ForPatients
by Roche

Angelman Syndrome

A Study To Investigate The Safety Of RO7248824 in Children With Angelman Syndrome And To Understand The Way The Body Processes The Investigational Therapy

A Study to Investigate the Safety, Tolerability, Pharmacokinetics (PK) and Pharmacodynamics (PD) of RO7248824 in Participants With Angelman Syndrome (AS)

Trial Status	Trial Runs In	Trial Identifier
Active, not recruiting	4 Countries	NCT04428281 2019-003787-48,RG6091 BP41674

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

Trial Summary:

This is a phase I, multicenter, non-randomized, adaptive, open-label, multiple ascending, intra-participant, dose-escalation study with a long-term extension (LTE) part and an optional open-label extension (OOE) part. The objective of the study is to investigate the safety, tolerability, PK and PD of RO7248824 administered intrathecally (IT) in participants with AS. Two linked sets of dose escalation cohorts are planned based on two different age groups, namely participants with AS aged # 5 to # 12 years in cohorts A1 to A5 (with at least 2 participants # 8 years old in each cohort) and AS participants aged # 1 to # 4 years in cohorts B1 to B5. The two sets of cohorts will be run in parallel, with each cohort A1 to A5 preceding and gating the linked cohort B1 to B5 (e.g., A1 precedes B1).

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Phase 1
Phase

NCT04428281 2019-003787-48,RG6091 BP41674
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	>=1 Year & <= 12 Years	No

A clinical study for children with Angelman Syndrome between 1 and 12 years old

This clinical study will test the safety of a new investigational therapy and study the way the body processes the study medication. The study will measure the impact of the investigational therapy using various clinical scales.

Angelman Syndrome (AS) is a rare genetic disease impacting the development of the nervous system. It results in life-long physical and cognitive disability. Standard treatments for AS are designed to treat disease symptoms such as seizures and sleep disturbance.

What is a clinical trial?

Who is eligible to participate?

Children diagnosed with AS between 1 to 12 years old will be evaluated by a hospital healthcare team.

Does this clinical study use placebo?

There is no placebo. All participants in the study will receive the investigational treatment.

Where do I go to get more information about this study?

As a caregiver for a child with AS, we understand your need for up-to-date information about this clinical study. Use the link to Contact us for further information. It may be possible to discuss this clinical study, one-to-one, with a study nurse. Your child's treating physician is also invited to contact us on your behalf.

How is this study set up?

Children participating in this clinical study will receive the investigational therapy at a hospital staffed with teams trained in AS care. Many hospitals will participate in the study and we encourage you to find out if there is a hospital participating in the study that is close to where you live. Tap **Find trial locations** at the top of this page.

This study is set up to measure the effects of taking the investigational therapy. The study will measure changes in AS symptoms, as well as many details of your child’s health and emotional wellbeing.

The study will also consider your treatment journey, as caregivers. This assessment is holistic. We consider fatigue associated with night time care of your child, the impact of your care schedule on everyday life, and how living with AS impacts family relationships.

Children taking sleep medications may be eligible for this study. When studying a new treatment under the conditions of a clinical study, it is important that any existing treatments remain unchanged throughout the study period.

In practical terms, this means that your child must already have taken the sleep medication for at least one (1) month prior to joining the study. In addition, the dose of the sleep medication should be kept constant throughout the study period.

Children on a ketogenic diet may also be eligible for this study. Here, the conditions are that your child needs to stick closely to the ketogenic diet for a period of at least three (3) months prior to joining the study, and remain on it throughout the study period.

Our study has similar conditions around medication for epilepsy. It’s worth reaching out to the study team to find out which medications, and which dosing regimens, are compatible with participation in the study.

Global developmental delay. What does this mean?

A child with AS may show recognisable delays in the development of movement, speech and cognitive abilities from an early age, relative to their peers. These delays are global, meaning that they impact many aspects of brain functioning.

The onset of AS coincides with critical phases of early brain development and learning, causing disabilities that remain throughout life. Providing treatment at an early age may reduce the impact of AS on the developing brain.

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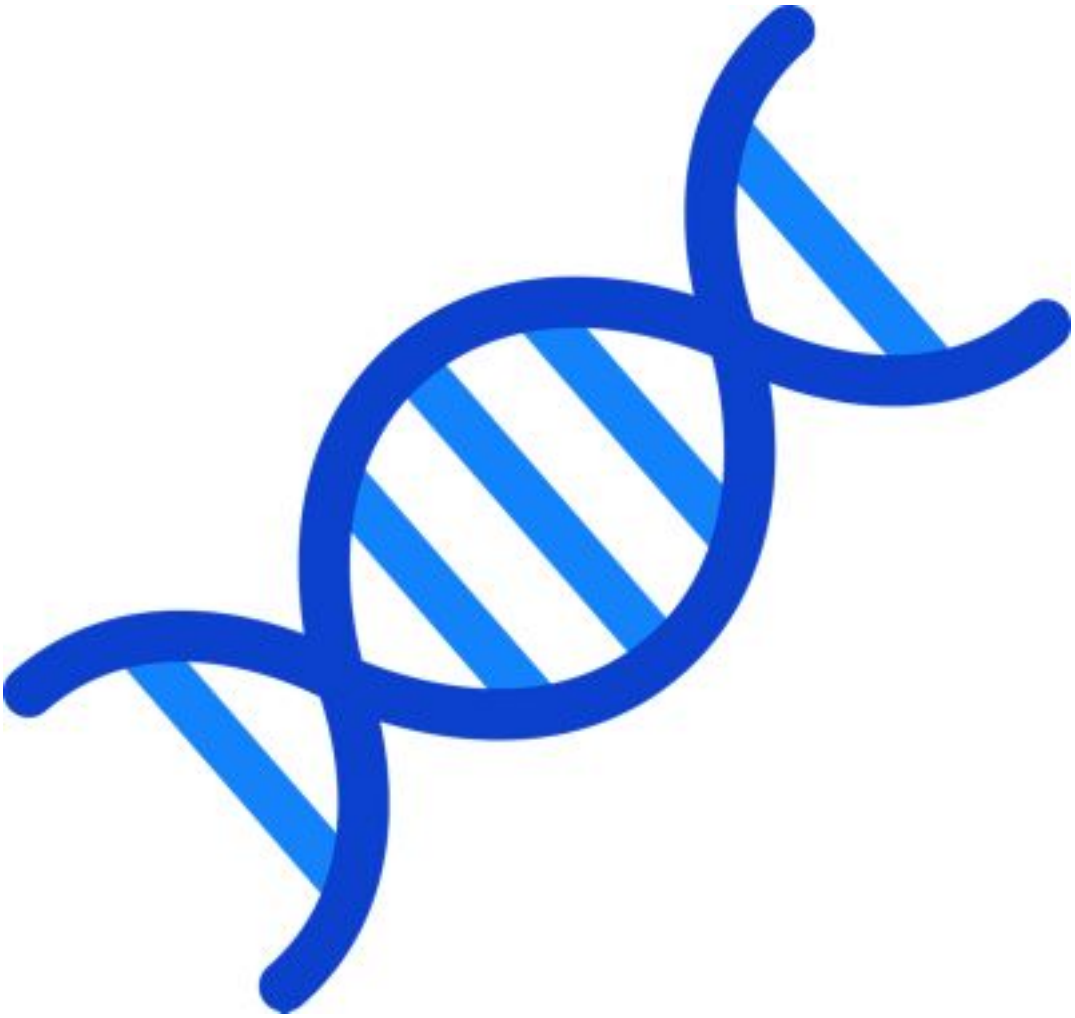

Why is the AS community so important?

Parents and other caregivers were involved in the design of this study from the very beginning. Patient advocacy groups gathered caregiver's views about the impact of AS symptoms relating to sleep, hyperactivity, seizures, self-care and inappropriate speech.

The study introduces a new measure of your child's communication ability. This measure was developed in collaboration with members of the AS community and reflects the importance of communication to the wellbeing of children living with AS, and to their families.

Caregivers who participate in this clinical study will gain firsthand experience with these measurements. You will be asked to record your experiences, and those of your child, in special diaries. Insights captured in these diaries will be included in the evaluation of the investigational therapy.

How is treatment given?

The investigational therapy is given in a special injection to the spine. The injection will be made while your child is under general anaesthesia. This procedure will need to be made two(2) or three(3) times during an 8 week period.

The new investigational therapy is given in a special injection to the spine. The procedure is made under general anaesthesia.

Your study healthcare team monitors and follows your child's treatment throughout the clinical study. They are available to answer your questions and are your point of contact if you have any concerns. Your existing healthcare team may also bring questions to the study doctor.

How often will my child be seen in follow-up appointments, and for how long?

The investigational therapy will be given over an eight (8) week period. During that time your child will have up to three(3) overnight stays in hospital following each injection procedure. In order to ensure the safety of your child the study team will schedule eight (8) follow-up visits ending with a final follow-up visit 12 months after treatment began.

The investigational therapy will be given 3 times over an 8 week period. The study team will schedule 8 follow-up visits ending with a final follow-up 12 months after treatment began.

Who has approved this clinical study?

This study is designed by physicians, scientists and the AS community. The study is carefully reviewed by the US Food and Drug Administration (FDA), for studies in the USA. Studies in Europe are reviewed by Regulatory Authorities in each country.

Every study is overseen closely until all participants complete their treatment and treatment follow-up, and until the study findings have been presented for a final review.

Who can answer my questions about this clinical study?

If you have further questions about your participation in the study, we suggest you bring them directly to the study medical team located nearest to you. Study teams have detailed knowledge to support parents and other caregivers participating in the study.

We encourage you to discuss clinical studies with your healthcare team, and with others who can support you to make decisions about care and treatment for your child.

Every clinical study is listed on a public registry.

This study can be found on the ClinicalTrials.gov public registry by searching the registry, using the Trial Identifier NCT04428281.

You can share the Trial Identifier with healthcare workers and with other members of the AS community.

How do I take part in this clinical study?

To join the study, healthcare professionals must carefully evaluate your child.

If your child is eligible to join the study and you give consent for them to enroll, they can join the study.

Tap **Find trial locations** at the top of this page for a list of hospitals nearest to you.

What happens if I am unable to take part in this clinical trial?

If it turns out that your child is not eligible for this study, the ClinicalTrials.gov website may have information about alternative clinical trials for your child.

Thank you for your time and interest in this clinical study.

Date of last medical review: June 2020.