ForPatients
by Roche

Dup15q Syndrome

A clinical trial to compare the effects of basmisanil with a placebo in children with Dup15q syndrome

A Study to Evaluate the Safety and Efficacy of Basmisanil Treatment in Children With Dup15q Syndrome

Trial Status	Trial Runs In	Trial Identifier
Terminated	5 Countries	NCT05307679 BP42992

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

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

Trial Summary:

This study consists of two parts. Part 1 will evaluate the safety, efficacy, and pharmacodynamics of 52-weeks of basmisanil treatment in children and adolescents (aged 2-14 years) with Dup15q syndrome. Part 1 will test the hypothesis that negative allosteric modulation of a GABAA receptor subtype can address excessive receptor function and positively impact core neurodevelopmental disease feature in individuals with Dup15q syndrome. Part 2 is an optional 2-year open-label extension to evaluate long-term safety, tolerability, and to provide supportive evidence of benefit of continued treatment with basmisanil in selected efficacy outcomes.

Hoffmann-La Roche
Sponsor

Phase 2
Phase

NCT05307679 BP42992
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	#2 Years & # 14 Years	No

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Why is the Quindecim clinical trial needed?

Dup15q syndrome is a rare neurodevelopmental disorder with a genetic cause (meaning a change in the DNA sequence causes it). In this disorder, there are extra copies of a certain portion of a child's DNA (chromosome 15). This can cause symptoms such as decreased tension in skeletal muscles (hypotonia); seizures (epilepsy); delayed ability to think, learn, and remember (cognitive delay); delayed development of muscle control (motor delays); and difficulties communicating and understanding what people think and feel (autism spectrum disorder). These symptoms will differ widely from child to child.

Basmisanil is a drug that is currently being investigated for the treatment of Dup15q syndrome. In this trial, researchers aim to find out what effects, good or bad, basmisanil may have on language, social skills, and ability to do day-to-day activities of children with Dup15q syndrome, compared with a placebo.

Find out more: Dup15q study

How does the Quindecim clinical trial work?

This clinical trial is recruiting children between 2 and 11 years of age who have been diagnosed with Dup15q syndrome.

The purpose of this clinical trial is to compare the effects, good or bad, of basmisanil with a substance with no active ingredients (known as a placebo) in children with Dup15q syndrome. People who take part in this clinical trial will receive either basmisanil or a placebo.

Participants will be given the clinical trial treatment basmisanil OR placebo twice a day on Day 1, then three times a day every day for 52 weeks. Participants will be seen by the clinical trial doctor at different times throughout the trial period. Visits may be carried out at a clinic, or for some visits, a nurse may visit the participant's home (remote visit), depending on regulations in the participant's country. Participants will go to the clinic for 2 days in a row, on Day 1 and Day 2. Following this, there will be six additional visits until the end of the clinical trial. These visits will include checks to see how the participant responds to the treatment and investigate any side effects they may be experiencing.

A follow-up visit will take place 4 weeks after participants have completed 52 weeks of treatment. Participants' total time in the clinical trial will be roughly 14 months. Caregivers may decide to stop trial treatment and leave the clinical trial at any time.

What are the main endpoints of the Quindecim clinical trial?

The main clinical trial endpoint (the main result that is measured in the trial to see if the medicine has worked) is to test the effects of basmisanil on language, social skills, and ability to do day-to-day activities.

Other clinical trial endpoints include testing the effect of basmisanil on activity or movement (motor function), the ability to learn and remember (cognition), measurement of how the body processes basmisanil, and the number and seriousness of any side effects that occur in children with Dup15q syndrome while on treatment.

Who can take part in this clinical trial?

People can take part in this trial if they are aged between 2#11 years old and have documented extra copies of a specific part of chromosome 15. Children who want to participate must have a caregiver (such as a parent, medical caregiver, or legally authorised representative of at least 18 years of age) present throughout the trial.

People may not be able to take part in this trial if they have certain other medical conditions or have previously received certain treatments. The clinical trial doctor will check to see if potential participants fit the criteria for the trial.

What treatment will participants be given in this clinical trial?

Everyone who joins this clinical trial will be assigned to one of two groups randomly (like flipping a coin) and given either:

- Basmisanil, given as granuales (packaged in stick packs), to be swallowed with soft food twice a day on Day 1, then three times every day from Day 2 onwards for 52 weeks
- OR a placebo, given as granules (packaged in stick packs), to be swallowed with soft food twice a day on Day 1, then three times every day from Day 2 onwards for 52 weeks

Participants will have a 2 in 3 (66%) chance of being given basmisanil.

This is a ‘placebo-controlled’ clinical trial, which means that one of the groups will be given a substance with no active ingredients (also known as a ‘placebo’); it looks like the drug being tested. Comparing results from the different groups helps the researchers know whether any changes seen are a result of the drug or occurring by chance.

This is a double-blinded trial, which means that neither the participant nor the clinical trial doctor can choose or know the group the participant is in, until the trial is over. This

approach helps to prevent bias and expectations about what will happen. However, the participant's clinical trial doctor can find out which group the participant is in, if their safety is at risk.

Are there any risks or benefits in taking part in this clinical trial?

The safety or effectiveness of the experimental treatment or use may not be fully known at the time of the trial. Most trials involve some risks to the participant, although it may not be greater than the risks related to routine medical care or the natural progression of the health condition. Potential participants will be told about any risks and benefits of taking part in the clinical trial, as well as any additional procedures, tests, or assessments they will be asked to undergo. These will all be described in an informed consent document (a document that provides people with the information they need to make a decision to volunteer for a clinical trial). A potential participant should also discuss these with members of the research team and with their usual healthcare provider. Anyone interested in taking part in a clinical trial should know as much as possible about the trial and feel comfortable asking the research team any questions about the trial.

Risks associated with the clinical trial

Participants may have side effects (an unwanted effect of a drug or medical treatment) from the drug used in this clinical trial. Side effects can be mild to severe and even life-threatening, and can vary from person to person.

Basmisanil

Potential participants will be told about the known side effects of basmisanil, and, where relevant, potential side effects based on human and laboratory studies or knowledge of similar drugs.

Basmisanil will be given as granules to be swallowed with yoghurt, applesauce or pudding. Participants will be told about any known side effects of oral administration.

Potential benefits associated with the clinical trial

Participants' health may or may not improve from participation in the clinical trial, but the information that is collected may help other people who have a similar medical condition in the future.

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For more information about this clinical trial see the **For Expert** tab on the specific ForPatients page or follow this link to ClinicalTrials.gov

Inclusion Criteria:

- Documented maternal duplication (3 copies) or triplication (4 copies) of the chromosome 15q11.2-q13.1 region that includes the Prader Willi/Angelman critical region defined as [BP2-BP3] segment
- Dup15q syndrome Clinician Global Impression of Severity scale (Dup15q CGI-S) overall severity score # 4 (at least moderately ill)
- Body weight equal to or above the third percentile for age
- Participant has a parent, caregiver, or legally authorized representative (hereinafter "caregiver") of at least 18 years of age, who is fluent in the local language at the site, and capable and willing to provide written informed consent for the participant, according to International Council for Harmonisation and local regulations
- Participant's caregiver must be living with the participant and, in the opinion of the Investigator, able and willing to reliably assess the participant's ongoing condition, to accompany the participant to all clinic visits, and ensure compliance to study treatment throughout the study. The same caregiver is able and willing to complete the caregiver assessments and is available to the Investigational Site by telephone or email if needed
- Participant's caregiver is able and willing to use electronic devices to record information on the participant's condition and to complete assessments at home and agrees to home nursing visits, if local regulations allow for it and if home nursing service is available in the country/region

Exclusion Criteria:

- Uncontrolled epilepsy at screening (as defined by the protocol)
- Lymphoma, leukemia, or any malignancy within the past 5 years, except for basal cell or squamous epithelial carcinomas of the skin that have been resected with no evidence of metastatic disease for 3 years
- Clinically significant ECG abnormalities at Screening
- Clinically significant abnormalities in laboratory test results at screening (including positive results for HIV, hepatitis B and/or hepatitis C)
- Allowed prior existing medication should be on a stable regimen (or frequency of intervention) for at least 6 weeks, and at least 8 weeks for anti-epileptic treatment, prior to Screening
- Non-pharmacological / behavioral therapies should not be stopped or newly started at least 6 weeks prior to Screening and are expected to remain stable for the entire study duration (excluding changes related to standard age and educational interventional programs and minor interruptions such as illness or vacation
- Concomitant use of prohibited medications
- Participation in an investigational drug study within one month or within 6 × the elimination half-life, whichever is longer, prior to dosing in the study
- Significant risk for suicidal behavior, as assessed through the suicidal behavior question adapted from the Columbia Classification Algorithm for Suicide Assessment (C-CASA) (participants # 6 years of age only)
- Known sensitivity to any of the study treatments or components thereof or drug or other allergy that, in the opinion of the Investigator, contraindicates the participation in the study, including severe lactose intolerance (e.g., unable to tolerate 250 mL [8 oz. or 1 cup] of milk, ice cream, or yogurt)

- Concomitant clinically relevant disease or condition or any clinically significant finding at screening that could interfere with, or for which, the treatment might interfere with, the conduct of the study or that would pose an unacceptable risk to the participants in this study
- Known active or uncontrolled bacterial, viral, or other infection (excluding fungal infections of nail beds) or any major clinically significant episode of infection or hospitalization (relating to the completion of the course of antibiotics) within 6 weeks prior to the start of drug administration