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Cure SMA leads the way to a world where everyone impacted by spinal muscular atrophy is empowered to lead independent, successful, and fulfilling lives. We strive to create a community where every individual is heard and feels welcomed. Cure SMA provides practical support programs for our community and advocates for their needs. We fund and direct comprehensive research that drives breakthroughs in treatment, and we advance access to high quality care. We will not stop until we have a cure.

Clinical trials for spinal muscular atrophy (SMA) help researchers answer important questions about the disease and how to treat it. Researchers test new drugs in clinical trials to determine if they are safe and effective in people. The clinical trial process can be long. It contains many steps to protect your safety.

You may be wondering if clinical trial participation is the right choice for you or your family member. This booklet explains what a clinical trial is and what it is like to participate. It also explains some common misunderstandings about clinical trials.
A clinical trial is a type of study in which researchers test whether a new drug is safe and effective for people. Researchers must first study a new drug in the laboratory before they can test it in people. Data from these laboratory studies may suggest that the new drug can safely help people with a certain disease. If so, researchers can apply for permission to study the drug in a clinical trial.

To find out if a drug is safe and effective for the treatment of SMA, researchers may test it in a clinical trial:

- By itself.
- At different doses, for different lengths of time, and by different routes (for example, by mouth or by injecting it into a vein).
- In combination with an SMA treatment that has already been approved. This type of trial is called a "combination trial."
- Against a baseline therapy. A baseline therapy is an approved drug or treatment.
- In some cases, researchers may test the new drug against a placebo or a sham.
  - A placebo is an inactive substance that looks like the new drug but has no effect on health.
  - A sham is a procedure that is the same as the one used to deliver the drug but does not include the drug.
If the clinical trial data suggests the new drug is safe and effective, the researchers can present the results to a regulatory authority. A regulatory authority is a government organization that has the power to approve a drug for widespread use. To approve a new drug, the regulatory authority must agree that the clinical trial data shows the drug is safe and effective.

Each country has its own regulatory authority. In the United States, the regulatory authority is the Food and Drug Administration (FDA).

**WANT TO KNOW MORE ABOUT PARTICIPATING IN A CLINICAL TRIAL?**

It is natural to have a lot of questions about participating in a clinical trial. If you would like to learn more after reading this booklet, please speak with your doctor, visit [www.cureSMA.org/clinical-trials/](http://www.cureSMA.org/clinical-trials/), or contact Cure SMA at [info@curesma.org](mailto:info@curesma.org). If you are interested in participating in a clinical trial, you can find a list of the next steps to take at the end of this booklet.
KNOWING WHAT TO EXPECT

If you are considering participating in a clinical trial, it is important for you to know that receiving a new drug in a clinical trial does not guarantee that your symptoms will improve. In fact, there is no guarantee that the new drug will have any effect. It may even make you feel worse or have unwanted side effects. In some clinical trials, some participants may not receive the new drug. Instead, they will receive either an approved drug or a placebo or sham designed not to have any effect on the disease.

Deciding whether to participate in a clinical trial can be difficult. Participation is always up to you, and you or your family member can stop participating at any time and for any reason (or no reason at all). No longer participating in a clinical trial will not affect your standard healthcare if you withdraw.

MAIN POINTS:

• A clinical trial is a study in which researchers test whether a new drug is safe and effective in people.

• Researchers must first test a new drug in laboratory studies to determine if it may be helpful and can be safely tested in people.

• The FDA reviews the laboratory data to decide whether it will allow researchers to test the drug in people in a clinical trial.

• The FDA also reviews the data from the clinical trial to decide whether to approve the drug for widespread use in people.

• Receiving a new drug in a clinical trial does not guarantee that it will improve your health.

NATURAL HISTORY STUDIES

You may also hear about another type of SMA research study called a “natural history study.” In a natural history study, researchers observe the disease over time without making any changes to the participants’ care. Understanding the natural history of a disease helps researchers come up with new ideas about how to treat it. Findings from natural history studies can also give researchers something to compare clinical trial results against.
WHO CONDUCTS CLINICAL TRIALS?

An organization that conducts a clinical trial is called a “sponsor.” A clinical trial sponsor may be a drug company, a non-profit group, or a government organization.

The sponsor funds the clinical trial and selects the trial site(s). Doctors’ offices, medical centers, and hospitals are examples of clinical trial sites. The clinical trial sponsor also gathers a team of medical and research professionals who will work with the clinical trial participants. You may hear this group of professionals referred to as the “study team.”

THE STUDY TEAM TYPICALLY INCLUDES THE FOLLOWING:

**Principal Investigator**

The principal investigator (PI) is usually a medical doctor who is often called the “study doctor.” The PI is responsible for directing the trial and the other doctors, nurses, and medical staff on the study team.

**Clinical Research Coordinator**

The clinical research coordinator (CRC) or “study coordinator” is often the first member of the study team you will meet when you are deciding whether to participate in a trial. The CRC manages the day-to-day activities of the trial.

**Clinical Evaluator**

A clinical evaluator (CE) is a physical therapist who measures participants’ motor and respiratory function, as well as other changes in their health, throughout the trial.

The type and number of other staff members on the study team depends on the needs of the participants and how the trial is set up. A study team may also include language interpreters who help translate information for participants who do not speak English as their first language.

**MAIN POINTS:**

- Every clinical trial has a sponsor that funds the study, chooses the trial site(s), and selects the study team for each site.
- The principal investigator, clinical research coordinator, and clinical evaluator are among the medical staff that make up a clinical trial study team.

SMA ADVOCACY GROUPS / NON-PROFIT ORGANIZATIONS

Patient advocacy groups like Cure SMA and other non-profit organizations play important roles in the SMA community by:

- Funding research for SMA treatments.
- Providing information on SMA clinical trials.
- Connecting families to the SMA community.
Every country has its own regulatory authority that protects the health, safety, and privacy of clinical trial participants. The regulatory authority in the U.S. is the Food and Drug Administration (FDA).

The FDA approves a detailed plan known as a trial protocol for each clinical trial. The trial protocol explains why and how researchers will conduct the trial. It includes information about:

- Questions researchers are trying to answer.
- How researchers will select participants.
- How much of the drug researchers will give to participants and how often.
- How researchers will monitor the health and safety of participants.
- Known drug side effects or risks.

The FDA also makes sure that the clinical trial follows national rules and laws that protect the rights of participants. It does this in part by requiring that a special committee called an “Institutional Review Board (IRB)” monitors each clinical trial.
**Institutional Review Board (IRB)**

An Institutional Review Board (IRB) is an independent committee that includes medical, scientific, and non-scientific members. A specific IRB monitors each clinical trial site. The IRB is responsible for:

- Making sure that the study team takes steps to protect the health, safety, and privacy of participants.
- Reviewing all written information that the study team gives to participants.
- Monitoring active clinical trials and making sure that researchers report anything that may harm participants.
- Reviewing the clinical trial protocol.

**Data and Safety Monitoring Board (DSMB)**

The Data and Safety Monitoring Board (DSMB) is an independent group that reviews data during the clinical trial. The DSMB is also in charge of creating stopping rules. Stopping rules are reasons that the sponsor should stop the clinical trial early, such as if the new drug is creating serious side effects.

**MAIN POINTS:**

- Three separate groups each perform tasks designed to protect your health, safety, and privacy during a clinical trial.
- These groups are the FDA, the Institutional Review Board (IRB), and the Data and Safety Monitoring Board (DSMB).
HOW ARE CLINICAL TRIAL PARTICIPANTS CHOSEN?

Age, gender, sex, racial and ethnic background, and other factors may affect how a person responds to a drug. For this reason, it is important that people from many different backgrounds participate in clinical trials. This will ensure that approved drugs are safe and effective for everyone who will use them.

All clinical trials have **eligibility criteria**. Eligibility criteria are traits that all participants in a study must share. Clear eligibility criteria make it easier for researchers to evaluate the new drug’s safety and efficacy.

Sometimes you will hear eligibility criteria called “**inclusion**” or “**exclusion**” criteria. Inclusion criteria are traits that a person must have to participate in a trial. Exclusion criteria are factors that prevent a person from being able to participate.

**In SMA clinical trials, common eligibility criteria may include:**

- Age when a person was diagnosed with SMA or age when symptoms began.
- Number of copies of the *SMN2* gene.
- Motor function.
- Respiratory function.
- Current health status and medical conditions that are not related to SMA.
- Surgical history, including prior or planned surgeries.
- Current treatments or therapies for SMA.
- Having already tried the new drug.
- Having already tried other drugs for SMA.
- Recent participation in another clinical trial.
- Distance from the clinical trial site.
If you are interested in participating in a clinical trial, you will attend what is called a “screening visit” so that you can learn more about the trial, and the study team can learn more about you or your child. During the screening visit, the PI and study team will ask you questions and review your or your child’s medical records to determine if you or they meet the clinical study’s eligibility criteria. They may also perform medical tests.

Not everyone who is interested in a clinical trial will have the chance to participate. This could be because you do not meet the eligibility requirements. It could also be that the trial has already enrolled the required number of participants. If you or your family member cannot participate, the PI may be able to discuss the possibility of participating in future trials.

MAIN POINTS:

• It is important that people from many different backgrounds participate in clinical trials so that researchers can make sure approved drugs are safe and effective for everyone who will use them.

• Clinical trials have eligibility criteria that all participants must meet.

• You can learn more about a trial’s eligibility criteria at your screening visit.

• Not everyone who is interested in participating in a clinical trial will be able to participate.
HOW ARE DRUGS SELECTED FOR CLINICAL TRIALS?

Developing a new drug through research is a long and complicated process. Researchers follow several steps to determine whether a new drug may be safe and effective in people. Once they identify a new drug, researchers test the drug in laboratory cells and animals before testing them in people. These tests often take years.

If laboratory test data suggests that a drug will be safe and effective in people, a sponsor may submit a request to the FDA to start a clinical trial. About 1 in every 1,000 new drugs will make it to human testing in a clinical trial.

YOUR HEALTHCARE DECISIONS

Your healthcare decisions should be based on your current medical situation. You should not avoid treatment or care because you believe it may prevent you or your family member from participating in a clinical trial.

There is no guarantee that this will make you or your family member eligible. In fact, some healthcare measures may be required to participate in a clinical trial.
WHAT IS A CLINICAL TRIAL “PHASE”?  

A clinical trial is made up of four phases that build on each other. The study team has a detailed plan and specific research goals for each clinical trial phase. At the end of each phase, the clinical trial sponsor and the FDA review the results and decide if the new drug will advance to the next phase. At the end of the third phase, the sponsor can ask the FDA to approve the new drug for widespread use in people.

The FDA chooses NOT to approve more than 90% of new drugs that enter clinical trials because of:

- Safety concerns.
- Lack of effectiveness.
- Technical issues related to not being able to make enough of the new drug or not being able to make it reliably.

THE FOUR PHASES OF CLINICAL TRIALS

Phase 1: Researchers test the new drug in a small group of people to make sure the drug is safe and the dose is correct. Participants in this phase are usually healthy volunteers. Researchers do not try to answer questions about whether the new drug is effective for SMA in this phase.

Phase 2: Researchers give the new drug to a larger number of participants with SMA. The study team looks for signs that it may be effective and further evaluate its safety.

Phase 3: Researchers give the new drug to an even larger number of people with SMA to confirm its effectiveness and safety. They often compare the new drug to a placebo or sham in this phase.

At the end of Phase 3, a sponsor may ask the FDA to approve the drug for use in people affected by SMA by submitting a new drug application (NDA). An NDA is the document a study team puts together to formally ask the FDA to approve a new drug. The application contains all the data gathered during the clinical trial.

Phase 4: Even after a new drug has been approved, researchers keep gathering information on its effects in different groups of people. They look for benefits and unwanted side effects that may show up when people use the drug for a long time. A sponsor may invite people who participated in a Phase 3 trial to continue to take the drug and be monitored after Phase 3 has ended. This is sometimes known as an “extension trial” or “follow-up study.”
WHAT IS A PLACEBO OR SHAM AND WHY IS IT USED?

Researchers may use a placebo (or a sham) in some clinical trials. A placebo looks like the new drug but has no active ingredients. A sham copies the way the new drug is given to the participants but does not include the actual drug. Using a placebo or sham helps researchers learn if changes in participants’ disease or condition are caused by the new drug itself or by other factors.

SMA is a serious, progressive disease. The FDA has already approved three treatments for SMA. For these reasons, it may be unfair to give some participants a placebo or sham in a clinical trial for a new SMA drug. However, researchers may use a placebo or sham in a combination trial for a new SMA drug. A combination trial is designed to test whether using a new drug along with an approved drug improves SMA symptoms. In a combination trial, all participants would receive the approved drug for SMA and would also receive either the new SMA drug or a placebo or sham. For more information on participating in combination trials, scan the QR code:
WHY ARE SOME DRUGS APPROVED FASTER THAN OTHERS?

Sometimes a new drug will move through the clinical trial process more quickly than usual. The following are some ways the FDA can speed up the review and approval of a new drug:

**Option 1 – Accelerated Approval**
The FDA can allow faster or “accelerated” approval of drugs that treat serious conditions or fill an unmet medical need. An unmet medical need is one for which there is currently no effective treatment. Accelerated approval is based on an endpoint. An endpoint is a measurement that predicts how well the drug may work.

**Option 2 – Fast Track**
The Fast Track process shortens the time it takes to develop and review a new drug that treats a serious condition and will fill an unmet medical need. The Fast Track process increases communication between the FDA and the drug company to clear up questions quickly.

**Option 3 – Breakthrough Therapy**
If early clinical trial data suggests that a new drug is more effective than other available treatments, the FDA can speed up its development and review by treating the drug as a “breakthrough therapy.”

**Option 4 – Priority Review**
Priority review means the FDA will try to review the new drug application within 6 months instead of the standard time of 10 months. Priority review may be an option for drugs that treat a serious condition and appear to be safer and more effective than current treatments.
ORPHAN DRUG DESIGNATION
Rare diseases like SMA are those that affect fewer than 200,000 people in the U.S. A sponsor who is developing a drug to treat a rare disease can apply to the FDA for orphan designation of the drug. Orphan designation can reduce the cost of developing the new drug. Researchers must still test the safety and effectiveness of an orphan drug in a clinical trial.

REAL-WORLD DATA
After the FDA has approved a new drug, doctors and patient registries continue to collect data on the drug’s benefits and side effects. You may hear this data referred to as real-world data. Real-world data are important because they help researchers understand the long-term benefits and risks of a drug. As more individuals with SMA receive treatment, researchers have more opportunities to collect real-world data. This data may help researchers learn how to improve SMA drugs. For these reasons, enrolling in an SMA patient registry and participating in long-term follow-up care can benefit you and the SMA community.

MAIN POINTS:
• The FDA may speed up the review and approval of drugs for serious diseases like SMA.
• The FDA may give a new drug for a rare disease a special designation to make it less expensive to develop.
• You can help researchers develop SMA treatments by joining a patient registry and receiving regular follow-up care.
Individuals and families affected by SMA participate in clinical trials for many reasons, including:

- **To help researchers gain knowledge about SMA.**

- **To contribute to the development of a new treatment.**

- **To help others affected by SMA.**

It is important to remember that there is no guarantee clinical trial participation will benefit your health. There may also be risks – some known, some unknown. The study team must describe known risks to potential participants (or family members, depending on the age of the participant). They may include unpleasant or even serious side effects. If the study team identifies new risks during the trial, they will also share this information.

You should decide whether to participate in a clinical trial only after you have thought about the risks that may be involved and understand what your responsibilities will be. You will have the chance to discuss the potential risks and benefits with the PI and study team before and during your screening visit.
INFORMED CONSENT

At your screening visit, the PI or another member of the study team will give you an informed consent form. The informed consent form explains the details of the trial, including its purpose, length of time, required procedures, key contacts, and any possible benefits and risks. The study team will review the informed consent form with you and answer any questions. You may take the form with you after the screening visit to review it and discuss it with family members or friends.

If you decide to participate, you must provide your consent by signing the informed consent form. This process is called the “informed consent process.” The informed consent process is in place to make sure you have all the information you need to make the best possible decision about whether or not to participate in a clinical trial.

During the clinical trial, the PI may make changes to the trial protocol. If any changes are made, you will be asked to review and sign an updated informed consent form. This is called “re-consenting.”

If you become sick or develop other medical issues during the trial, the study team may end your participation without your consent.

MAIN POINTS:

- People participate in clinical trials for many reasons, such as to receive care from the study team or to help advance research.
- Participation in a clinical trial is always voluntary. You can change your mind at any time and for any reason.
- You will learn about the potential risks of participating in a clinical trial during the informed consent process.
- The PI must inform clinical trial participants of any changes that may affect their decision to continue to participate.

Follow this link to view a helpful video on the informed consent process:

SPECIAL CONSIDERATIONS FOR CHILDREN

If your child is 7 years of age or older, the PI may ask him/her/them if they agree (or “assent”) to participate in the clinical trial. Not all trials require assent from children. For those that do, the age at which it is requested can vary. However, it is important to discuss participation with your child if they are old enough to understand.
Clinical trial participants (or family members, depending on the age of the participant) have certain responsibilities. These may include:

- Following all instructions given by the study team.
- Attending all scheduled visits.
- Completing questionnaires about your or the participant’s health between visits.
- Telling the PI about any new health problems. Even if you don’t think a problem is caused by the new drug, it is very important to report any change.
- Telling the PI about any new medications.
- Telling the PI about changes in how much or how often you take a medication.
- Being careful about discussing the clinical trial with other participants, including whether you think you or your family member may be receiving a placebo or sham.

SOCIAL MEDIA

You should limit what you say about the clinical trial to family members, close friends, and doctors. It may also be tempting to share information about your experience on social media networks like Facebook, Twitter, and YouTube, but doing so can affect the results of the trial.

Posting your information online could influence other participants and how they report the side effects of the new drug. Sharing information online could also accidentally reveal whether participants are receiving the new drug or a placebo/sham. This could make it impossible for researchers to make accurate conclusions from the trial data.

If you have questions about what may be appropriate to share, please speak with a member of the study team.
FOR PARENTS OF CHILDREN WITH SMA

Organizing and managing clinical trial appointments for a child living with SMA can be a challenge. These tips may be helpful:

• With the help of the study team, develop a calendar showing study appointments and telephone calls.
• Consider bringing toys and other materials to keep your child busy while waiting to meet with the study team.
• If you are traveling by plane and your flight has been cancelled or delayed, let the study team know so that they can adjust the time of your appointment.
• Make sure you have your pediatrician’s information with you.
• Put all your child’s trial-related information into a folder and bring it with you to each appointment.

TRAVEL SUPPORT

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

The PI may recommend that individuals who are extremely weak do not travel. It is best to speak with the study team about your particular situation.

MAIN POINTS:

• Clinical trial participants have many responsibilities. It is important to understand these responsibilities before agreeing to participate in a clinical trial.
• Participating in a clinical trial with a child living with SMA can take extra planning.
• Financial support for travel and other expenses may be available to trial participants.
I AM INTERESTED IN PARTICIPATING IN A CLINICAL TRIAL! WHAT’S NEXT?

FIND A CLINICAL TRIAL NEAR YOU

If you think you may be interested in participating in an SMA clinical trial, the first step is to find out if there is a clinical trial near you. Several clinical trials are currently taking place across the U.S. for drugs designed to manage or slow the progression of SMA.

Here are four ways to find a clinical trial near you:

1. Ask your doctor. Neuromuscular disease specialists may be the most likely to have information on SMA clinical trials.

2. Find out about SMA clinical trials by visiting Cure SMA’s clinical trial page and going through the following steps.
   • Paste this address into your web browser: cureSMA.org/clinical-trials.
   • Under “SMA Clinical Trials,” click on any trial within the list. This will take you to that trial’s page within ClinicalTrials.gov. ClinicalTrials.gov is a government database containing information about clinical trials in the U.S. and other countries.
   • On the trial’s page, click on Contacts and Locations in the green box. This will take you to a page that lists the trial site locations and contact information for each site.
   • If the trial is being conducted at a site near you, use the phone number or email address provided to contact the study team and tell them you are interested in participating.

3. Link directly to a list of all the SMA clinical trials that are registered with ClinicalTrials.gov.

4. Create your own search for SMA clinical trials by going to the ClinicalTrials.gov website at https://clinicaltrials.gov/ and entering search terms under “Find a Study.”

Make sure you look for studies that are currently enrolling participants. They are labeled “Recruiting” under “Status.” You may also be interested in clinical trials that plan to recruit participants in the future. They are labeled “Not Yet Recruiting.”
PLAN FOR YOUR SCREENING VISIT

When you speak with the study team, they will help you decide if the trial is a good match for you. If it is, the study team will schedule a screening visit. You may have many questions when you meet with the PI at the screening visit.

Here are a few tips to help you prepare for your screening visit:

• Think about questions you may want to ask the PI. Write them down in advance so that you remember to ask them all.
• Bring a family member or friend to support you.
• Write down the PI’s answers 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 and the side effects that could occur. These are important things to consider when deciding whether to participate.

QUESTIONS TO ASK AT YOUR SCREENING VISIT

You may want to ask the following questions at your screening visit:

• What are the researchers studying?
• If researchers are studying a new drug, why do they believe it may be effective for SMA?
• How long will participation last?
• How often will you have to visit the hospital or clinic? Will any of those visits require an overnight stay?
• Is there a chance of receiving a placebo or sham?
• What types of medical tests and procedures will the study team perform?
• What are the possible risks/benefits of participation?
• Who will oversee you or your family member’s medical care while participating?
• Will the results of the clinical trial be available to you?
• Who will pay the costs associated with participation?
• Will you be reimbursed for other expenses?
• Is travel support included?
• Is there a planned extension trial?
• How many people will take part in this study?
• What other choices do you have if you do not take part in the study?
• What happens if you are injured because you take part in this study?
• Who can answer your other questions about the study?
• Will your study-related information be confidential?

More information about SMA is available at www.curesma.org and on the Cure SMA YouTube channel:
Cure SMA is a non-profit organization and the largest worldwide network of families, clinicians, and research scientists working together to advance SMA research, support affected individuals/caregivers, and educate the public and professional communities about SMA.

Cure SMA is a resource for unbiased support. We are here to help all individuals living with SMA and their loved ones, and do not advocate any specific choices or decisions. Individuals and caregivers make different choices regarding what is best for their situation and consistent with their personal beliefs. Parents and other important family members should be able to discuss their feelings about these topics, and to ask questions of their SMA care team. Such decisions should not be made lightly, and all options should be considered and weighed carefully. All choices related to SMA are highly personal and should reflect personal values, as well as what is best for each individual and their caregivers.

Remember that your healthcare team and Cure SMA are here to support you. To continue learning, please see other available Care Series booklets:

- Breathing Basics
- Caring Choices
- Genetics of SMA
- Musculoskeletal System
- Nutrition Basics
- Understanding SMA