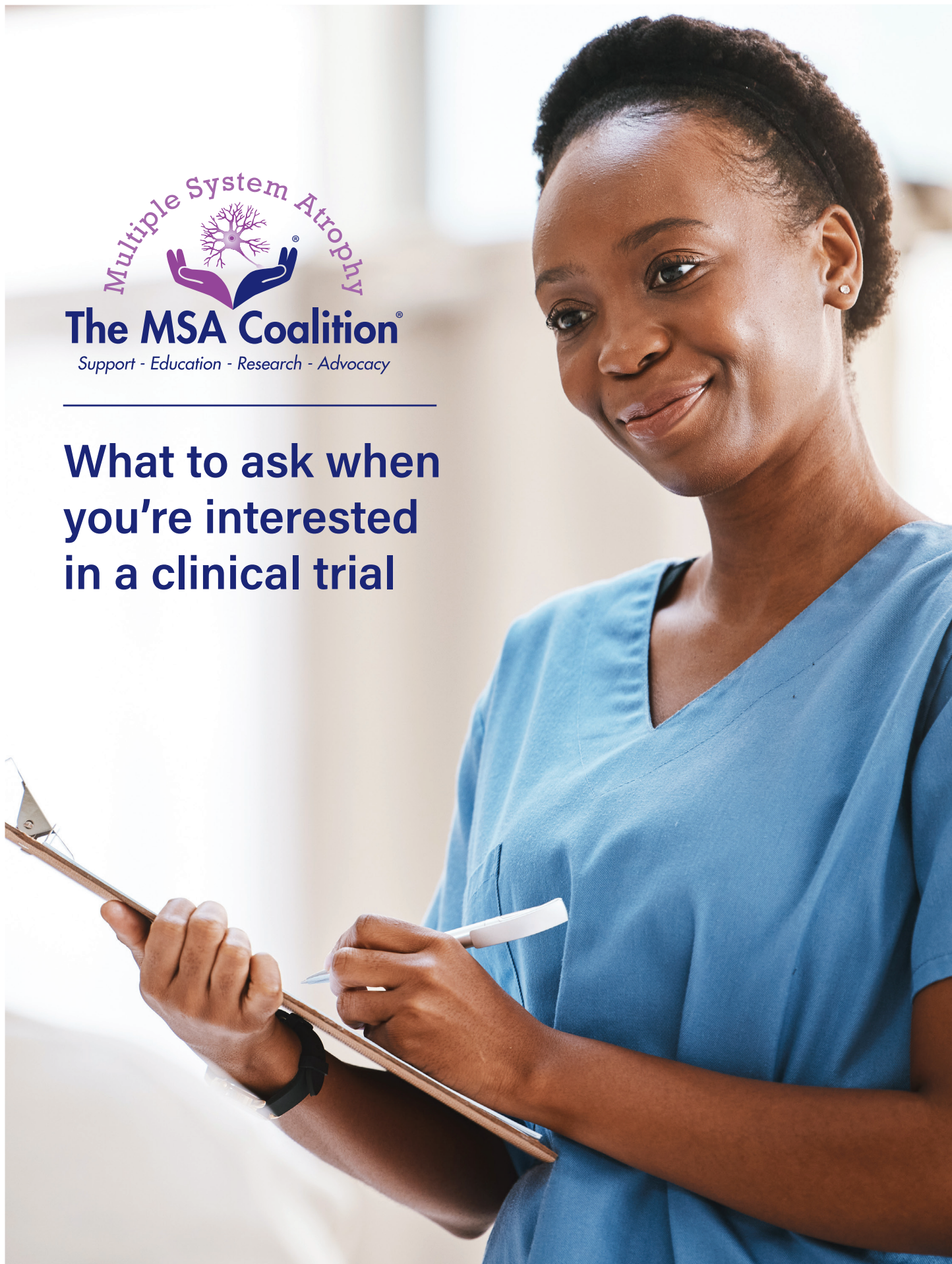




The MSA Coalition®

Support - Education - Research - Advocacy

What to ask when you're interested in a clinical trial



Acknowledgments

The MSA Coalition, in collaboration with ICON, would like to express their deepest gratitude for and recognize the members of the 2020-2021 Rare Disease Advisory Council. The MSA Coalition is especially grateful for their understanding of the lived reality of rare disease patients and caregivers experience each day, their expertise in clinical development, and their willingness to join us on this journey through their immense contributions to this toolkit.



Introduction

You've probably heard that it's important to consider all of your options if you are thinking of joining a clinical trial.

It's also important to consider how easy or difficult each trial would be for you and for the people close to you who matter—and to ask what can be done to make the harder things easier. It can help to know what kind of support is possible so you can know what to ask for.

ICON has helped pharmaceutical companies run more than 350 rare disease clinical trials. If you or a loved one are affected by a rare disease and are thinking about taking part in a clinical trial, we know it can feel like a whole different world—especially if it's something that is new to you.



We hope this resource will give you both information and confidence as you explore clinical trials that may be right for you!

When to ask...

There's no wrong time to ask for what you need.

The best time to ask these questions is before you start going through the process to find out if you can be accepted into a specific clinical trial. This process is called the "screening process" or the "eligibility process." Usually, there's a lot to be done during this time, such as providing medical information, going to a medical center for a variety of tests, filling out forms, and answering questions.

You will only find out if you qualify at the end of the screening process, and it's important to understand that there are no guarantees yet. So before you start the process, it may be helpful to use this list of questions, asking yourself and the person you speak to about the trial so you know what to expect if you are eligible to participate.

This can help you figure out how being in the trial would fit into your daily life before you decide if it's right for you.

This list will help you to ask questions about:

- What you'll have to do to find out if you can get into the trial
- How you'll get to the trial's medical center for visits
- What assistance you'll have during each trial visit
- The medical tests that will be performed during the trial
- What you'll be required to do at home during the trial
- What happens when the trial ends

How to use this list

- Read each question and think about which ones are most important to you personally.
- Keep the list with you when you're speaking to a doctor, coordinator, genetic counselor, or researcher about a trial you may be interested in.
- Ask any and all of the questions that are important to you. You can be confident that these are great questions to ask and that they should be able to give you the answers or find the answers for you before you make your decision to join a trial or not.
- If it won't be easy for you personally, ask about what could be done to make it easier! Read the "What may be possible..." column for some examples!
- Have confidence that your needs are important and that research staff want to help make it possible for you to participate in their trial. No clinical trial can happen without the patients and caregivers who decide to take part.
- Speak up about what you need to make your participation easier because research staff may not realize that it's important to you! Each rare disease is unique, and each person is unique. Let the people you speak with know what will work for you.

What you'll have to do to find out if you can get into the trial

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

<p>1 Will I/they have to stop taking any medicines/treatments/supplements for a few weeks before screening can begin? (Known as a 'washout period')</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>If a trial doesn't allow people to take specific medications while they participate, there's usually no flexibility about that. It could be for their safety or because it might interfere with the results of the trial. But you can ask if there are any other medications or therapies that may make it easier to stop taking the ones not allowed on the trial. You can also ask how you will be monitored to make sure you're doing well if you've stopped a medication you usually take. You may be able to ask to be seen by the research team more often (do some additional visits).</p>
<p>2 Will I/they only have one chance to get into the trial? (Trying more than once is called 're-screening' and not every trial allows this)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Some trials let people try to get in more than once, others don't. It's not always obvious, so it's good to ask if it wasn't clear how many chances you'll have to get in. If you're not sure if you'd meet the requirements right away, it's especially important to ask this question. If you don't meet the screening criteria and re-screening isn't allowed, you can ask if there are other treatment options outside of the trial with the doctor/hospital. If there are no additional options, you should request a follow-up plan.</p>
<p>3 Will I/we have to do a lot of medical tests during the screening process to find out if I/they can be in the trial?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>You would have to complete all the tests required to see if you qualify for a trial. But if this is hard for you, be sure to tell the research staff and let them know why. For example—have they scheduled your appointments at a bad time of day for you? Is it hard to understand how you're supposed to complete some of these tests? If you speak up, the research staff may be able to better accommodate your needs.</p>
<p>4 Will I/we be able to bring in results from tests I/we have already done to avoid doing them again during screening?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>In some trials, this is possible for some of the screening tests. If you're not sure, ask! Especially if there are tests you sometimes do that you'd like to avoid doing again if possible.</p>
<p>5 Will I/we have to go to a specific medical center for all the screening process tests?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>In some trials, people are allowed to do some of their screening tests closer to or at home. If it is easier for you to do any of the tests required at a medical center closer to you or at home, ask if this would be possible.</p>

What you'll have to do to find out if you can get into the trial

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

<p>6 Will I/we have to make arrangements myself to do any of tests required? (for example, blood work or genetic testing)</p>	<p><input type="checkbox"/> Yes</p> <p><input type="checkbox"/> No</p> <p><input type="checkbox"/> Not Sure</p>	<p><input type="checkbox"/> Easy</p> <p><input type="checkbox"/> Hard</p> <p><input type="checkbox"/> Impossible</p> <p><input type="checkbox"/> Not Sure</p>	<p>In some trials, this is required/allowed and in others it's not. Ask how much time you would have to complete all the tests. If you don't think that's enough time, let them know and ask if you could have more time. Also ask if the cost of these tests is covered by the trial.</p>
<p>7 Will I/they be giving up the opportunity to participate in other trials or get any approved treatments by joining this trial?</p>	<p><input type="checkbox"/> Yes</p> <p><input type="checkbox"/> No</p> <p><input type="checkbox"/> Not Sure</p>	<p><input type="checkbox"/> Easy</p> <p><input type="checkbox"/> Hard</p> <p><input type="checkbox"/> Impossible</p> <p><input type="checkbox"/> Not Sure</p>	<p>In most drug trials, participants can't participate in two trials at the same time. Sometimes, participating in a trial can also limit what treatments you can have while you're in the trial. But in many cases, this doesn't mean that you couldn't participate in future trials or take other treatments once you finish.</p> <p>Ask the research staff what other trials or approved treatments may be available and whether taking part in the trial may prevent you from taking any current or future treatment options.</p>
<p>8 Is there a chance that I/they would get a placebo instead of the trial drug?</p>	<p><input type="checkbox"/> Yes</p> <p><input type="checkbox"/> No</p> <p><input type="checkbox"/> Not Sure</p>	<p><input type="checkbox"/> Easy</p> <p><input type="checkbox"/> Hard</p> <p><input type="checkbox"/> Impossible</p> <p><input type="checkbox"/> Not Sure</p>	<p>Some trials give some participants the trial drug and other participants a harmless 'fake' that has no therapeutic effect called a 'placebo'. This is done so that results can be compared to see if the drug really did have an effect and it wasn't just that people (patients and doctors) believed it did.</p> <p>Ask what are the chances of getting a placebo if you joined the trial? Would it be 1 in 2 (50%) or 1 in 4 (25%)? Also ask if at some point in the trial you would be guaranteed to get the trial drug—in many rare disease trials, some people may start on a placebo at first, but can get the drug in a later stage of the trial or after the trial.</p>

How you'll get to the trial's medical center for "visits"

In nearly all rare disease clinical trials, participants have to go to a medical center for at least some or all of the tests required. How often (for example, daily, weekly, or monthly) and how many total "visits" you would have to do depends on the clinical trial.

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself...
how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

9 Will I/we have to travel more than a couple of hours to get to the trial's medical center?	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>There are many options for helping to make this easier, from having travel arrangements made for you to getting pre-paid cards for expenses you might have on the way or having an aid assistant accompany you on the way there and back. Ask research staff what transportation support options are available to you if you participate. Be sure to tell them if you and anyone helping you get to the site (caregiver/family/friend) will need any Personal Protective Equipment (clothing, helmets, goggles, masks, etc) or need a special car seat or vehicle that can accommodate a wheelchair.</p>
10 Will I/we have to pay for gas/parking/train travel/air travel to get there?	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>In some trials, it's possible to either:</p> <ul style="list-style-type: none"> – Get a refund after you submit your receipts for things you had to pay for to get to the medical center – Get a pre-paid card in advance to pay for expenses like these – Have travel bookings made for you and paid by the trial ahead of time <p>Ask which of these would be available to you in the trial. If you'll have to submit receipts to get refunded for costs, ask how quickly you can expect to be paid back. Tell them what your preference would be.</p>
11 Will I/we have all the equipment needed to travel that distance? (eg, wheelchair, feeding equipment, respiratory equipment, etc)	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Special equipment that people may need for trips to the medical center can be provided but might only be supplied to you if you ask for it. Be sure to tell the research staff about any equipment you would need and ask if it can be provided to you.</p>
12 Will I/we have to be away from home overnight for any of the trial visits?	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Overnight arrangements may be available to make sleeping away from home easier. For example, having an aid assistant to help you or help you care for the person you are accompanying. A family-friendly hotel room, Airbnb, or apartment may be available to you. Some trials cover the cost of babysitting services or pet sitting services and can help make arrangements for this. Speak up about what your needs and preferences are and ask if they can be provided.</p>

How you'll get to the trial's medical center for "visits"

In nearly all rare disease clinical trials, participants have to go to a medical center for at least some or all of the tests required. How often (for example, daily, weekly, or monthly) and how many total "visits" you would have to do depends on the clinical trial.

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)	What was their answer?	Ask yourself... how hard would that be for you?	What may be possible...If it's not easy for you, speak up about what you need to make it easier...
13 Will I/we have to do telephone calls, video calls, or use apps, and if so, will this take up a lot of phone minutes/data?	<div><input type="checkbox"/> Yes</div> <div><input type="checkbox"/> No</div> <div><input type="checkbox"/> Not Sure</div>	<div><input type="checkbox"/> Easy</div> <div><input type="checkbox"/> Hard</div> <div><input type="checkbox"/> Impossible</div> <div><input type="checkbox"/> Not Sure</div>	If so, it may be possible to get the cost of cell phone minutes/ data reimbursed—or a separate smartphone with internet access provided to you to avoid out-of-pocket expenses.
14 Would I/we likely have to take some days off from work to be able to do these visits?	<div><input type="checkbox"/> Yes</div> <div><input type="checkbox"/> No</div> <div><input type="checkbox"/> Not Sure</div>	<div><input type="checkbox"/> Easy</div> <div><input type="checkbox"/> Hard</div> <div><input type="checkbox"/> Impossible</div> <div><input type="checkbox"/> Not Sure</div>	<p>Often, it may take a few hours or a full day to do the tests at each visit. Many people find that means they would have to take time away from work. Ask if you could be paid a 'stipend' (a payment for your time that is sometimes given to participants, usually a set amount for each visit they complete).</p> <p>Ask if it would be possible to do any of the tests during the times/days you are not working. It may be possible for the re-search staff to arrange your visits so they have as little impact on your work as possible.</p>

What assistance you'll have during each trial "visit"

Whether you have to go to a medical center for all the visits or some can be done at your home, these questions can help you to find out what to expect and what support may be important for you to make trial participation as easy as possible.

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

<p>15 Will someone help us go from one department to another at the medical center?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>If this is something you want, ask if the center can arrange for someone to meet you and help you get from one department to another while you're there. This can be useful for people who have limited mobility or difficulty seeing, are bringing young kids along, or just want help getting around a medical center that's new to them.</p>
<p>16 Will I/we have accessibility support during visits? (eg for cognitive, motor, visual, or hearing needs)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>It's often possible for the medical center to provide accessibility support. Let the research team know what your specific accessibility concerns are because it may not be obvious to them and ask if the kind of support you would need will be provided.</p>
<p>17 Will I/we have access to amenities during clinical trial visits? (eg, power outlets to charge phones or equipment, free Wi-Fi access, devices for entertainment during wait times, a refrigerator to store medicine(s) or food, etc)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Ask the research staff so that they can assist with any needs that would make clinical trial participation more comfortable for you. Don't be afraid to tell them exactly what you would need, because the research staff may not know you need it.</p>

The medical tests you'll have to do during the trial

Most clinical trials for rare diseases do require people to do a lot of medical tests. Some of these tests are done to make sure the person is safe while taking the trial drug and some are done to check if the trial drug is working as hoped. Which tests have to be done depends on the trial. How easy or hard it would be for someone to complete the tests really depends on each person and each rare disease. These questions may help you to figure out what would be easy or hard for you to do.

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

18 Will I/they have to do any tests that take a long time/are painful or inconvenient, like fasting (not eating for a while)?	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>If Yes, ask how often you would have to do this and what could be done to make these easier for you. The medical center may be able to do some things to help make it easier. For example, by scheduling the tests in which you have to fast first thing in the morning, breaking up the visit to let you have some rest in between tests, or making sure you have Wi-Fi access while you wait.</p>
19 Will any of the tests cause inconvenient after effects like pain or fatigue that I/they would have to plan for? (eg, could make it hard for you to do the daily activities you usually do)	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>The medical center may be able to do some things to make these effects easier. For example, by giving you medication to help manage unpleasant effects or arranging a hotel room for you so you don't have to travel home until the next day. Tell the research staff what impacts on your daily activities would be a problem for you and ask if there are ways to manage this.</p>

What you'll have to do at home during the trial

Most clinical trials for rare diseases also require people to do some things at home, like preparing and taking the trial drug, filling out questionnaires, and watching for and reporting any side effects (changes to their health).

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

<p>20 Will someone help us go from one department to another at the medical center?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Many new treatments may cause serious side effects or be uncomfortable. The medical term for these is "adverse events."</p> <p>Ask what "adverse events" are expected with the trial drug. Since new drugs are tested in many ways before they are allowed to be tested in people, usually research staff know about many of the side effects that can be expected. But unexpected side effects are also possible (known as "unanticipated adverse events"). It's also possible the new treatment may not work for you and your condition may worsen.</p> <p>It is important to know research staff are legally required to monitor your health and make sure that you are safe while you participate in the trial.</p>
<p>21 Will I/we have accessibility support during visits? (eg for cognitive, motor, visual, or hearing needs)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>If you have concerns about how to take the drug, work with the research staff to receive training. You can also ask for any additional tools that may be available. For example, some clinical trials provide written instructions, pictures, and/or videos. Not all clinical trials provide the same tools, but it is important to take advantage of any resources that are available if you feel they would be helpful.</p>
<p>22 Will I/we have access to amenities during clinical trial visits? (eg, power outlets to charge phones or equipment, free Wi-Fi access, devices for entertainment during wait times, a refrigerator to store medicine(s) or food, etc)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Most equipment can be provided if you need it to prepare to take the trial drug. Ask research staff to tell you what equipment will be needed and how it will be delivered to you.</p>

What happens when the trial ends?

Ask the person you speak with about the trial...this may be a Coordinator, Doctor, or Researcher (sometimes called a principal investigator)

What was their answer?

Ask yourself... how hard would that be for you?

What may be possible...If it's not easy for you, speak up about what you need to make it easier...

<p>23 Will I have to watch for and report any unpleasant or dangerous side effects?</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Some trials allow for patients to continue taking the drug after the trial ends, but not all trials do. The trials that allow for patients to continue taking the drug do this by:</p> <ul style="list-style-type: none"> - Transferring patients to a Special Access Program (SAP), - Transferring participants to a 'Long-Term Follow-Up' trial, or - Giving them priority access to enroll in the next clinical trials for the drug <p>Ask the research staff if any of these options will be made available to you if you participate.</p>
<p>24 Will I/we have to prepare and take the trial drug while at home? (mix, measure, and inject)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Some trials guarantee that participants will get an easy-to-read summary of what the results of the trial were. Some don't. If this is important to you, ask if it will be provided and how you will get a copy.</p>
<p>25 Will I/we need to have any special equipment to prepare and take the trial drug at home? (refrigerator space, weight scales, calculators, etc)</p>	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Not Sure	<input type="checkbox"/> Easy <input type="checkbox"/> Hard <input type="checkbox"/> Impossible <input type="checkbox"/> Not Sure	<p>Many clinical trials allow people to have access to their records, but not all, and in some cases, only after the trial ends or the participant completes the trial. Ask the research staff if the trial will allow you to have access to the results of the tests you will be doing. Be sure to ask to know when and how you will receive these results.</p>

A special thank you to ICON and the 2020-2021 Rare Disease Advisory Council (RDAC) members:

Luke Rosen

Founder of KIF1A.org, VP of Patient Engagement & Government Affairs, Ovid Therapeutics

Amy Price

Director of Client Services, VOZ advisors & Metachromatic Leukodystrophy Parent Advocate

Tracy Dixon-Salazar, PhD

Executive Director at LGS Foundation

Maureen McArthur Hart

Board Member, Cure VCP; Scientific Advisory Council member, Uplifting Athletes

Kyle Brant

Founder & Director of ride ATAXIA
Spokesperson for FARA

Terry Jo Bichell

Founder & Director of COMBINEDBrain

Kyle Brant

Founder & Director of ride ATAXIA
Spokesperson for FARA

Andra Stratton

Program Associate, Rare As One at Chan Zuckerberg Initiative & President & Co-founder Lipodystrophy United





CONTACT US

Community members are encouraged to contact us via email: info@multiplesystematrophy.org or by postal mail:

MSA Coalition
1660 International Drive, Suite 600
McLean, VA 22102

or by phone: Support Hotline: **866-737-5999** Business Line: **866-737-4999**

Please review our comprehensive website for complete information and resources: <https://www.msacoalition.org>

Donate at: <https://www.multiplesystematrophy.org/donate-now>

The MSA Coalition is honored to have served the MSA community for over 30 years and counting.
