

What do autism clinical trials mean for me?



Why is research important in autism?

Autism is a complicated set of core and associated behavioural characteristics, which can affect people in many different ways. Some autistic people find that certain characteristics are challenging to manage and require additional support.

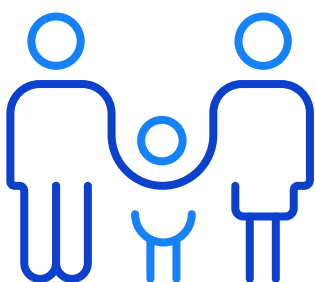
Medical interventions for associated characteristics and behavioral therapies can potentially improve development, function, and quality of life. These treatments do not aim to cure autism. Their aim is to help those who want or need them. However, medicines and therapies don't always work on core characteristics and are not effective for everyone.

Therefore, the autism research community need to do more research to understand which autistic people will benefit from safe and effective medication and therapies. To do this, they conduct clinical trials.

What is a clinical trial?

New medications and therapies are tested through clinical trials (also known as research or clinical studies). These involve volunteers who trial the new medication or therapy, usually compared against already available medication, therapy, or medication with no effect, known as a placebo.

Participants of trials receive a new medication or therapy and are closely assessed by a team of trained doctors, nurses, and research staff to see its effects. Data are collected from all the participants, which allow investigators (those running the trial) to accurately determine whether the medication or therapy is safe and effective.



Why do clinical trials matter to me, my family, and the autism community?

Clinical trials have enabled the wider scientific community to discover many different and essential medicines, like painkillers, antibiotics, and vaccines that we have today. This also means that the autism research community can find new interventions and therapies as their understanding of autism improves.

In autism, clinical trials aim to:



understand more about autism and how it affects people differently



appreciate how addressing certain associated autism characteristics can be beneficial for autistic people



discover medication and therapies that are effective, safe and work alongside other interventions

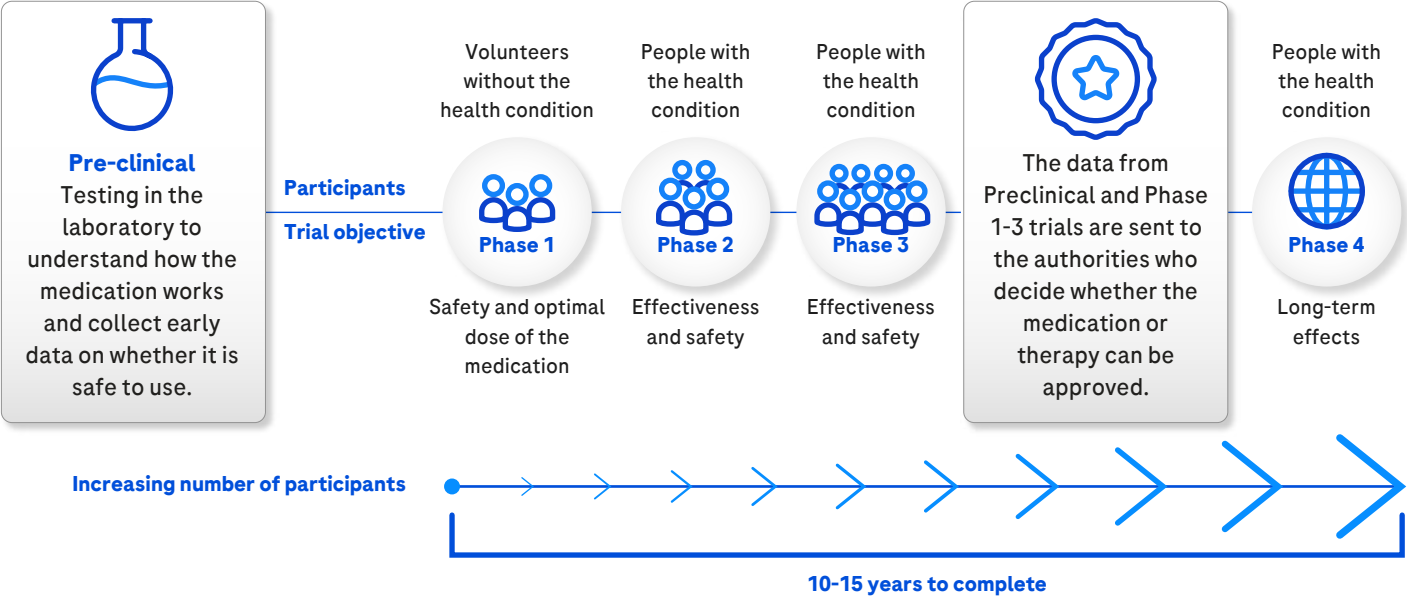


improve the lives of autistic people, their families, and supporters.

The clinical trial process:



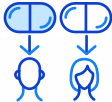




Every medication or therapy, including those for autism, goes through various Phases of clinical trials, each with different objectives, before it can be approved for use. Data found at each Phase determines whether research continues. Once the medication or therapy is approved and available, it will continue to be monitored for many years to investigate any potential long-term effects.

The diagram below describes a typical medication or therapy’s journey through a successful clinical trial process:



Key clinical trial terms

There are many different types of autism clinical trials. The following terminology explains some key words that you are likely to encounter regarding types of clinical trials and how they will be conducted.

 Standard of care	The medication or therapy that is already available outside of the trial. <i>At the time of print, no medication is available for core autism characteristics.</i>
 Placebo	A substance that has no effect but is designed to look the same as the trial medication or therapy and may be given to some participants. Placebos are vital in clinical trials to show if an actual medication or therapy works.
 Comparative trial	A type of study where one group receive the trial medication and other groups receive either the standard of care or placebo to compare how safe and effective the trial medication or therapy is.
 Randomised	Participants may be put into different groups, without knowing which, at the start of the trial. For example, one group could be given a certain dose of the trial medication and the other group could receive a different dose or a placebo.
 Double-blinded	Neither the investigators or the participants know which group is receiving the medication or placebo until after the trial finishes.
 Anonymised data	Your data collected throughout the trial cannot be linked back to you. All clinical trials will ensure that your data is anonymised.
 Open label extension	Once the trial finishes, all participants, regardless of whether they received the trial medication or placebo, are invited to take the trial medication for a further period to gain further data on how the drug works and how safe it is. <i>Not all trials will offer an open label extension.</i>



What should my family and I expect to happen during a clinical trial?

How a clinical trial is conducted will vary depending on factors like the Phase and location. However, you can usually expect the following stages to occur (your support person will be a part of each stage as well).

Recruitment

You may be approached by your doctor or a community organisation who think a clinical trial may be of interest to you and will describe it to you.

Screening

You will travel to the trial site, meet the staff, and ask any questions you may have. You will then undergo further physical tests, questionnaires, and possibly laboratory testing (e.g. blood or urine tests) or scans (e.g. MRI). Based on the results, you may or may not qualify to participate in the rest of the trial, without penalty.

Enrolment and group assignment

If you are selected for the trial, you will be assigned into different groups (often randomly) to receive the trial medication or therapy, standard of care, or a placebo, depending on the trial.

Analysis and completion

Your data, along with data from the other participants, are reviewed, analysed, and interpreted. The results are presented at scientific conferences, published online in scientific journals, and made available to participants, supporters, and the public (there is sometimes a cost to access).

Pre-screening

You answer a set of questions to help the trial organisers understand more about you and whether you will be suitable for the trial. These are typically done over the phone or online.

Informed consent

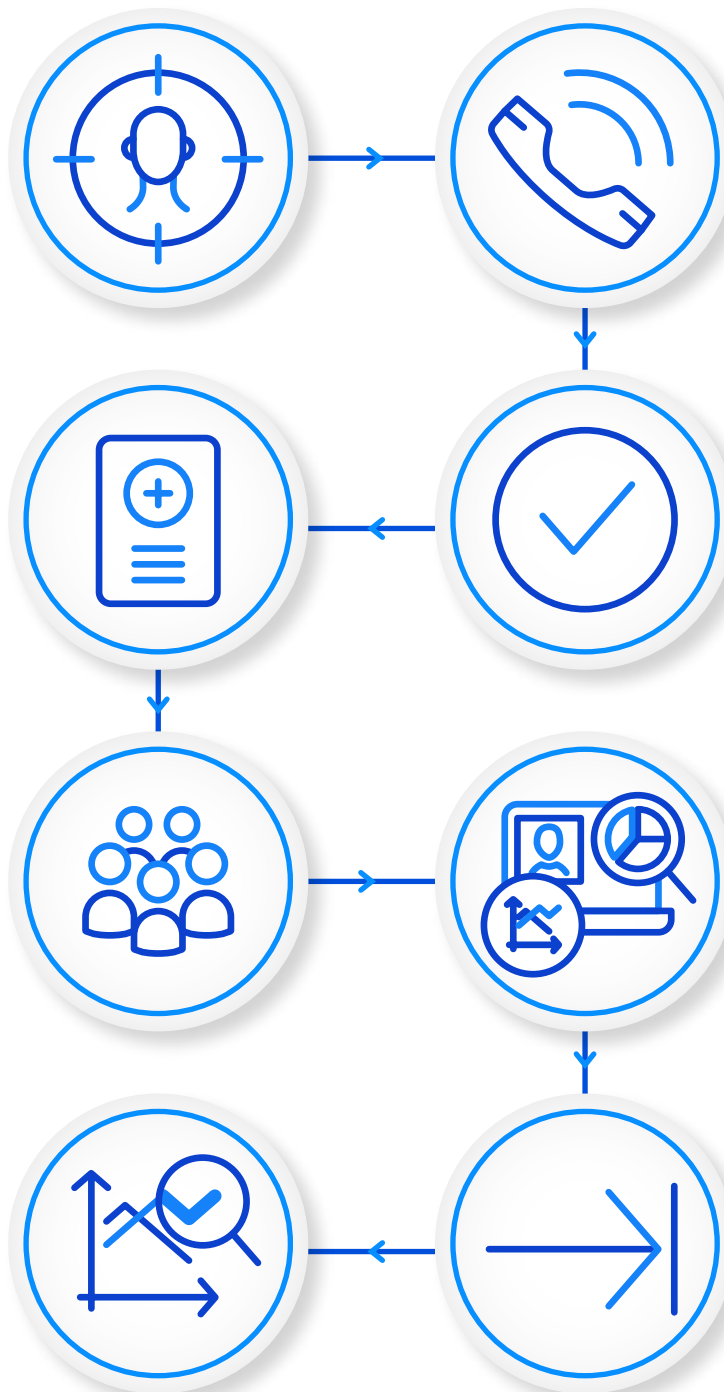
You are given all the facts about the trial, such as what the medication or therapy is, any expected side effects, the tests that you will be a part of etc. You can then decide whether you wish to participate by choosing to sign an informed consent form.

Study visits and monitoring

You will attend the trial site and receive different types of tests and questionnaires. You can ask any questions or raise concerns. (You may also be visited by trial staff, fill out questionnaires, or wear measuring devices at home, meaning you will not need to visit the trial site).

End of study

The trial ends and you may stop receiving the trial medication, therapy, or placebo. If there is an open-label extension, you may be invited to continue or start receiving the trial medication or therapy.



Clinical trials are conducted under very strict safety controls, and you will be regularly monitored to make sure you are well and healthy throughout.

You can leave the trial at any point for whatever reason, even after signing the informed consent form.



What will happen with my data?

Your data are absolutely essential to achieve the aims of the clinical trial. The infographic below explains why.



Your clinical trial data are important, but they can only show how you react to a medication or therapy.



When your data (which will be anonymised) is combined with that of hundreds or thousands of other autistic people, they provide a much more accurate idea of how many autistic people will react to a medication or therapy.



These large amounts of data are then analysed to show that the effects of the medication or therapy didn't happen by chance.



Your data could be key in ensuring a safe and effective medication or therapy is approved for use by other autistic people.



If you consent to it, your data could also become part of community registries, which securely collect massive amounts of information in order to further help improve the lives of autistic people now and in the future.

Data are collected continuously throughout the trial in many different forms, such as your results in tests, questionnaires, blood samples, vital signs like your pulse rate and blood pressure, brain activity, and DNA.

As part of the informed consent process, you should be clearly told how your data will be used and who will have access to it. Please raise any data concerns you may have with the trial organisers as early as possible.



What are the advantages and disadvantages for me and my family in joining a clinical trial?

You should take some time before deciding to join a clinical trial. While there are several positives of participating, there are also a number of potential negatives. It is important that you choose what is right for you.

The possible advantages of joining a clinical trial are:	The possible disadvantages of joining a clinical trial are:
receiving a medication or therapy that improves your life more than what's already available	receiving a medication or therapy that is no better or worse than what's already available, including a placebo
receiving the best available monitoring and care and more opportunities to ask questions	you may need to stop receiving a medication or therapy that you currently take
free care during the trial	having side effects
contributing to important research that can help you and other autistic people and their families in the future.	having tests that can be uncomfortable or scary
	time and travel commitments.



What important questions should I ask my doctor about clinical trials?

If you are interested in participating in a clinical trial, you should ask your doctor:

Are there any clinical trials that I can join?

If your doctor offers you a place on a clinical trial, you may want to ask the following questions.

What is the purpose of the trial?

Will I have to stop taking any medication that I already take?

How long will the trial last?

What if I want to leave the trial?

What is the treatment or therapy that you are investigating?

Who can I speak to if I have further questions?

What are the treatment or therapy's side effects?

Who should my family and I speak to if we want more information on clinical trials?

Speak to your doctor or local autism community organisation (if available) for more information on available clinical trials or if you have any questions about joining a trial.

Websites like ClinicalTrials.gov contain all current and future studies in autism, as well as trial details and results.

The following online videos explain the clinical trial process further.



Clinical Trials

<https://www.youtube.com/watch?v=5zXuON7Rueo>



What is a Clinical Research Study?

<https://www.youtube.com/watch?v=KCmfCWHMi5s>



The Importance of Clinical Trials

<https://www.youtube.com/watch?v=15hknL5m5W0>



Designing a Clinical Trial

<https://www.youtube.com/watch?v=vV6UzbZP3IU>



The Journey of Clinical Trials

<https://www.youtube.com/watch?v=u-2mkxhpm54>



Scan the QR code to watch the above videos on your device



This Clinical Trials Explorer can be freely downloaded, shared (copy and redistributed), and adapted (remixed, transformed, and built upon) in line with the Creative Commons Attribution-NonCommercial-ShareAlike 4.0 International license (CC BY-NC-SA 4.0), with full credit given to Roche and The Clinical Trials in Autism Council. Learn more about the license here: <https://creativecommons.org/licenses/by-nc-sa/4.0/>

Roche funded the production of this Explorer, and it was developed in collaboration with representatives of The Clinical Trials in Autism Council and the autism community. Members include independent representatives from the autism community, and representatives from the following organisations: Autism Science Foundation, ANGSA, Autism Europe, Malta Autism Parents' Association, Autismo Burgos and Selbsthilfe-autismus.

The linked videos in this Explorer are not Roche owned and sit under a separate Creative Commons license, but have been sourced from the European Federation of Pharmaceutical Industries and Associations (efpia), which represents the biopharmaceutical industry operating in Europe. These videos have been approved for use in this Explorer by the Clinical Trials in Autism Council. Learn more about efpia here: <https://www.efpia.eu/about-us/who-we-are/>