

Clinical Trial Crocodile™



A Children's Guide to Clinical Trials


This book belongs to:

Hi!

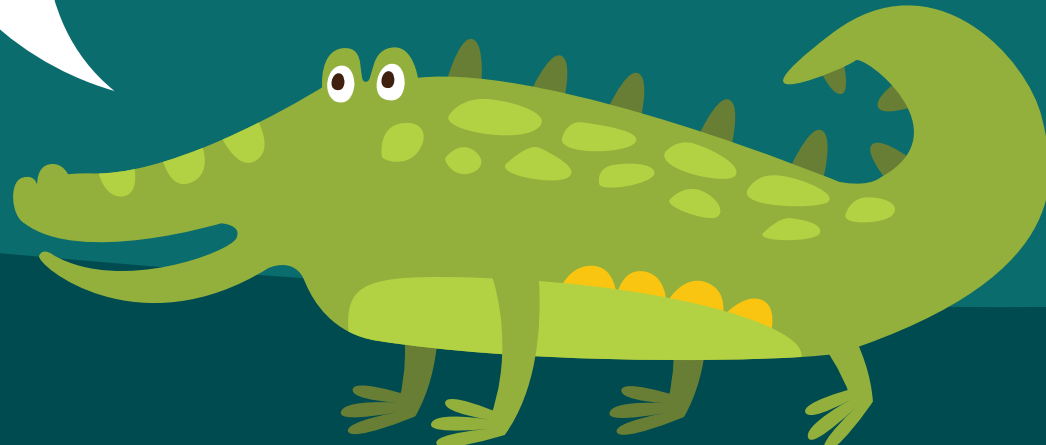
I heard that you want to learn about clinical trials. I can help with that. In this short book, I will answer questions you might have. You can ask a parent or someone else who takes care of you for help if you don't understand something.

Let's get started.





Hi, Mr. Crocodile!
What is a clinical trial?



Think about what happens when you get sick. You might see a doctor who gives you some medicine. You might wonder how the doctor knows the medicine may help. That's where clinical trials come in.

Before you can take a medicine, it goes through lots of tests. These tests are called clinical research, and they help us find out what a medicine will do when you take it.

Clinical trials are being done all the time to find new ways to help people. This is because there are a lot of diseases that there is no medicine for right now.



Hey, Mr. Crocodile,
why are medicines
tested?

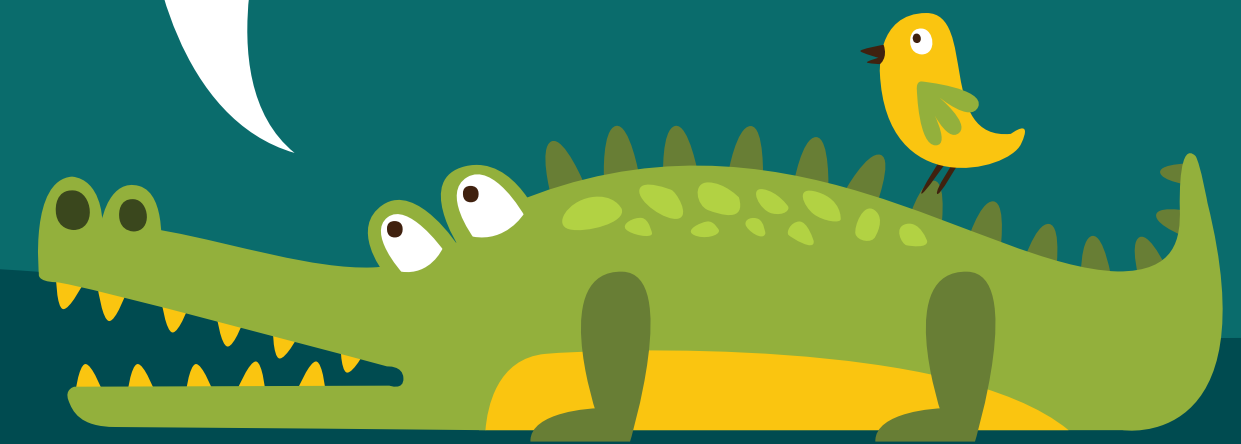
We need to learn as much as we can about medicines. That's why we test them in people in clinical trials. This helps answer important questions, such as:

- How safe is a medicine?
- What happens when people take it?
- Does it help them feel better?
- Does it make them feel worse?
- How much of it should people take?
- How often should they take it?

After these questions have been answered, experts decide if a medicine can be used.




Mr. Crocodile,
who takes part in
clinical trials?



We need all types of people to be in clinical trials, and the types of people are sometimes determined by the disease and medicine being tested. This is so we can learn how a medicine may work in anyone who might take it in the future.

- For some trials, we need children.
- For other trials, we need adults.
- We might need both children and adults in some trials.
- We need boys and girls, men and women.
- We need people from all parts of the world.
- We need people of all sizes, shapes, and colors.



Hi, Mr. Crocodile!
Why do children
take part in
clinical trials?


Some diseases may affect children more than adults. Medicines to treat these diseases have to be tested in children. This is because your body may not react to the medicines in the same way as an older person's body reacts to the medicines. Clinical trials help test medicines in children to see if they are safe and effective for younger people.



Hey, Mr. Crocodile,
can anyone join a
clinical trial?

Not everyone can be in a trial. We need to make sure a trial is right for someone first. That's why each trial has rules for who can be in it. The rules may be about:

- How old someone is
- The type of disease they have
- Other health conditions they may have
- Medicines they are taking
- Types of care they have had
- The results of health tests
- If they agree to go to a clinic where they meet the trial team and have some medical tests done



Mr. Crocodile,
how many people are
in a clinical trial?

Trials are done in steps, called “phases.” How many people are in a trial can depend on the phase.

Phase 1

In most phase 1 trials, a medicine is tested in a small group of people. This is to learn about how safe it might be. If the results are good, the medicine can be given to more people in the next phase.

Phase 2 and Phase 3

These trials have more people. The medicine may be tested in hundreds of people or more.


Combined Phases

It can be hard to get a large group of people for some trials. If this is the case, phases can be combined. For example, there might be a phase 1-2 trial or a phase 2-3 trial. This can make it easier to get more people in a trial.

Review of the Trial Results

After a medicine is tested in these phases, groups of experts look at the results of the trials. They decide if the medicine can be used for treating people who are not part of a trial. This is based on how safe the medicine is and if it can help people with the disease being studied.






Hi, Mr. Crocodile!
Do all the people in
a clinical trial get a
“trial medicine”?

It depends on the trial. In some trials, all the people get a trial medicine. In other trials, people are randomly put in groups.

- Some groups get the trial medicine.
- Some groups may get a different medicine.
- Some groups may get a “pretend” medicine. A pretend medicine is called a placebo. It does not have real medicine in it.
- People who get a placebo may get a trial medicine later.

When a trial has more than one group, we look at what happens in each group. We compare the results. This helps us learn more about the trial medicine.



Hey, Mr. Crocodile,
why do people join
clinical trials?

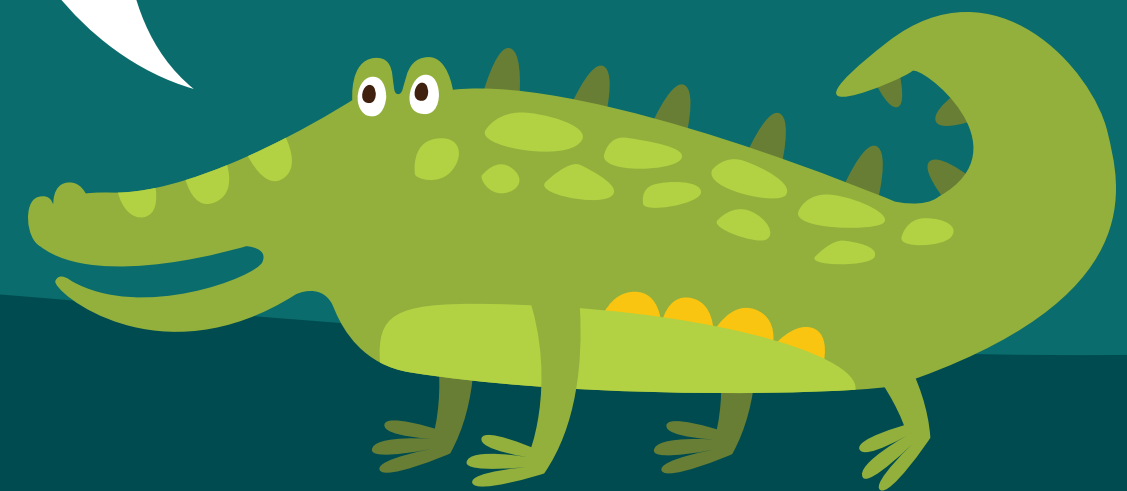



Do you have
to join a
clinical trial?

There are lots of answers to this. People have their own reasons for joining a clinical trial. For example, they may join a clinical trial to:

- Have a chance to get a trial medicine
- Get care from a trial team that includes experts for their disease
- Help find new ways to care for people with the same disease in the future

You get to choose if you want to be in a trial. You can say “yes” or “no.” No one will be upset if you say “no.” You can say “yes” and change your mind later. If you don’t join a trial, you can still get care for your disease. The trial team will talk with you about other kinds of care you can get.






Who will care for me in a clinical trial, Mr. Crocodile?

The trial team will include a doctor and at least one nurse. They will care for you in the trial. This care will include:

- Checking your health
- Watching you for any side effects
- Doing health tests that help them learn more about your disease and the trial medicine

The trial team will know how to care for people who have the disease being studied. The team may include more people who can help with tests to check your health. You will still go to your usual doctor for care that is not part of the trial.



Mr. Crocodile,
what happens in
a clinical trial?



1 First, the trial team will give you or your parent (or someone else who takes care of you) a form to read. This form explains the trial.

- The team will go through the form with you and the adult who takes care of you. They will explain each part of the trial.
- You can ask all the questions you want to ask.
- You can talk with your family about the trial.
- You can decide if you want to join the trial.
- Your parent or someone else who takes care of you will also need to decide if they want you to join the trial.


2 If you choose to join the trial and someone who takes care of you signs the form, the team will find out if the trial is right for you.

- They will ask questions about your health and the care you have had in the past.
- You will have tests done to help them learn more about your health and your disease.
- The team will look at all the results and let you know if you can be in the trial.

3 If you can be in the trial, you will follow a schedule.

The schedule will tell you when you need to see the trial team.

- When you see the team, they will do tests to check your health. The team will tell you what each test is and what you will need to do.
- A parent or someone else who takes care of you will be with you each time you see the team.



What kinds of health tests will be done, Mr. Crocodile?

Some tests will be like tests you have when you go to your usual doctor. In a trial, these tests may be done more often. You may also have tests done that are new to you. Your trial team (the trial doctor or nurse) will tell you how each test is done. You can ask them questions about all the tests. A few tests you may have in a trial are:

- **A check of your body.** This is done to make sure you are well. It can include measuring your weight and height, checking your vital signs (body temperature, heart rate, breathing rate, and blood pressure), and looking at your general appearance. This is usually called a physical exam.
- **Blood tests.** Blood is taken from your arm.
- **Urine test.** You will be asked to pee in a cup.
- **Pictures of your inside.** These help a trial team look at parts of your body they can't see on their own. This may include looking at your heart, bones, and muscles.
- **Heart tests.** Tests are done to see how your heart beats and moves in your chest.
- **Walking and strength tests.** Tests may be performed to see how far or fast you can walk. Certain tests may also measure how strong you are.
- **Muscle biopsy.** This is a surgical operation. A small amount of muscle is removed and sent to a laboratory. This small amount of muscle is tested to see how your muscles are changing while you are in a trial.




Mr. Crocodile, how long could I be in a clinical trial?

Some trials may last a few months. Others may go on for years.

How long you are in a trial can also be based on the effects a trial medicine has on you.


You can choose to stop being in a trial at any time. If you want to stop, all you need to do is tell the trial team. You can also ask a parent or someone else who takes care of you to tell the team.



Hey, Mr. Crocodile,
Will being in a clinical
trial help me?

There is no way to know if a trial will help you. If you join a trial, you will:

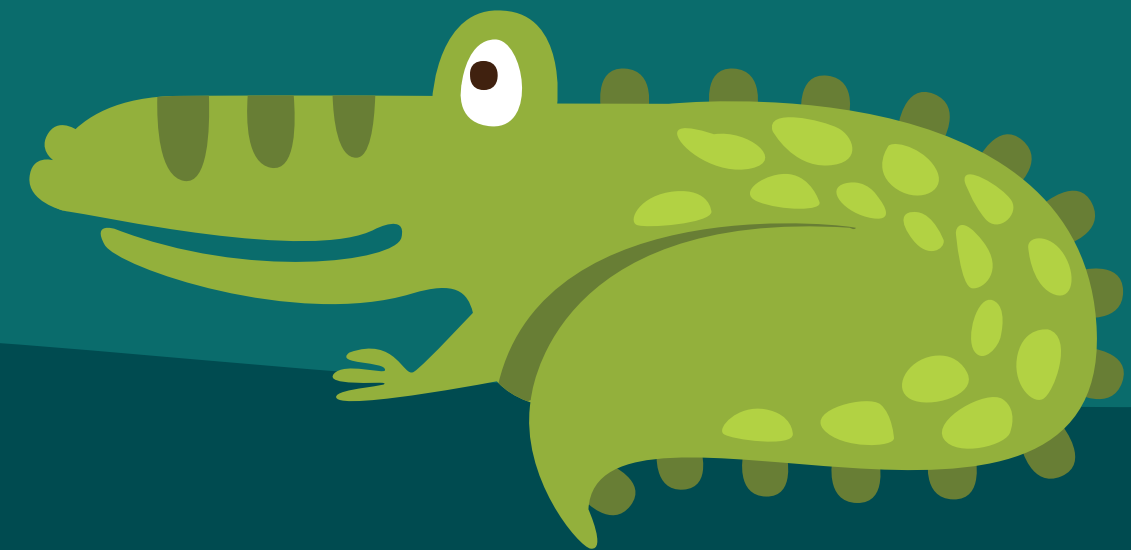
- Get care from a team that knows a lot about your disease
- Be watched for signs of any changes in your health
- Have the chance to ask any questions you want about your disease




Will being in a clinical trial make me feel worse, Mr. Crocodile?

Any medicine can have “side effects” that might make you feel bad. Some of these are worse than others. Some people might have side effects. Others might not. Some side effects of a trial medicine might not be known. That is part of what we will try to learn. In a trial, the team will:

- Explain the side effects that you might have
- Watch you for signs of any side effects
- Try to make you feel better if you have side effects



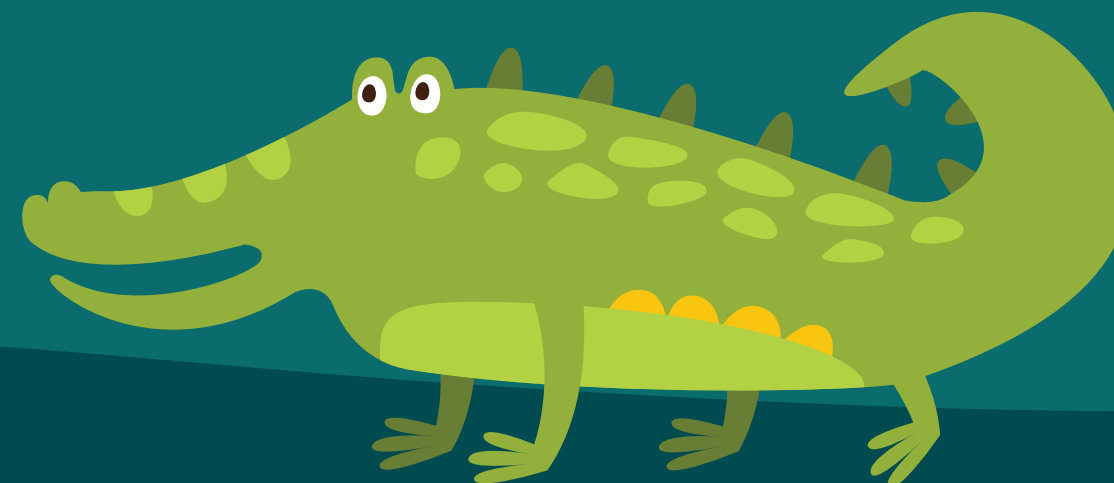


Hey, Mr. Crocodile, how
can I learn more about
Sarepta's clinical trials?

We have a program called SareptAlly, in partnership with a group called myTomorrows, that is designed to help patients, families, and physicians around the world gain access to resources and information about Sarepta's clinical trials.

To get started with SareptAlly, your parent or someone else who takes care of you can:

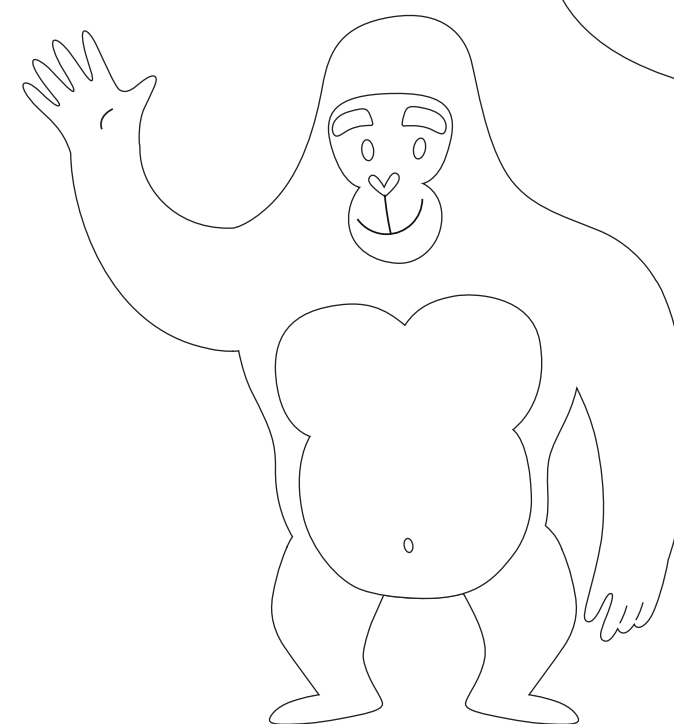
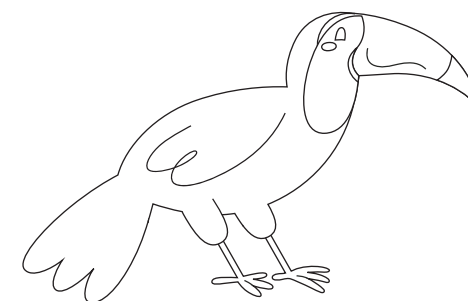
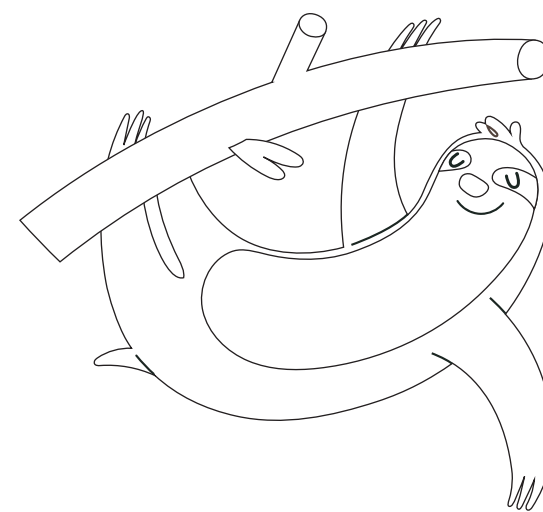
- Schedule a telephone call with a dedicated Patient Navigator by going online to:
<https://mytomorrows.com/sareptally/en/start>
- Send an email to SareptAlly@sarepta.com



I hope you enjoyed learning about clinical trials!



Color me and my friends!





SAREPTALLY

