



A Participant's Guide to Autism Drug Research

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Purpose of this guide

The decision to become a participant in drug research is an important one, all the more so if you are making this decision for your child or other dependent. It is an option that is becoming increasingly available to individuals and families affected by autism spectrum disorder (ASD). This is because research into the causes and biology of autism is advancing in ways that make it feasible to identify drugs that may relieve disabling symptoms.

The goal of this guide is to both answer common questions and pose additional ones that may be important for you to consider. The decision of whether or not to participate in autism drug studies should be guided by your goals and values as well as a clear understanding of potential risks and –

benefits and possible demands upon you, your child and your family. We encourage you to jot down questions that may arise as you review this material – questions that you may want to discuss with your family, personal physician and the researchers leading the study you are considering.

What is clinical drug research?

Clinical research involves humans. Some early clinical studies involve unaffected volunteers whose participation enables scientists to understand how the human body processes a drug. Other clinical trials generally involve participants who are affected by symptoms that the experimental drug may relieve.

Until recently, most research in the field of autism has involved collecting information on individuals and families affected by this disorder of brain development. Such studies are crucial to better understanding autism's causes and underlying biology. This research has now advanced to the point where scientists are identifying biological mechanisms that can be targeted by experimental drugs – the goal being to relieve disabling symptoms. Generally, these experimental compounds are first tested in animal models to gather preliminary evidence of effectiveness and safety. Early drug research can also involve laboratory studies using human cells and tissues – either cultured or donated after death.

Only after considerable tissue and/or animal research do the best drug candidates move into clinical trials with human volunteers. Typically these studies start off small. If evidence of safety and effectiveness continues to build, a drug may then move into studies with larger numbers of participants. Larger studies are vital to ensure that a perceived benefit is real and not the result of chance or some unidentified influence.

The National Institutes of Health (NIH) and the U.S. Food and Drug Administration (FDA) both have strict rules for promoting drug trial safety and obtaining the informed consent and assent of participants. In this context, “consent” refers to permission that is typically provided by an adult research participant. In the case of a child participant, “consent” can be provided by a parent or other legal guardian. In addition to a guardian’s consent, it is also important to obtain a child’s “assent,” or agreement to participate.

To recap: Both parental consent (permission) and a child’s assent (agreement) are necessary before a child can participate in clinical drug research. This should be documented in writing by those overseeing the research. Additional ethical considerations arise when study participants are nonverbal or have significant intellectual disability. In these cases, it is particularly important for legal guardians, researchers and, if necessary, other advocates to work together to safeguard the well being of these vulnerable participants and respect their wishes to the extent possible.

Sometimes, study participation requires changes in the volunteer’s current therapies. Some studies require participants to be monitored with techniques such as brain scans, behavioral observation, blood samples and other tests. Clear information about these requirements should be part of the “informed consent” that all study participants and/or their legal guardians should receive and review prior to joining a study.

Why participate in clinical drug research?

Many people participate in clinical drug research with the hope of receiving an advanced treatment. At present, we have no FDA-approved drugs for relieving autism’s core symptoms of social and communication impairment and repetitive behaviors. The FDA has approved two drugs for treating autism-related irritability (aripiprazole and risperidone), and many families report that these can improve sociability while reducing tantrums, violent outbursts and self-destructive behaviors.

Clearly the decision to take autism-related medications – whether they are experimental or FDA approved – is a highly personal one. For this reason, Autism Speaks Autism Treatment Network has created a decision aid – [*Autism: Should My Child Take Medicine for Challenging Behavior*](#) – available for free download. (Follow the hyperlinked title, or go to <http://www.autismspeaks.org/science/resources-programs/autism-treatment-network/tools-you-can-use/medication-guide>.)

At the same time, we know there is great interest in new medical treatments for core symptoms, particularly for those who are severely affected. There is likewise interest among high-functioning adults who struggle with autism-related difficulties that they feel lower their quality of life (e.g. problems with social anxiety, attention, concentration or sleep).

However, it is important for the person or family who enters clinical research to balance the desire for advanced treatment against a number of factors that separate clinical research from conventional medical treatment. These are described in more detail in the section below.

Importantly, participation in clinical research offers individuals and families the opportunity to help advance the development of treatments that can improve the lives of all those affected by autism. New medicines cannot be brought to market without clinical trials and those who volunteer to participate in them. Along these lines, researchers may request permission to collect biological samples such as blood or saliva for genetic testing and future research. You are in no way obligated to provide such samples, but by doing so you can further advance research into the causes and treatments of ASD.

How is participating in research different from seeing a doctor?

While every researcher has the responsibility of safeguarding study participants, enrolling in clinical research is not the same as seeing a personal physician. Here are some of the key differences to consider:

Key differences	Participation in Clinical Research	Care from a Personal Physician
Primary purpose	The researcher’s goal is to learn about your illness.	Your doctor’s goal is to treat your condition.
Treatment flexibility	The researcher must use standardized procedures.	Your doctor will change/customize your treatment as needed.

Possibility of dummy treatment	You may be randomly assigned to a group taking a standard treatment or placebo (an inactive pill) versus the group taking the new treatment.	The patient always receives an active drug, typically the best standard treatment available.
Level of medical knowledge	The safety and effectiveness of experimental medicines are not fully known.	Typically, physicians prescribe FDA-approved medicines whose side effects and benefits have been studied. However this can involve “off label” use, which means the medication may not have been tested in the context of the patient’s particular condition or age group.
Additional procedures and time commitments	You may be asked to undergo procedures (blood tests, imaging scans, etc.) and complete questionnaires important for research but not necessary for your care.	Testing procedures and questionnaires are restricted to those necessary for your medical care.
Costs	Medical and other costs associated with participation may be partly or fully covered.	You are likely to be responsible for treatment costs and/or the cost of health insurance coverage/co-pays, etc.
Altruistic benefits	Participating in research provides the opportunity to advance scientific understanding and help others.	Your treatment is focused on your needs, not that of others.

What do researchers mean by clinical trial “phases”?

Clinical trials are often conducted in four, graduated phases. Each of these four phases has a different primary purpose.

Phase I trials

Phase 1 trials tend to be relatively small in the number of participants, and these participants may or may not be affected by the condition the drug is hoped to relieve. The primary goal is to evaluate whether an experimental medicine is safe and well-tolerated and to identify potential side effects. Often these trials examine different doses to determine which works best. Building confidence in safety is key.

Phase II trials

The design of phase 2 trials is guided by the knowledge of safety, tolerability and dose gained in phase 1 trials. The goal of a phase 2 trial is to determine whether an experiment agent shows promise in alleviating a specific condition or symptoms. For this reason, these trials enroll volunteers who are affected by this condition or symptoms. Phase 2 trials usually involve more participants than do phase 1 studies. This increases the researcher's ability to detect benefits and potential side effects and also increases reliability of their results. To gauge the drug's ability to relieve symptoms, researchers typically apply tests called "outcome measures" throughout the trial. If these measures show that the treatment produces significant benefits, then it is said that the study achieved "proof of concept." In essence, this means that there is sufficient reason to believe that the treatment will have real-world benefits and has the potential to become a drug used in medical practice.

Although encouraging, phase 2 "proof of concept" is not, by itself, sufficient to support a treatment's approval for medical use. Further study is needed to assure safety and effectiveness.

Phase III trials

The goal of phase 3 trials is to provide definitive evidence that an experimental drug has real-life benefit for a particular group of patients. Guided by the findings of phase 2 trials, researchers provide the experimental treatment to still-larger groups of volunteers who represent the broad population of individuals affected by the disorder or disease being studied. Phase 3 trials can also refine the most effective dose for one or more groups; better identify the symptoms most responsive to the medicine; improve detection of side effects; and compare the medicine's benefits and side effects to those of commonly used treatments.

This information is vital to refining what is known about a drug that may eventually be introduced into widespread use. If the safety and benefits documented in a phase 3 trial confirm and expand confidence in a high level of safety and effectiveness, the next step becomes application for approval by the FDA.

Phase IV trials

The goal of phase 4 is to ensure that safety and effectiveness continue to be monitored after the FDA approves a medicine for use. These "post-marketing" studies generally involve newly approved drugs and gather additional safety and effectiveness information as more and more people use the drug under the care of their personal physicians.

What questions should I ask the researcher?

First, never hesitate to ask questions! Study participants and their families become essential partners in research. So it's important for you to understand what will be asked of you and how those conducting the study will safeguard your health or the health of your loved one. We encourage you to ask the study physician and his or her staff questions *before* you agree to participate as well as any time afterwards. Importantly, the researchers should welcome your questions and answer them fully. Below are some suggested questions. Feel free to add your own.

- What previous studies have been done on this medication and to what degree has this clarified its risks and benefits? (See explanations of phase 1, 2, and 3 trials in previous sections.)
- How might this study help me or my child?
- What side effects are possible?
- What comparable standard treatments are available?
- Is there a chance that I or my child will NOT receive the experimental treatment (become part of a “control” group)?
- Will this study involve any change in my or my child’s current medications or other treatments? If so, what risks does this pose?
- How will participation affect my daily life and/or that of my child? (What tests will I or my child be asked to undergo and how often? What discomfort or risks, if any, do they pose? What steps are being taken to reduce the inconvenience, discomfort or risks associated with this testing?)
- What measures are being taken to ensure the health and safety of participants?
- How long will the study last, and how often will we need to visit the research clinic?
- What should I do/what will happen if I or my child experiences side effects or begins to feel worse during the trial?
- Has the design of this study been assessed by an independent ethics committee? What were their recommendations for improving the likelihood of benefits and reducing risks for participants, and how were these suggestions implemented?
- Are participants asked to bear any costs? What related costs (travel, lodging, etc.) will be reimbursed?
- Will you support my/our decision to withdraw from the study at any time?
- Will my family member be allowed to continue receiving the experimental medicine after the trial has ended?
- How will this research help other persons affected by ASD?

Don’t hesitate to ask for further explanation if you don’t understand or are unsatisfied by the answers you receive. If you forget to ask a question or forget the answers, ask again! Researchers have the responsibility to help you understand. Be wary if you feel they are less than forthcoming or impatient. You also have the right to directly contact the study’s ethics committee or institutional review board with any concerns. Their contact information should be in the informed consent form.

What rights do all participants have?

“Informed consent” is vital to participation in clinical studies, and you will be asked to read and sign a related document. At a minimum, it should include the following information, which should be clarified for you, as needed, by the researcher(s).

- A clear explanation of all significant risks and benefits
- Assurance of confidentiality (you and your child’s identity and personal medical information must be protected; medical data from the study should be anonymous, or “de-identified.”)

- A clear explanation of how the study will be carried out and what is expected of you or your child as a participant, including the location and length of the study, and what each visit is likely to involve.
- Any anticipated costs to you or your health insurer
- Details of expense reimbursement and/or financial compensation

After you join a clinical study, you have the right to:

- Leave the study at any time. Though you should not enroll if you/your child do not plan to complete the study, you retain the right to refuse any request and/or withdraw.
- Receive any new information that may affect risks, benefits and your decision to continue your participation
- Continue to ask questions and get answers
- Maintain your privacy
- Once the study is completed, find out about your treatment assignment (experimental/active or control). This concerns “blinded” studies that randomly assign participants to one or more treatment and/or control groups without disclosing the assignment to the researchers or participants until the results have been analyzed.
- Ask who you can contact – outside the research team – if you have concerns that are not being addressed or have misgivings about how the study is being conducted.

What rights do children have when participating in research?

Only legal guardians can give informed consent for a child to participate in a clinical trial. However, whenever possible and appropriate, ethics and regulations require that a child’s participation be informed and voluntary. The evaluation of a child’s ability to give assent, or agreement, should not be based solely on age, but also other factors such as developmental stage and intellectual and/or psychological maturity. Researchers should provide separate information sheets for adult guardians and children, each prepared with age-appropriate language.

Importantly, objections raised by a child at *any time* during a trial should be considered and respected. Children should not be forced to provide reasons for their objections. They should be informed of their right to freely withdraw from a trial at any time for any reason. When providing children with the above information (at an appropriate developmental level), their responses should be acknowledged and documented by the researchers.

Our pledge to you

We, at Autism Speaks, are dedicated to improving the lives of all who struggle with autism. As more potentially helpful medications enter the research pipeline, we will continue to work with families, individuals and the field’s most respected experts to ensure the safety, well being and best possible outcomes for study participants.

We also pledge to be available to you, the members of our community, if you have further concerns or questions not addressed here – now or in the future. We also welcome your comments and feedback. Please reach us via GotQuestions@autismspeaks.org or through the Autism Speaks Autism Response Team at 888-AUTISM 2 (288-4762).