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Learning About Clinical Trials

A Guide for Individuals and Families
Affected by Spinal Muscular Atrophy (SMA)



EXPERT REVIEW PANEL

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INTRODUCTION

Clinical trials for spinal muscular atrophy (SMA) are helping researchers answer important questions about the disease and investigational drugs, providing information that may help the development of future medical treatments. For many individuals and families affected by SMA, clinical trial participation may be a consideration, especially in the absence of an approved treatment for the disease.

Clinical trials are conducted to determine if investigational treatments (such as new drugs, procedures, or medical devices) are safe and effective. The process can be a long and complicated one – with many steps in place to protect the safety of trial participants. This brochure is designed to help explain what a clinical trial is and what participation may involve. It is also designed to clear up some common misconceptions about clinical trials so that individuals and family members affected by SMA can make an informed decision about participation.

It is natural to have questions about clinical trials. If after reading this brochure you would like to learn more, please speak with your doctor or contact Cure SMA.

Cure SMA is dedicated to supporting the SMA community through the advancement of research. This includes funding and directing comprehensive research programs in an effort to develop a treatment for SMA (cureSMA.org/clinicaltrials).

Clinical Trials Overview

Clinical trials are conducted to test investigational drugs so that the regulatory authority in your country can decide whether or not they can be approved for use as a treatment in the future. Researchers must prove scientifically that an investigational drug is safe and effective in order for it to be submitted for approval. Each country or region has its own regulatory authority. In the United States, the regulatory authority is the Food and Drug Administration (FDA).

To help generate scientific data showing safety and efficacy, researchers may test an investigational drug for SMA:



- at different doses, at different frequencies, or for a different length of time
- against a placebo (an inactive substance designed to have no effect on health) or a sham (a procedure that mimics the actual procedure but does not include the actual delivery of the investigational drug)
- by itself

Deciding to participate in a clinical trial can be a difficult decision, and one that may involve many considerations. Participation is always voluntary, and you or your family member can withdraw at any time and for any reason (or no reason at all) – and doing so will not affect the care provided now or that may be provided in the future.

Several clinical trials are currently taking place for SMA, many evaluating investigational drugs designed to potentially help manage or slow disease progression.

[ClinicalTrials.gov](https://clinicaltrials.gov) is a U.S. registry of clinical trials where you can find several listed – just type “spinal muscular atrophy” or “SMA” into the search field. You can also find the latest information about SMA trials on the Cure SMA website (cureSMA.org/news).



A COMMON MISUNDERSTANDING

Receiving an investigational drug as a trial participant does not guarantee improvement of symptoms. In fact, there is no guarantee that the investigational drug will have any effect – it may even make symptoms or the progression of a disease worse or have unwanted side effects. In some clinical trials, participants will not receive the investigational drug at all – they will receive a placebo or sham (see Glossary of Terms), which are designed not to have any effect on the disease.

There are also natural history studies that just collect health information. They do not involve the administration of an investigational drug; rather researchers focus on observing the natural progression of the disease.

GLOSSARY OF TERMS

This brochure contains many terms that may not be familiar to you. If you would like to learn more about some of them, please go to the Glossary of Terms at the end of this brochure.



Who Regulates Clinical Trials?

Each country has its own regulatory authority with rules, or laws, for conducting clinical trials. The regulatory authority reviews and approves the protocol (a detailed plan for the trial), and ensures that the clinical trial follows national regulations. Regulatory authorities make sure that the rights of participants are protected by requiring that all clinical trials are approved and monitored by special committees. In the United States, these committees are called Institutional Review Boards (IRBs), and in other countries they are called Ethics Committees (ECs). These committees are responsible for:

- Ensuring that steps are taken to protect the rights, safety, and welfare of participants.
- Reviewing and approving the protocol, informed consent forms, recruitment methods, and all written information provided to participants.
- Monitoring aspects of a clinical trial and requiring that serious adverse events are reported.

WHAT IS A CLINICAL TRIAL PROTOCOL?

All clinical trials follow a detailed plan, which is called a “protocol.” It explains the purpose of the trial as well as many details regarding how it will be conducted. It includes information about:

the reason why the clinical trial is being conducted	questions researchers are trying to answer	the population that will be studied	planned medical tests and procedures
the investigational drug(s), dosages, and frequency	timeframe and schedule	how the health and safety of participants will be monitored	information on known side effects or risks

Before an individual research center or hospital can participate as a clinical trial site, the protocol and documents that will be provided to participants must be approved by an institutional review board (IRB) or ethics committee (EC). These are independent committees responsible for protecting the rights, welfare, safety, and well-being of clinical trial participants.

How Is the Clinical Trial Population Chosen?

All clinical trials have standards about who can and cannot participate. These are called inclusion / exclusion (or eligibility) criteria, and they help researchers establish a group of participants that share similar qualities. This makes it easier and quicker for them to evaluate the investigational drug and compare its effect, if any, among participants – potentially leading to a faster review and approval. Inclusion / exclusion criteria also help researchers identify individuals who can safely participate in the clinical trial.

In SMA clinical trials, common eligibility criteria may include:



Principal investigators and their study teams evaluate potential clinical trial participants through a “screening process,” during which:

- the patient’s medical history is reviewed
- medical tests and evaluations may be performed
- a discussion with the potential participant (or family members) may occur to help the principal investigator make a decision about whether the patient’s status and medical history meet the eligibility criteria for participation. (The final decision is made by the principal investigator.)



It is important to understand that not everyone interested in a clinical trial may have the opportunity to participate. This could be because the status and the medical history of a patient might not meet the eligibility requirements. It is also possible that the trial has already enrolled the required number of participants. Even if you or your family member is not able to participate in a specific clinical trial after completing the screening process, the principal investigator may be able to discuss the possibility of participating in a future clinical trial.

HEALTHCARE CONSIDERATIONS

Basing a current healthcare decision on the potential to participate in a current or future clinical trial is not advised.

Healthcare decisions should be based on the current medical situation. It is never recommended to avoid treatment or care because you believe it may prevent you or your family member from being able to participate in a clinical trial. There is no guarantee that doing so will make you or your family member eligible – and, in fact, some healthcare measures may be required in order to participate.

Who Is Involved in Conducting Clinical Trials?

There are many people involved in planning, organizing, and conducting a clinical trial. They may include:

CLINICAL TRIAL SPONSOR

A clinical trial sponsor can be a company (e.g., pharmaceutical or biotech), a non-profit institution, or a government organization. The sponsor initiates, manages, and funds a clinical trial.



COMPANY



NON-PROFIT
INSTITUTION



GOVERNMENT
ORGANIZATION



REGULATORY AUTHORITY

Each country has its own regulatory authority with its own regulations, or laws, for conducting a clinical trial. The regulatory authority reviews and approves the protocol, and ensures that the clinical trial follows national regulations.

- In the United States, the regulatory authority is the Food and Drug Administration (FDA).

INSTITUTIONAL REVIEW BOARD (IRB) / ETHICS COMMITTEE (EC)

An IRB or EC is an independent committee that includes medical, scientific, and non-scientific members, whose responsibility is to protect the rights, welfare, safety, and well-being of clinical trial participants. Each clinical trial location is monitored by a specific IRB / EC. They are responsible for reviewing and approving all clinical trials, as well as conducting ongoing reviews of active clinical trials.

DATA AND SAFETY MONITORING BOARD (DSMB)

The DSMB is an independent group, or committee, of experts that monitors patient safety and treatment efficacy while a clinical trial is being conducted. The DSMB plays a critical role in the administration of clinical trials.

PRINCIPAL INVESTIGATOR

The principal investigator is often a medical doctor who is responsible for managing a clinical trial at an individual research center or hospital. The principal investigator is sometimes called the “study doctor,” and he / she is usually helped by other doctors, nurses, and clinical research coordinators who are part of the study team.



SMA ADVOCACY GROUPS / NON-PROFIT ORGANIZATIONS

These groups and organizations play an important role in the SMA community by:

- funding research to advance medical knowledge and potential treatments
- serving as a resource for current information on clinical trials
- providing a network for families seeking to connect with the SMA community

The Four Phases of Clinical Trials



Developing a new medical treatment is a long and complex process, averaging 10 – 15 years from start to finish. This is because there are several steps in place that are designed to evaluate whether an investigational drug is safe and effective before it is approved by a regulatory authority.

Once an investigational drug is identified, pre-clinical testing is performed in the laboratory before it can be tested in humans. This initial testing may take several years. Approximately one in every 1,000 investigational drugs will make it to human testing.

Following the completion of each phase, the sponsor will evaluate the results and decide if the investigational drug will advance to the next phase.

More than 90% of investigational drugs that enter clinical trials do not end up being approved for use because of:

- safety issues
- lack of effectiveness
- manufacturing issues (e.g., technical issues related to not being able to make enough of the investigational drug or not being able to make it reliably)

EXTENSION TRIALS

Some clinical trials have an extension trial that participants may be eligible to participate in following the completion of the main trial. In most cases, during an extension trial, all participants receive the investigational drug over an extended period of time so that researchers can study its long-term effects.



AN OVERVIEW OF THE FOUR PHASES



The investigational drug is tested in a small group of people to evaluate its safety, determine a safe dose, and identify side effects. Sometimes phase 1 trials involve a group of healthy volunteers which helps researchers establish normal measurements and assess the natural history of the disease. This phase is not designed to answer questions about whether or not the investigational drug is effective for SMA; however, it still may capture this data.



The investigational drug is given to an increased number of people with SMA. Researchers look to identify initial signs that it may be effective and further evaluate its safety.

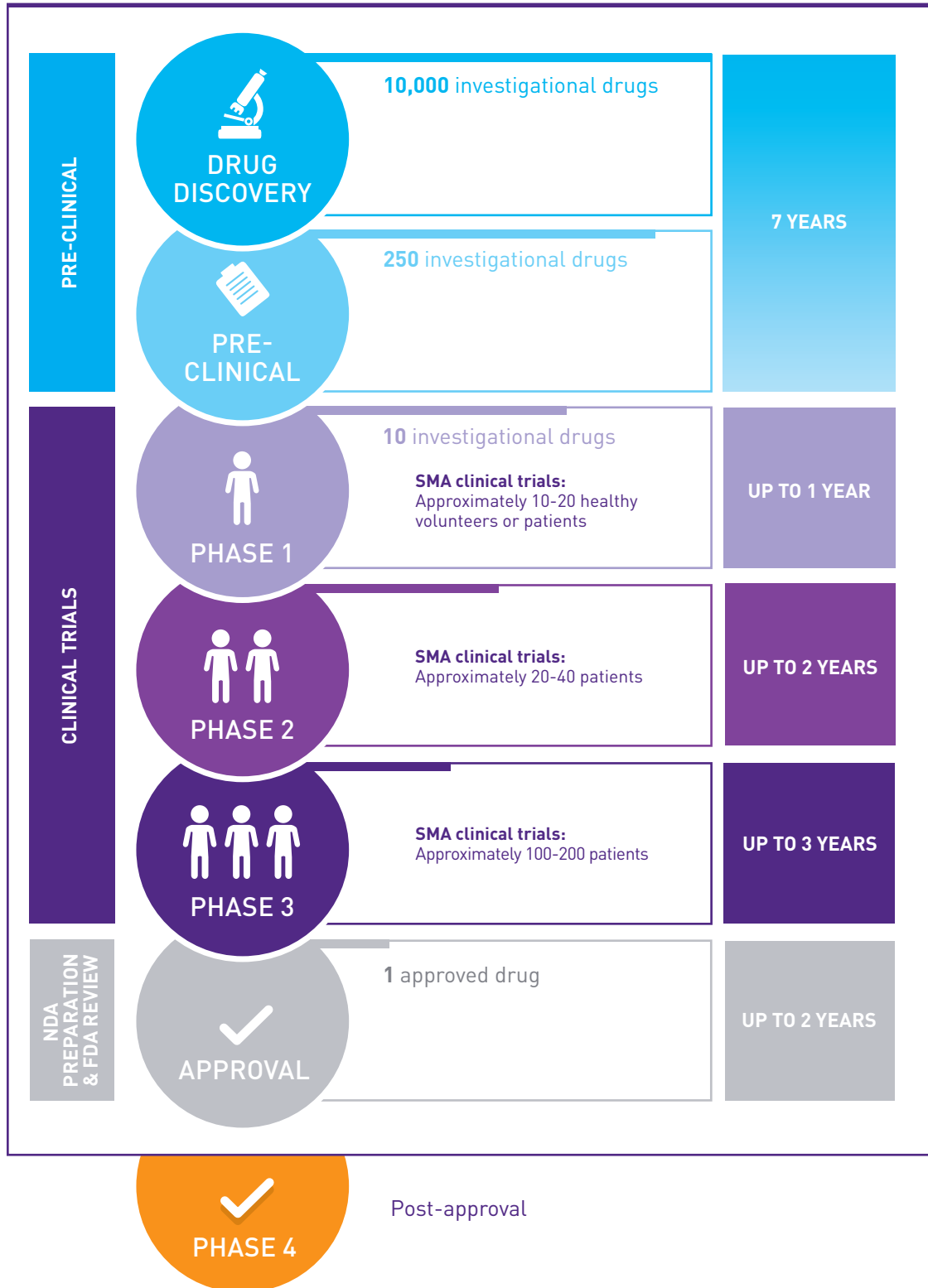


The investigational drug is given to an even larger number of people with SMA to confirm its effectiveness, monitor side effects, and collect information that will allow the investigational drug to be used safely. During this phase, the investigational drug is often compared to a placebo or sham.



After an investigational drug has been approved and made available to the public, researchers gather information on its effects in various populations and any side effects associated with long-term use.

THE CLINICAL TRIAL PROCESS EXPLAINED





FASTER METHODS

OPTION 1 ACCELERATED APPROVAL

The FDA has regulations that allow for faster approval of drugs that treat serious conditions or that fill an unmet medical need. The approval is based on an endpoint, or measurement, that predicts how well the drug may work.

OPTION 2 FAST-TRACK

Fast-track is a process designed to shorten the time to develop and review drugs that treat serious conditions and that fill an unmet medical need. This process increases communication between the FDA and the drug company to resolve questions.

OPTION 3 BREAKTHROUGH THERAPY

The FDA can accelerate the development and review of drugs – based on findings from early clinical trials – which may show a substantial improvement over other available treatments.

OPTION 4 PRIORITY REVIEW

A Priority Review means the FDA will try to take action on a new drug application within 6 months, instead of 10 months which is the standard review. This may be an option for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety and effectiveness over current treatments.

Accelerating the Approval Process

Sometimes an investigational drug may be moved through the clinical trial review and approval process more quickly than others. Drug companies have the opportunity to apply for different designations and processes at the FDA that, if granted, help speed up the review and approval process. These opportunities are available for investigational drugs that address diseases of high unmet need, like SMA, and show substantial improvement over available treatments based on scientific data.

ORPHAN DRUG DESIGNATION

SMA is classified as an orphan disease because it affects fewer than 200,000 people in the United States. Drug companies can apply for orphan designation for an investigational drug designed to treat a rare disease. For a drug to qualify, both the drug and the disease must meet certain criteria. Orphan designation qualifies the drug company for various incentives for development. The granting of an orphan designation request does not affect the requirements and process for obtaining approval from the regulatory authority. Safety and effectiveness must still be established through well-controlled trials.

What Is a Placebo (or Sham) and Why Is it Used?

Some clinical trials include the use of a placebo or a sham to help researchers evaluate the effect, if any, the investigational drug has on SMA symptoms. (These are commonly used in phases II and III clinical trials.)

- A placebo is designed to look like the investigational drug but has no active ingredients.
- A sham, or “sham procedure,” mimics the actual surgical procedure (including pre-procedure routine, anesthesia, and post-procedure follow-up), but does not include the actual delivery of the investigational drug to the patient.

In randomized controlled clinical trials, some participants will receive the investigational drug and some will receive the placebo, or sham. Researchers compare the results of the group receiving the investigational drug to the group receiving the placebo, or sham, to determine if the investigational drug is having the desired effect.

Using a placebo or sham helps to speed up a clinical trial as researchers can more quickly observe any differences between the groups. Natural history studies, in comparison, can take researchers much longer to conduct since they must determine if a difference between groups is real – without the help of a placebo, or sham.



THE PLACEBO EFFECT

During clinical trials, participants may show improvements or side effects simply because they think they are being treated. This is called the placebo effect and it usually lasts only a short period of time but it can have a significant effect.

RANDOMIZATION

The process that determines whether a participant will be assigned to the group receiving the investigational drug, or to the group receiving the placebo, or sham, is called randomization. This process is done by chance, like flipping a coin. In some clinical trials, neither the participant (nor family, if appropriate), nor the principal investigator knows to which group the participant has been assigned. This is called “double-blind,” and it is intended to eliminate potential bias on the part of the principal investigator. In other clinical trials, called “blinded,” only the participant does not know to which group he /she has been assigned.





To help advance knowledge about SMA



To contribute to medical research and to the development of a potential treatment



To help others affected by SMA in the future



Potential access to an investigational drug



Appointments with the SMA study team



Clinical trial-related care, monitoring, medical tests and assessments. [Participants will continue to receive standard medical care from their primary doctor.]

Why Consider Participation?

Individuals and families affected by SMA may choose to participate in clinical trials for a variety of reasons, including:



HOW DO I LEARN ABOUT THE POTENTIAL RISKS AND BENEFITS OF PARTICIPATION?

Clinical trials are investigational in nature. There is no guarantee that participating in one will provide a medical benefit, and in fact there may be risks involved – some known, some unknown. This is particularly true for phase I trials when the investigational drug is tested in humans for the first time. Although efforts are made to control potential risks, some may not be avoided because they may not be known.

When potential risks are known, they must be fully explained by the study team to potential participants (or family members, depending on the age of the participant). They may include unpleasant or even serious side effects. If new risks are learned during the trial, this information must be shared as well.

Participants may or may not experience a benefit from the investigational drug. However, there may be other potential benefits to consider that include:

- contributing to SMA medical research
- clinical trial-related care and monitoring of SMA
- potential access to an investigational drug

As part of the decision-making process, you will be given a chance to discuss the potential risks and benefits with the principal investigator. You should consider them as part of your decision, and you should make a decision only after having a full understanding of the risks that may be involved and what will be required of you or your family member as a participant.

WHAT IS THE INFORMED CONSENT PROCESS?

If you are interested in participating in a clinical trial for SMA, you will be given an opportunity to ask the principal investigator questions. You will be provided with an Informed Consent Form that explains the details of the trial, including its purpose, length of time, required procedures, key contacts, and any possible benefits and risks. You may take the form with you to review and discuss it with family members or friends while you consider participation.

If you decide to participate, you must provide your consent by signing the form. This process is called the informed consent process, and it is a standard process for participation in a clinical trial.

As you consider your decision, please remember:

- Participation in a clinical trial is always voluntary and you can change your mind at any time.
- Deciding not to participate will not affect care that may be provided now or in the future.
- Participants may withdraw at any time and for any reason – doing so will not affect the care they would normally receive outside of a clinical trial.
- The principal investigator is required to inform clinical trial participants of any new developments that may affect or influence their decision to participate. The IRB / EC plays a role in overseeing clinical trials to make sure this occurs.

For some individuals, it is a possibility that their participation may be ended, without their consent, if they become too sick or other medical issues develop.

SPECIAL CONSIDERATION FOR CHILDREN

If your child is 7 years of age or older, they may be asked by the principal investigator if they agree (or “assent”) to participate in the clinical trial. Not all trials require assent. For those that do, the age when it is requested can vary depending on the trial, as well as the requirements of the research center or hospital and the country where the trial is being conducted. However, it is important to discuss participation with your child if they are at an age that they can understand.

WHAT ARE THE RESPONSIBILITIES OF PARTICIPANTS?

There are certain responsibilities that clinical trial participants (or family members, depending on the age of the participant) are asked to follow.

These may include:

- Following all instructions given by the study team.
- Attending all scheduled visits.
- Completing questionnaires about the status of the participant between visits.
- Telling the principal investigator of any new health-related problems. (Even if you don't consider them to be caused by the clinical trial or the investigational drug, any small change is very important to report.)
- Telling the principal investigator about any new medications or changes in doses or the frequency of medication.
- Being mindful about discussing the clinical trial with other participants, including whether or not you think you or your family member may be receiving a placebo or sham.

SOCIAL MEDIA

You are encouraged to limit your discussions about clinical trial participation to family members, close friends, and doctors. It may be tempting to share information about your experience on Facebook, Twitter, or YouTube, but doing so can affect the results of trials.

Clinical trials are designed to allow for the unbiased collection of data. Posting your information online could influence others and could result in over-reporting or under-reporting of the side effects of the investigational drug. Sharing information in this manner could unintentionally unblind the trial, revealing if participants are receiving the investigational drug or placebo / sham. If this happens, it could compromise all of the trial data.

If you have questions about what may be appropriate to share, please speak with a member of the study team.



SOME CLINICAL TRIALS MAY INCLUDE TRAVEL SUPPORT

Some SMA clinical trials may include travel support and reimbursement for parking, meals, and other expenses. This may be helpful for families that need to travel a long distance to the clinical trial location, or that have a lengthy appointment or overnight stay. It is important that you understand the kind of travel support that will be available to you before you make a decision to participate.

For some children that are extremely weak, it may be recommended that they do not travel. It is best to speak with the study team about your individual situation.

CONSIDERATIONS FOR PARENTS OF CHILDREN WITH SMA

Organizing and managing clinical trial appointments can sometimes be a challenge. The tips below may be helpful if you are a parent of a child participating in an SMA clinical trial:



- With the help of the study team, develop a calendar showing key study appointments and telephone calls so that you are prepared in advance.
- Depending on the age of your child, consider bringing toys or games to keep them busy while waiting to meet with the study team. (These may include books, puzzles, games, colored markers and paper, etc.)
- If you are traveling by plane and your flight has been cancelled or delayed, please let the study team know so that they can adjust the time of your appointment.
- Make sure you have your child's pediatrician's information with you in case you need to share it with the study team.
- Put all of your child's trial-related information into a folder and bring it with you to each appointment so you can reference it, if necessary.

PLANNING AHEAD TO MEET WITH THE PRINCIPAL INVESTIGATOR

When you meet with the principal investigator for the first time, you may have many questions. Here are a few tips to help you plan ahead so that you are prepared.

- Think about questions you may want to ask and write them down in advance so that you don't forget to ask any of them.
- Bring a family member or friend to support you with asking questions.
- Write down the principal investigator's response to your questions so you can review them with family members who could not attend.

Make sure you leave with an understanding of the potential risks involved with participation and the side effects that could occur. These are important things to consider during the decision-making process.

SUGGESTED QUESTIONS TO ASK THE PRINCIPAL INVESTIGATOR

When meeting with the principal investigator to discuss potential participation, you may want to consider asking some of the following questions:

- What is being studied?
- If researchers are studying an investigational drug, why do they believe it may be effective for SMA?
- How long will participation last?
- How often will I have to visit the hospital or clinic? Will any of these visits require an overnight stay?
- Is there a chance of receiving a placebo or sham?
- What types of medical tests and procedures will be performed?
- What are the possible risks / benefits of participation?
- Who will oversee my or my family member's medical care while participating?
- Will the results of the clinical trial be available to participants?
- Who will pay the costs associated with participation?
- Will I be reimbursed for other expenses?
- Is travel support included?
- Is there a planned extension trial?



EXPANDED ACCESS PROGRAMS

If you or your family member is not eligible to participate in a clinical trial, expanded access – also called “compassionate use” – may be an option to explore. In the United States, the FDA allows some drug companies to provide investigational drugs to patients outside of a clinical trial – however, expanded access programs are highly regulated and patients must meet several criteria in order to be eligible.

The FDA must approve the investigational drug for expanded access based on preliminary safety and efficacy data. In addition, the drug company must be willing to make it available for expanded access.

Not all drug companies may want to make an investigational drug available or have the ability to do so based on:

- lack of safety and efficacy data
- the risks that it could pose to ongoing controlled clinical trials
- cost (the investigational drug may be too expensive and time-consuming to make)
- manufacturing (the ability to produce the investigational drug may be limited, especially for smaller drug companies)

In developing an expanded access program, a drug company may consider:

- ethical concerns about providing the investigational drug while a placebo- (or sham-) controlled trial is still enrolling participants
- whether they need to take resources from the drug development program, which may slow down the approval timeline
- whether early access to the investigational drug could make phase II and III clinical trials more difficult to conduct
- manufacturing capacity (and whether it may limit the availability for clinical trial participants)

Once an investigational drug has been approved for expanded access by the FDA and regulatory authorities, patients still need to meet criteria to be considered to receive it. These may include:

- If the patient is not eligible to participate in a clinical trial because of age, health problems, distance to the trial location, or other factors.
- If the patient has a serious or life-threatening disease, like SMA, for which there is no approved treatment.
- If the doctor believes that the potential benefits of the investigational drug will outweigh any potential risks to the patient.

The purpose of expanded access is to provide treatment for a patient's disease, rather than to collect data about the investigational drug.

POTENTIAL ADVANTAGES AND DISADVANTAGES OF EXPANDED ACCESS FOR SMA

Expanded access for SMA provides a potential option for patients who are not able to participate in a clinical trial. In the absence of a cure or approved treatment, it provides patients with an opportunity to pursue an investigational approach, and it may bridge the gap between the final stages of development and approval by making the investigational drug available during that period.

However, expanded access may also limit enrollment in clinical trials, which are needed for the approval process, carry unknown safety risks, and could reveal unrelated side effects that could result in the delay or termination of a drug development program. In addition, drug companies may need to take resources from drug development, which may delay approval and delay access to those who might benefit from it.

GLOSSARY OF TERMS

Listed below are some of the common words and terms associated with clinical trials along with their definitions.



Blinding

The design of a clinical trial in which participants do not know which treatment they have been assigned to receive (e.g., the investigational drug, or the placebo or sham). Blinding is done to prevent the unintentional bias that can occur when assignments are known.

Data and Safety Monitoring Board (DSMB)

The DSMB is an independent group, or committee, of experts that monitors patient safety and treatment efficacy while a clinical trial is being conducted. The DSMB plays a critical role in the administration of clinical trials.

Double-blind

The design of a clinical trial in which neither the participant, nor the family member / caretaker, nor the principal investigator knows whether the participant has been assigned to receive the investigational drug, placebo, or sham.

Expanded Access

When drug manufacturers provide investigational drugs to patients with serious diseases or conditions who cannot participate in a clinical trial, or when no other treatments are available.

Informed Consent

The process by which a person provides his / her consent, or agreement, to participate in a clinical trial. This occurs after the patient has reviewed the informed consent form and has an opportunity to ask the principal investigator any questions he / she may have about participation.

Institutional Review Board (IRB) / Ethics Committee (EC)

An independent committee that includes medical, scientific, and non-scientific members, whose responsibility is to protect the rights, welfare, safety and well-being of clinical trial participants.

Open-label

A clinical trial in which everyone involved (participant, doctor, and study team) is aware of the drug and dosing levels. In open-label trials, no one receives a placebo or sham.

Placebo

An inactive substance designed to resemble the drug being tested. It is used as a control to eliminate any psychological effects testing may present.

Randomized Controlled Trial

A type of clinical trial where participants are randomly assigned (like flipping a coin) to a particular group. Depending on the trial, participants may be assigned to receive the placebo or sham, the investigational drug, or a particular dose of the investigational drug.

Sham

A sham, or "sham procedure," serves the same purpose as a placebo. It mimics the actual surgical procedure (including pre-procedure routine, anesthesia, and post-procedure follow-up), but does not include the actual delivery of the investigational drug to the patient.

Standard Treatment (Standard-of-Care)

Treatment that is accepted by medical experts as a proper treatment for a certain type of disease and that is widely used by healthcare professionals.

HOW TO LEARN MORE ABOUT CLINICAL TRIALS AND THE DRUG DEVELOPMENT PROCESS

The Food and Drug Administration (FDA) has several resources for patients interested in learning more about clinical trials and the drug development process at [fda.gov/ForPatients](https://www.fda.gov/ForPatients). Highlighted below are some general sections of the website to consider exploring.

Drug Development Processes:

[fda.gov/ForPatients/Approvals/default.htm](https://www.fda.gov/ForPatients/Approvals/default.htm)

Clinical Trials: What Patients Need to Know:

[fda.gov/ForPatients/ClinicalTrials/default.htm](https://www.fda.gov/ForPatients/ClinicalTrials/default.htm)



HOW TO LEARN ABOUT SMA CLINICAL TRIALS

There are many resources available if you are interested in learning about SMA clinical trials. These include the Cure SMA website ([cureSMA.org/news](https://www.cureSMA.org/news)) and the U.S. registry of trials [ClinicalTrials.gov](https://www.clinicaltrials.gov).

Your doctor may also be able to provide information on SMA clinical trials.

About Cure SMA

Cure SMA is dedicated to the treatment and cure of spinal muscular atrophy (SMA) – a disease that takes away a person’s ability to walk, eat, or breathe. It is the number one genetic cause of death for infants.

Since 1984, we’ve directed and invested in comprehensive research that has shaped the scientific community’s understanding of SMA. We are currently on the verge of breakthroughs in treatment that will strengthen our children’s bodies, extend life, and lead to a cure.

We have deep expertise in every aspect of SMA – from the day-to-day realities to the nuances of care options – and until we have a cure, we’ll do everything we can to support children and families affected by the disease.

Learn more about how you can help us reach a treatment and cure at cureSMA.org.



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