Section 8: Miscellaneous

In this section:

- Financial Assistance Record
- Insurance Call Log
- Health Insurance Appeal Tracking Form
- Health Insurance Glossary
- What to ask when you are interested in a Clinical Trial

How to use this section:

- Use this section to keep track of miscellaneous items.
- We have included some information that patients & caregivers have found helpful.

Notes:

Financial Assistance Record

When you apply for financial assistance, keep track of the applications (as outlined in the chart below) to make sure you submit everything and hear back from the programs in a timely manner. You may have to follow up with some programs. If a program provides reoccurring support, note that in the frequency column.

Assistance/Organization	**Date Application Submitted**	**Status**	**Frequency: For example: one time, every month, etc.**	**Amount of Assistance Received**
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		
		☐ Applied/Pending ☐ Granted ☐ Denied		

Insurance Call Log

Keep a record of every time you call or communicate in any way with the health insurance company. You may have questions about coverage, need to file a claim, or need to obtain a preauthorization for a test or treatment. You can use the call log below to keep track of calls so you can reference the information later.

DATE	NAME OF REPRESENTATIVE	REASON FOR CALL	NEXT STEPS	NOTES

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Health Insurance Appeal Tracking Form

Step/Action	Date	Contact Name and Information	Comments/Notes
Before the appeal is started			
Date of service (the date when medical service was received) and what service was received			
Claim sent to the insurance provider			
Received response from insurance company (Explanation of Benefits and/or other written communication.			
If claim denied, the date I talked to my healthcare team and asked for supporting documentation I need			
Received supporting documentation from healthcare team			
Internal appeal			
Sent insurance company my first appeal form (1st internal appeal)			
Received a response from my insurance company			
If internal appeal is denied, I received a written explanation from my plan stating the reason it used to deny my claim			
I filed my second appeal form (2nd internal appeal-[only in cases where it is required by state law or company policy])			
If claim denied, I talked to my healthcare team and asked for any additional supporting documentation			
Received supporting documentation from healthcare team			
External appeal			
Filed forms and documentation for external appeal with the appropriate agency • Triage Cancer has the contact information for every state's health insurance agency available at **www.triagecancer.org/stateresources**			
Received a response to my external appeal from the independent review organization/entity			

Health Insurance Glossary

Affordable Care Act (ACA) The comprehensive health care reform law enacted in March 2010 by President Barack Obama (sometimes known as ACA or “Obamacare”).

Allowed Amount / Maximum Allowable The maximum dollar amount that your plan will pay for covered health care services.

Appeal A request which is typically made to a health insurer by you or your provider to review and reconsider a coverage decision.

Balance Billing When a physician bills you for the difference between the physician’s charges and the amount that is allowed by your plan (i.e., allowed amount). Patients are not typically responsible for this cost when services are in-network and covered.

Benefits The health care services (e.g., office visits, diagnostic tests, etc.) and items (e.g., prescription drugs) that are covered by your insurance plan.

Claim The document your physician submits to your health insurance company to receive payment for services provided.

Coinsurance The percentage of costs of a covered health care service or item that you are responsible for paying generally after you have paid any deductibles required by your plan.

Commercial Health Insurance Health insurance administered by a private entity rather than a government agency. You may receive commercial insurance through an employer (nearly half of Americans receive commercial insurance through their job), or you can purchase health insurance directly from an insurance carrier, through federal marketplace or through an insurance broker.

Coordination of Benefits If you have more than one health insurance plan; the plans work to determine their respective payment responsibilities for a claim.

Copayment A set dollar amount that you pay for a specific health-related service, such as a doctor’s visit or prescription drugs.

Cost Sharing The amount you pay out-of-pocket for health care services covered by your health insurance plan, after the plan determines their respective payment responsibilities for a claim. This includes copayments, coinsurance and deductibles, but does not include premiums (with the exception of Medicaid and the Children’s Health Insurance Program), balance billing amounts for out-of-network care, or the cost of non-covered services.

Deductible A set amount that you must pay each benefit year before your health insurance plan will start to pay for most covered health services. After your deductible is met, you are typically only responsible for a copayment or coinsurance for covered services.

Dual-Eligible Beneficiaries Beneficiaries who are enrolled (or eligible to enroll) in both Medicaid and Medicare. Dual-eligible beneficiaries typically have limited cost sharing and may qualify for assistance with Medicare premiums.

Employer-Sponsored Health Insurance / Group Health Plan An insurance policy selected by the employer and offered to eligible employees and their dependents.

Explanation of Benefits (EOB) A notice that summarizes the services, charges, and payment for services you have received. An EOB is not a bill, although it may look like one.

Flexible Spending Account (FSA) An account, typically arranged through your employer, that lets you pay for many medical expenses with tax-free dollars.

Formulary / Drug List A list of prescription drugs covered by your health insurance plan. Formularies are typically divided into tiers or levels of coverage with defined cost sharing that increases for higher-tier drugs.

5-tier plan

- Tier 1: preferred generic drugs (lowest cost)
- Tier 2: non-preferred generic drugs and preferred brand name drugs
- Tier 3: non-preferred brand name drugs name drugs
- Tier 4: preferred specialty drugs
- Tier 5: non-preferred specialty drugs (highest cost)

Health Insurance Portability and Accountability Act of 1996 (HIPAA) A federal law that protects sensitive patient health information from being disclosed without the patient's consent.

Health Maintenance Organization (HMO) A health insurance plan that provides care for members through a defined network of providers and hospitals. Under an HMO, members are typically required to select a primary care provider (PCP) and obtain referrals for specialists, and out-of-network care is typically not covered (except in an emergency).

Health Reimbursement Arrangement (HRA) Employer-funded group health plans from which employees are reimbursed tax-free for qualified medical expenses up to a fixed dollar amount per year.

Health Savings Account (HSA) A type of savings account for high deductible health plan (HDHP) beneficiaries that lets you set aside money on a pre-tax basis to pay for qualified medical expenses (e.g., deductibles, copayments, coinsurance, and some other expenses). HSA funds generally may not be used to pay premiums.

High Deductible Health Plan (HDHP) Health insurance plans with relatively low monthly premiums and high deductibles. With a HDHP, preventative care may be covered in full prior to meeting your plan's deductible. For 2021, the IRS defines a high deductible health plan as any plan with a deductible of at least $1,400 for an individual or $2,800 for a family.

In-Network / Preferred Provider A provider who accepts your insurance and has contracted with the health plan to provide services at a discounted rate. Some plans may have a tiered network where you pay extra to see select providers.

Medicaid A joint state and federal government insurance program that provides free or low-cost health insurance to some low-income people, families and children, pregnant women, the elderly, and people with disabilities.

Medicare A government program that provides health insurance for individuals 65 years or older and certain people with disabilities under the age of 65.

- **Medicare currently offers four different insurance programs: Parts, A, B, C, and D**

  Part A includes:

  - Hospital inpatient services
  - Hospice Care
  - Certain home health services
  - Qualified skilled nursing facility care

  Part B includes:

  - Procedures in the physician office and hospital outpatient settings
  - Physician services in all settings of care
  - Office and hospital outpatient administered drugs
  - Laboratory drugs
  - Ambulance services
  - Durable medical equipment (DME)

  Part C includes:

  - Privately managed alternative to Medicare Fee-for-Service (or Original Medicare)
  - Private healthcare plans (primarily managed care plans) offer combined Part A and Part B Medicare benefits
  - Part C is also known as Medicare Advantage (MA)

  Part D includes:

  - Prescription drugs
  - Part D benefits can be offered as standalone Prescription Drug Plans (PDPs) or Medicare Advantage-Part D prescription drug plans (MA-PDs)

Medically Necessary Health care services or supplies needed to prevent, diagnose or treat an illness, injury, condition, disease or its symptoms and that meet accepted standards of medicine. Medical services generally must be considered medically necessary in order to be covered by insurance. Your health plan may request additional information from your physician to confirm whether a treatment is medically necessary.

Medigap A supplemental insurance policy sold by private insurance companies to help fill gaps in Original Medicare by covering some outstanding medical costs beyond what is paid for by Medicare (e.g., copayments, coinsurance, and deductibles).

Open Enrollment Period The time of year when you are able to sign up for a health insurance plan for the following year. If you are not satisfied with your current health plan, the open enrollment period may be the only opportunity to switch to a different plan.

Out-of-Network / Non-Preferred Provider A provider who has not contracted with your health insurance plan. Out-of-pocket costs are typically higher for out-of-network care; in some cases, you may be responsible for the full cost when you see an out-of-network provider.

Out-of-Pocket Limit / Maximum Amount The amount you will have to pay for covered services in a plan year. After this amount is met, the health insurance will pay 100% of the costs for covered services. The out-of-pocket limit often excludes: • Your monthly premiums • Anything you spend for services that your plan does not cover • Office visit copay • Out-of-network services • Costs above the allowed amount for a service that a provider may charge (i.e., balance billing)

Pre-Authorization/ Prior Authorization / Pre-Determination / Pre-Certification A request submitted (typically by a physician) to your health insurance plan prior to treatment to determine if specific health care services or items are considered medically necessary by your plan. Some plans may require one of these for certain services before you receive them; however, these requests are not promises that your health insurance or plan will cover the cost.

Pre-Existing Condition A disability or illness that you have been treated for before enrolling in a new health plan.

Preferred Provider Organization (PPO) A health insurance plan with a defined network of providers and hospitals where patients can access services at a lower cost when compared to out-of-network care. Under a PPO, members may be able to go to specialists without a referral from a PCP and obtain out-of-network care for an additional cost.

Premium The amount that you pay to your health insurance plan for coverage (generally on a monthly basis).

Primary Care Provider (PCP) A physician, nurse practitioner, nurse specialist, or physician assistant that provides and coordinates your care. Some health plans (e.g., HMOs) may require that you go through your PCP to get a referral to specialists.

Referral A written order (i.e., prescription) from your PCP to see a specialist or to get certain medical services. Health Maintenance Organizations (HMOs) often require a referral before you can get medical care from anyone other than your PCP (without a referral, your plan may not pay for the services).

Specialty Drug Typically a high-cost drug to treat serious, chronic, rare, or life threatening diseases. These drugs may have special storage or shipment requirements and require additional education and support from a health care professional.

Subsidized Coverage Health coverage available at reduced or no cost for people that meet certain income eligibility requirements.

Special Enrollment Period (SEP) A time outside of the annual open enrollment period when you can enroll in a health insurance plan if you've had a certain life event, such as marriage, birth of a child, or loss of health insurance coverage (for example, due to losing a job).

Specialist A provider that focuses on specific areas of medicine rather than a general practitioner.

Specialty Pharmacy Pharmacies that are able to distribute specialty drugs. They are typically equipped to handle specialty products that may have unique storage or handling requirements and may provide patient support beyond a traditional pharmacy including coordination of care and disease management program. Some payers may require you to obtain specialty drugs through a "preferred" or in-network specialty pharmacy or pay an additional cost to use an out-of-network specialty pharmacy.

Supplemental / Secondary Insurance An additional health insurance plan that helps pay for services and/or out-of-pocket costs that are not covered by your main health insurance plan.

These definitions have been adapted from the following sources:

Healthcare.gov. Glossary. Available at: https://www.healthcare.gov/glossary/

Medicare.gov. What's Medicare Supplement Insurance (Medigap)? Available at HSAs, FSAs, and other types of job-based coverage https://www.healthcare.gov/job-based-help/#/coverage

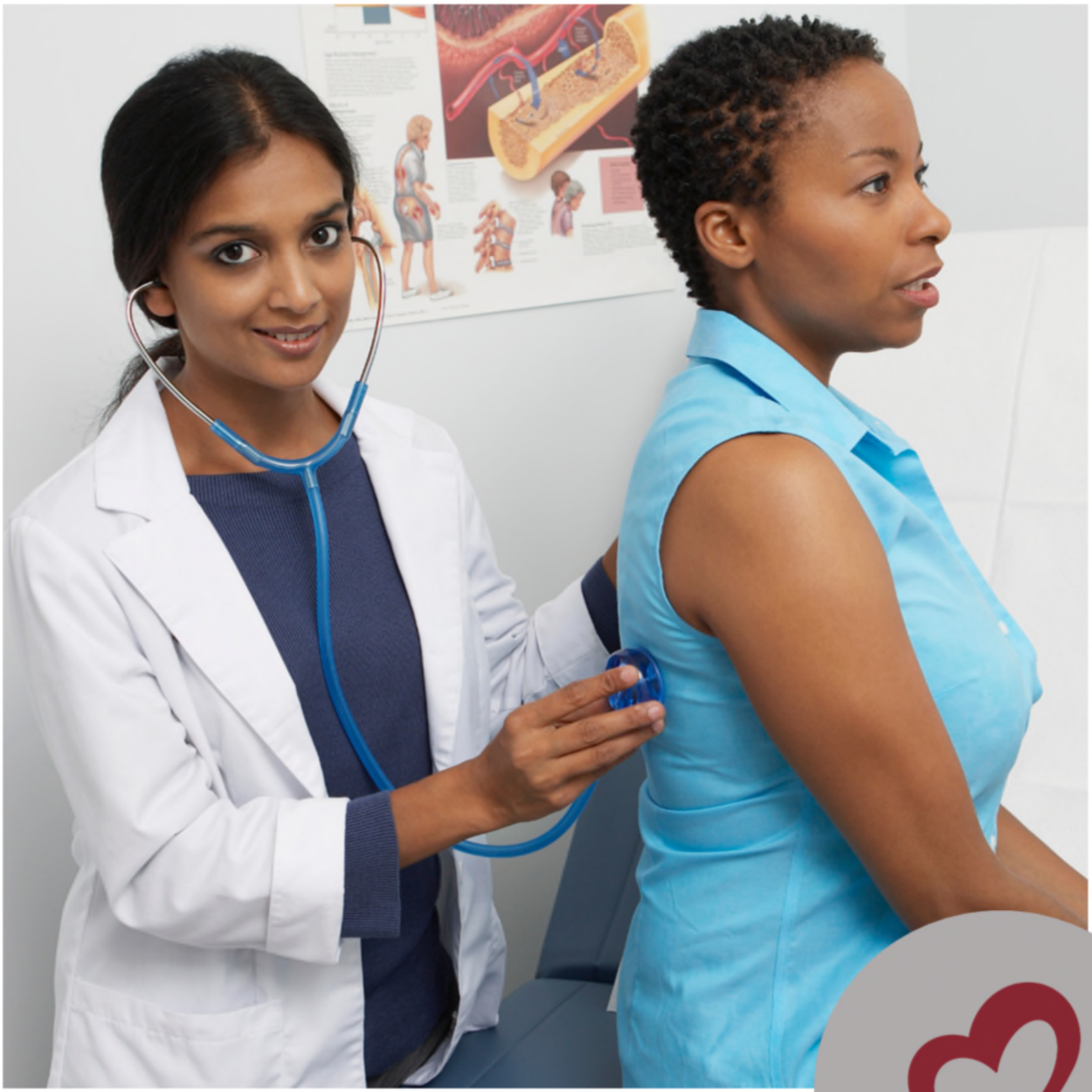
Medicare.gov. Glossary. Available at: https://www.medicare.gov/supplements-otherinsurance/whats-medicare-supplement-insurance-medigap

CMS. What's Medicare Supplement Insurance (Medigap)? Available at: https://www.medicare.gov/supplements-other-insurance/whats-medicare-supplement-insurance-medigap

CMS Medicare Learning Network. Dually Eligible Beneficiaries Under Medicare and Medicaid. Available at: https://www.cms.gov/Outreach-and-Education/Medicare-Learning-Network-MLN/MLNProducts/Downloads/Medicare_Beneficiaries_Dual_Eligibles_At_a_Glance.pdf

AMCP. Managed Care Glossary. Available at: https://www.amcp.org/about/managed-carepharmacy-101/managed-care-glossary

What to ask when you are interested in a clinical trial



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Contents

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2020 – 2021 Rare Disease Advisory Council (RDAC) members:

Luke Rosen

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

Amy Price

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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 just not realize that it's important to you! Each rare disease is unique and each person is unique, so 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 trail…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…**
1	**Will I/they have to stop taking any medicines/ treatments/supplements for a few weeks**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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).
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	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
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?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
4	**Will I/we be able to bring in results from tests I/we have already done to avoid doing them again during screening?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
5	**Will I/we have to go to a specific medical center for all the screening process tests?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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**.

Ask the person you speak with about the trail…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…**
6 **Will I/we have to make arrangements myself to do any of tests required?** (for example, blood work or genetic testing)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
7 **Will I/they be giving up the opportunity to participate in other trials or get any approved treatments by joining this trial?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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. **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.
8 **Is there a chance that I/they would get a placebo instead of the trial drug?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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. **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.

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 may total "visits" you would have to do depends on the clinical trial.

Ask the person you speak with about the trail...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?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
10 **Will I/we have to pay for gas/parking/train travel/air travel to get there?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	In some trials, it's possible to either: ⇒ Get a refund after you submit your receipts for things you had to pay for to get to the medical center ⇒ Get 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 **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.
11 **Will I/we have all the equipment needed to travel that distance?** (eg, wheelchair, feeding equipment, respiratory equipment, etc)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
12 **Will I/we have to be away from home overnight for any of the trial visits?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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 apart-ment 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.

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 may total “visits” you would have to do depends on the clinical trial.

	Ask the person you speak with about the trail…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?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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). **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 research staff to arrange your visits so they have as little impact on your work as possible.

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 don 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 trail…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…**
15 **Will someone help us go from one department to another at the medical center?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
16 **Will I/we have accessibility support during visits?** (eg for cognitive, motor, visual, or hearing needs)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
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)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	**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.

The medical test 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 trail…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)?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
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)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.

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 trail…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…**
20 **Will I have to watch for and report any unpleasant or dangerous side effects?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	Many new treatments may cause serious side effects or be uncomfortable. The medical term for these is “adverse events.” **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. 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.
21 **Will I/we have to prepare and take the trial drug while at home?** (mix, measure, and inject)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
22 **Will I/we need to have any special equipment to prepare and take the trial drug at home?** (refrigerator space, weight scales, calculators, etc)	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.

What happens when the trial ends

Ask the person you speak with about the trail…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…**
23 **Will I/they be able to continue taking the drug after the trial ends if it helped?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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: –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 **Ask** the research staff if any of these options will be made available to you if you participate.
24 **Will I/we get a summary of what the results of the trial were?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.
25 **Can I get a copy of my test results?**	○ Yes ○ No ○ Not Sure	○ Easy ○ Hard ○ Impossible ○ Not Sure	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.