05307679
- https://www.clinicaltrialsregister.eu/ctr-search/trial/2022-502165-20-00/results
- https://forpatients.roche.com/en/trials/neurodevelopmental-disorder/dup15q-syndrome/a-study-to-evaluate-the-safety-and-efficacy-of-basmisan-98150.html

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

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/neurodevelopmental-disorder/dup15q-syndrome/a-study-to-evaluate-the-safety-and-efficacy-of-basmisan-98150.html
- Contact a representative at your local Roche office.

If you took part in this study and have any questions about the results:

- Speak with the study doctor or staff at the study hospital or clinic.

If you have questions about your own treatment:

- Speak to the doctor in charge of your treatment.

Who organised and paid for this study?

This study was organised and paid for by F. Hoffmann-La Roche Ltd who have their headquarters in Basel, Switzerland.

Full title of the study and other identifying information

The full title of this study is: 'A Phase II, Randomized, Double-Blind, Placebo-Controlled, Parallel Group Study to Evaluate the Safety, Efficacy, and Pharmacodynamics of 52 Weeks of Treatment with Basmisanil in Participants Aged 2 to 14 Years Old with Dup15q Syndrome followed by a 2-Year Optional Open-Label Extension'.

The study is known as 'Quindecim'.

- The protocol number for this study is: BP42992.
- The ClinicalTrials.gov identifier for this study is: NCT05307679.
- The EudraCT number for this study is:2022-502165-20-00.