

STANDING UP for your health



SELF-ADVOCACY FOR PATIENTS WITH RARE DISEASES



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This self-advocacy guide outlines key principles that empowered patients with rare diseases can use to advocate for their best healthcare. This booklet is designed to encourage you to fully participate in your healthcare and is not meant to be a substitute for the advice of your doctor.

Developed by



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For more information on patient advocacy, visit us online
at www.AAMDS.org or call us at
(301) 279-7202 or (800) 747-2820.

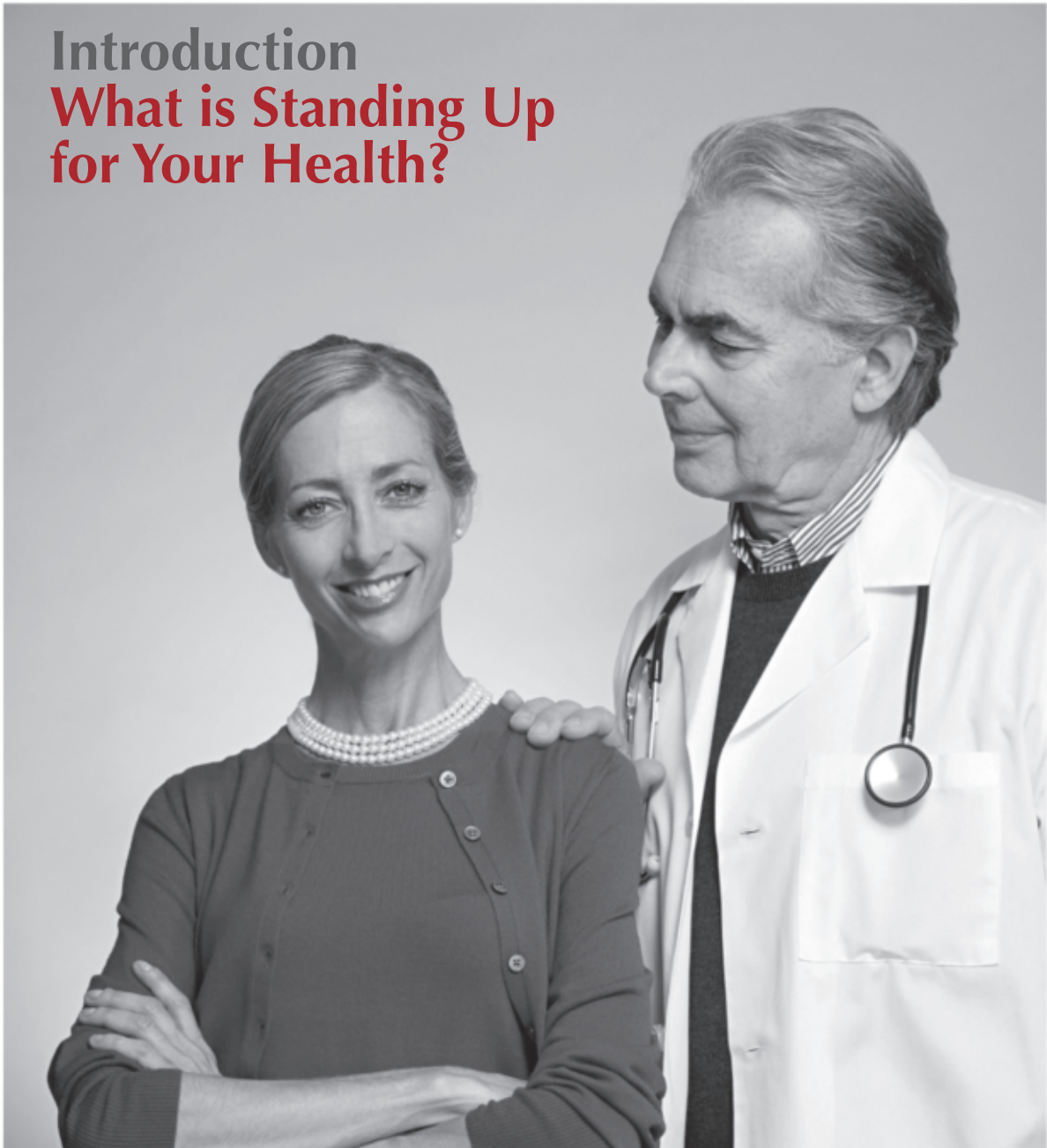


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Introduction

What is Standing Up for Your Health?



“I’m definitely the kind of patient who just takes charge. It can be a scary thing to have other people be in charge of your healthcare.”

-Valerie, patient

“It’s really important for patients to be strong advocates for themselves. Being in control can lead to better outcomes and better quality of life.”

-Isabel Schuermeyer, MD

What is Standing Up for Your Health?



This guide was developed to empower a large, often forgotten and underserved population - the 30 million Americans with rare diseases who face challenges in obtaining an accurate diagnosis and have difficulty finding medical experts with experience to manage their rare diseases.

Standing Up for Your Health means being a strong self-advocate and an empowered patient. It means taking a more active role in your care to ensure you or your loved one receive the best care and treatment available. Empowered patients learn all they can about their disease or condition and their treatment options so they can fully participate in their care.

Originally created by the Aplastic Anemia & MDS International Foundation, this program was developed to discover the steps that empowered patients and strong self-advocates take to enable them to receive high quality care.

To bring you the most up-to-date information, we consulted with medical experts and interviewed patients with rare diseases about the skills they use as they effectively navigate the healthcare system. We read the latest research and talked with physicians, nurse specialists, and patients and their families to get their perspective on the skills needed for good health advocacy. Then, we compiled what we had learned into logical steps you can take toward standing up for your health.

This book includes tools, learning experiences, materials, and resources for patients with rare diseases and their families. Our goal is to help patients with rare diseases on their journey to become a more effective advocates for their own healthcare and more powerful patients. For more information, including videos, stories and other components, visit the *Standing Up for Your Health* website at www.AAMDS.org/StandingUp.

Whether you are newly diagnosed or have been living with a rare disease for many years, the practical strategies offered in this book are designed to help you to stand up for your health.

Why Self-Advocacy Matters

Why is Self-Advocacy Important to Patients With Rare Diseases?

Self-advocacy implies that a person makes an informed decision about a matter of importance to her or him and then takes responsibility for bringing about the change necessary to make that choice a reality. A rare disease is an illness that affects fewer than 200,000 people in the United States. There are approximately 6,800 different rare diseases, which together affect up to 30 million Americans. Rare diseases encompass a wide variety of conditions including cancers and genetic diseases.

Rare diseases are often chronic. Because a disease is rare, there may be a limited number of “experts” in the treatment of the disease. Individual patients with rare diseases must search for information about their conditions and seek out other resources and individuals with their disease, often on their own. Self-advocacy can help patients and their families be empowered to fully participate in their care. Self-advocacy is a necessity for patients with rare diseases.

Self-Advocacy Can Help You Get High-Quality Health Care

High quality health care means getting the right care at the right time, delivered by the right health care professional for you. When standing up for your health, you do everything you can to get high quality care. Many strategies for improving the quality of care in the United States increasingly focus on the patient role in managing his or her own health. Patients play an important role in determining the need for care and the outcomes of that care.

Self-Advocacy Can Help You Feel in Control

At times, you may feel like your rare disease is running your life. Standing up for your health can give you back a sense of control. When standing up for your health, you get the information you need to make informed decisions about your medical treatment and your healthcare providers.

Self-Advocacy Can Help You Get Support

When standing up for your health, you ask for help from family, friends, and others. Their support can help you cope with your disease.

Are You Standing Up for Your Health?

Read this book and share it with your family and friends. You’ll find practical tips, patient stories, and other resources and tools you can use to become an empowered patient and strong self-advocate.



Principle 1

Build a Strong Healthcare Team



“The best thing I ever did? Finding healthcare providers I trust. This way I’m comfortable being an active participant. I truly feel it is not only my right, but also my responsibility to fully participate in making decisions about my care and medical treatment.”

-Earl, patient

Principle 1 Build a Strong Healthcare Team



“Make sure your provider is a good fit. You should have a shared understanding of your disease and treatment and be able to communicate well together. It’s a relationship that can last for years.”

-Ruben Mesa, MD

“Patients need to realize that their doctor is their partner.”

-David Steensma, MD

Be a Strong Player on Your Healthcare Team

Your doctors, nurses, and specialists will offer you much of the information you need to make informed health decisions. Your healthcare team can also include your pharmacist, nurse practitioner, and physician assistant. You may also have a mental health provider such as a social worker, psycho-oncologist, or psychologist on your team. Other team members may include researchers, lab technicians, and naturopaths.

Empowered patients see themselves at the center of their healthcare team. Only you can make sure you get your questions answered and ensure that your goals for treatment are considered.

Only you can:

- Keep your healthcare team informed about symptoms and side effects
- Share your goals related to treating your rare disease
- Follow through on treatments
- Make the tough choices about your healthcare

Building a strong healthcare team is important for effective self-advocacy. First, find an expert in your rare disease. Put together a team of skilled healthcare providers whom you trust and can talk openly with. Finally, take steps to make sure you are doing your part.

Step 1: Find an Expert in Your Rare Disease

In order to receive the best treatment, you'll want to see a doctor who is an expert in your disease. This will be someone who has treated similar cases and stays up-to-date on treatments. While ideal, it is not always possible to find an expert near you who has experience with your rare disease.

Although there is no list of experts in rare diseases, the guidelines below from the National Institutes of Health (NIH) Office of Rare Diseases Research include several ways to identify healthcare professionals who have experience with a particular condition. Potential resources include patient advocacy groups, researchers who have conducted or are conducting clinical trials, and authors of articles published in the medical literature.

How can I find a healthcare professional with experience in a specific condition?

Here are some resources that may be able to assist in your search for a healthcare professional with experience in your rare disease:

- Contact a disease or patient advocacy organization specific to your disease. Many disease advocacy organizations have medical advisory boards, physician locator services, or patient networks, all of which may help you find a healthcare professional who is familiar with a particular condition.
- You can search for a condition on the NIH website (www.nih.gov) to find related disease advocacy organizations. These would be located in the "Organizations" section. If you don't find a specific group, search the Genetic Alliance (www.geneticalliance.org) and the National Organization for Rare Disorders (NORD) websites (www.rarediseases.org).



- You can also search the medical literature to find healthcare professionals or researchers who have published recent articles or case reports on a particular condition. You can find relevant articles through the U.S. National Library of Medicine PubMed, a searchable database of biomedical journal articles (visit www.ncbi.nlm.nih.gov/pubmed). Although not all of the articles are available online for free, most articles listed in PubMed have a summary/abstract available. In addition, contact information for one of the authors may be listed. On the “Results” page, select “Abstract” under Display Settings to view information about the authors.
- Contact a teaching hospital. Call the appropriate medical department at a teaching hospital affiliated with a local university. In many cases, the doctors at these teaching hospitals are most familiar with rare diseases. Because they are in a research and teaching setting, they may be able to offer information on both the standard therapies and new investigational treatments.
- Ask the local community specialists you are seeing. Many times, local doctors will know of specialists who are treating your specific rare diseases.

You may need to go out of your area to visit an expert who is very experienced with your condition. Ask this expert to keep in touch with your local healthcare team. For resources, you can use to receive help paying to see an expert, see page 28.

How can I find a researcher who is studying my condition?

Researchers who are studying a specific condition are another source for identifying an expert. You may want to look for researchers who are conducting a clinical trial, as they are often medical doctors. In addition to asking for more information on their research, you can also find out if they see patients who are not enrolled in a study. If a researcher does not see patients, you may consider asking if they know a colleague who could help you. Some researchers publish periodic updates on their discoveries, and it may be possible for you to receive the latest information about their research.

- **The Rare Diseases Clinical Research Network (RDCRN)** is made up of 19 consortia that are working to improve the availability of rare disease information, treatment, clinical studies, and general awareness for both patients and the medical community. The RDCRN also aims to provide up-to-date information for patients and to assist in connecting patients with advocacy groups, expert doctors, and clinical research opportunities. Visit the www.rarediseasenetwork.org to see if your condition is supported by one of the RDCRN consortia.
- **ClinicalTrials.gov** was developed by the U.S. National Institutes of Health, through the National Library of Medicine, to provide patients, family members, and members of the public with current information on clinical research studies. Through this resource, you may be able to find researchers who are studying your condition. You can conduct a basic search using the condition name as your search term. After you click on a study, review its “eligibility” criteria to determine its appropriateness. Use the study’s contact information to learn more. Check clinicaltrials.gov often for regular updates.
- **The Patient Recruitment and Public Liaison (PRPL)** Office at the National Institutes of Health (NIH) provides information about participating in research being conducted at the NIH Clinical Center hospital. Call the PRPL toll-free at (800) 411-1222, or send an e-mail to prpl@mail.cc.nih.gov to contact a liaison who can help you determine if there are any open clinical trials that interest you. They may also be able to provide you with contact information for researchers involved in these trials.
- **The Genetic Testing Registry** www.ncbi.nlm.nih.gov/gtr lists laboratories offering research genetic testing for many conditions. Much of this research is conducted by physicians and scientists with an interest in a particular disease. To see if there are researchers studying your condition, visit the website above and use the condition name as your search term. Click on the “Research” tab on the results page.

Learn about Your Doctor's Background

To learn more about your doctor's competence and experience, you may want to visit:

Federation of State Medical Boards

This group offers profiles on licensed doctors, osteopathic doctors, and physician assistants. For a small fee, you can find out facts about your doctor's education, any disciplinary actions, and certification, among other areas. To order a report, go to www.docinfo.org.

Some information on licensed doctors may also be available online for free through your state medical board. To find a listing of websites for state medical boards, go to <http://www.fsmb.org/state-medical-boards/contacts>

American Board of Medical Specialties

This group will tell you for free if your doctor is board certified in the specialty area that treats your disease. A doctor who is board certified has shown great expertise in a specific area of medicine.

To find out if your doctor is board certified, call (866) 275-2267. Or, go to www.abms.org

Interview the Expert

Once you find an expert in treating your disease, you will want to interview him or her. In some cases, you can call the office and speak directly to the physician or a nurse manager for a couple of minutes at no charge. In other cases, you will need to schedule an initial visit.

Before you go to see an expert, find out whether the doctor is covered under your insurance. Some insurance plans allow you to see someone outside your insurance plan if you get pre-approval first. Others require you to pay out of pocket, or find another expert who is covered by your insurance.

Questions to Ask an Expert

- In what practice areas are you board certified?
- How many patients with my disease have you treated?
- What were the outcomes?
- Can I talk with a nurse or social worker who works with you?
- At which hospitals do you have privileges to practice?

Consider having a mental health provider on your team. A social worker, psychologist, psychiatrist, or psycho-oncologist can help you manage stress, anxiety, depression, and other emotional symptoms. Be sure to let your doctor(s) know if you have any of these symptoms. Don't be afraid to ask for a referral.

Step 2: Choose Healthcare Providers Who Are a Good Fit for You

“My doctor, specialist, and nurse practitioner all have a great bedside manner and are up to speed on the information I need. I did a lot of background checking before picking my team, and it has paid off for me. We put our heads together to come up with solutions.”

- Lynn, patient

You have seen a lot of doctors, nurses, and other health care professionals over the years. Some you have liked better than others. Use the worksheet on page 18 to help you understand what is most important to you in the members of your healthcare team.

Feel Free to Get a Second Opinion

“The doctor is there to serve you, and if you're not comfortable with what he or she is recommending, you can switch doctors and get a second opinion.”

-Anthony, patient

“I was very nervous going to see another specialist. I was sort of feeling like I was betraying my specialist. My sister went with me and that really helped. Turns out my original specialist didn't have a problem with me seeking a second opinion. In the end, I wound up switching because I liked working with a female doctor better.”

-Kim, patient



Even if you're happy with your healthcare team, it's okay to get a second opinion. Getting a second opinion will not offend your doctor. In fact, most healthcare providers appreciate and encourage another point of view. And it's your right.

Seeking a second opinion can help you and your family with making difficult decisions about your treatment. While most insurance companies will cover a second opinion, it is a good idea to check with your insurance company before seeing the new healthcare provider. You should be aware of and keep track of your out of pocket expenses.

Step 3: Select a Care Coordinator

"Since I live in a rural area, there isn't a doctor in town who has seen many patients with my rare disease. Still, I want to see a specialist who knows a lot about my diagnosis—someone who has treated people like me and who stays up-to-date on the latest treatments. So, I go outside my area a few times a year to see a doctor who is very familiar with treating my disease. My local doctor coordinates my care by staying in touch with my specialist."

- Ruth, patient

Your care may be very complex and involve many specialists. You may find it helpful to choose one healthcare professional who has the main responsibility for coordinating your care. Some patients use a medical specialist as their care coordinator, others work with their primary care physician.

Your care coordinator can:

- Help arrange consultations with specialty doctors and supportive care providers
- Maintain your medical records in his or her office
(Of course, you'll also want your own copies of your records.)
- Talk with your other healthcare team members when needed

Care coordinators and health advocates can play different roles. To learn about health advocates, read page 46 in this book.



Know How to Reach Your Healthcare Providers

You need to know how to reach your healthcare providers, day or night. Find out the best ways to contact them. Some healthcare providers like email, while others prefer the telephone. Be sure to get their email addresses and phone numbers.

If your healthcare provider is not available when you call, leave a detailed message. This will help you avoid telephone tag. Tell the office if it is okay to talk to a family member or leave a message if you're not able to come to the phone. You may need to give the office permission in writing.

Self-Advocacy When You Are in the Hospital

While you know about the healthcare providers you work with on a regular basis, it is different when you need to go into the hospital. You'll want to find out what the healthcare providers' names are and who does what. Ask healthcare providers to write down their names and what they do. Or, ask if they can leave their business cards. Find out who to talk with about:

- Medical issues
- Home care
- School or job issues
- Emotional issues
- Family issues

Step 4: Do Your Part to Become an Empowered Patient

Remind yourself that you are at the center of your healthcare team. Once you have your medical care in place, you'll want to concentrate on what you can do to be a strong player on your healthcare team. Try the tips listed below as you do your part:

Share Information

- Share information with your healthcare team. Tell them your symptoms—no matter how minor they seem.
- List all medicines (including over the counter medicines), vitamins, and complementary and alternative therapies (including herbal remedies or supplements) you are taking.
- Discuss any emotional symptoms you are having. If you are feeling anxious or having trouble sleeping, let them know. The more you share, the better your healthcare team can help you.
- Share your goals for treatment. What are you hoping treatment will do for you?

Be a Team Player

- Go to all your scheduled office visits.
- Once you agree to a treatment plan, be sure to follow through. If the treatment is not what you expected, always talk to your doctor before stopping the treatment.

Make the Most of Office Visits

- Think about what you hope to get out of the visit. Bring your list of questions along with you.
- Ask for a copy of lab results or any health records while you are still at the doctor's office. It can be time consuming and difficult to get records once you leave the office. Some states charge a fee for photocopying your records after the visit.
- Ask your healthcare providers to give you any medicine or treatment instructions in writing. For more tips on making the most of every office visit, see Principle 3 on page 33.

Be Politely Assertive

- Be polite while still being assertive when asking for what you need.
- Try using "I" statements, such as "I think," "I feel," or "I don't understand."

Determine What You Are Looking for in Your Healthcare Providers

Use the checklist below to help you assess what you are looking for when choosing members for your healthcare team. You can also use this checklist to assess how satisfied you are with your current healthcare providers.

1. My doctor considers me an important part of the team and asks me for my feedback.

Very important Somewhat important Not very important to me

2. My doctor is willing to share information freely with me and with other healthcare providers as needed.

Very important Somewhat important Not very important to me

3. My doctor talks openly with me about what I can expect from my treatment and my prognosis (life expectancy).

Very important Somewhat important Not very important to me

4. The office is nearby, and the office hours fit my schedule.

Very important Somewhat important Not very important to me

5. The time in the waiting room is not too long.

Very important Somewhat important Not very important to me

6. The doctor takes the time to explain the staff members' roles.

Very important Somewhat important Not very important to me

7. My doctor has a back-up healthcare provider whom I can reach during emergencies or non-office hours.

Very important Somewhat important Not very important to me

8. My healthcare team members answer my questions and explain things in terms I can understand. If I don't understand, they will offer examples or rephrase in lay terms.

Very important Somewhat important Not very important to me

9. My healthcare team members refer me to the right source if they don't know the answers to my questions.

Very important Somewhat important Not very important to me

10. My healthcare team members give me articles and brochures about my disease.

Very important Somewhat important Not very important to me

11. My healthcare team members return my phone calls and emails in a timely manner.

Very important Somewhat important Not very important to me

List other areas that are important to you as you put together your healthcare team.

Summary

Building a strong healthcare team you trust and can talk freely to is key to becoming an empowered patient. Being comfortable with your team can help you get the answers you seek. And remember, you have the right to seek a second opinion.

It can take time to feel at ease when taking the lead in your healthcare. You've done other difficult things in the past, and you can do this, too. Be patient with yourself and know that with practice, you can develop solid skills as you advocate for your best healthcare.



Skills Checklist

Use this checklist to assess your skills in building an effective healthcare team.

- I have an expert in my disease on my healthcare team.
 - I feel comfortable with each of my healthcare providers.
 - I believe I'm doing everything I can to be an empowered team player.
 - I'm able to reach my key healthcare providers whenever I need to.
 - I feel comfortable getting a second opinion. I find my healthcare team supportive of my seeking a second opinion.
-



Principle 2 **Learn About Your Disease and Treatment Options**

“It’s scary to be diagnosed with a rare disease. Education eases the fear factor. It empowers the patient. You realize, the more I understand this disease, the better I can deal with it.”

- Stephen, patient

“If you have a rare disease, it’s important to be well informed—even before you visit the doctor—so you know the right questions to ask.”

- Mikkael Sekeres, MD

Principle 2 Learn About Your Disease and Treatment Options



Get Up-To-Date Information

Many rare diseases are complex. You need to learn all you can about your condition and the available treatments.

Staying well informed helps empower you to be knowledgeable about your disease and actively share in your treatment decisions. It allows you to educate family and friends, get more out of office visits, make choices about your treatment, and keep an eye on your care.

Today, it's easier than ever to learn about your disease and treatment options. You can get information from books, brochures, newspapers, medical journals, magazines, and—of course—the Internet.

Tips for Getting Information About Rare Diseases

Talk to your healthcare providers. Find a patient advocacy organization. Locate reliable information on the internet. Learn all you can about your treatment options. Consider participating in a clinical trial, and keep an eye on your care.



Questions to Ask Your Healthcare Team

You may want to take this patient guide with you as you ask the following questions:

About Your Disease

- What disease do I have?
- How severe is my disease?
- What is my prognosis (a prognosis is a prediction of the course or outcome of a disease or disorder)?
- What have other people with a similar disease and treatment gone through?

About Treatment

- What are all my treatment options?
- What treatment option do you recommend for me? Why?
- How likely am I to get better with the treatment?
- Has this treatment been used a lot or is it a new or experimental treatment?
- How long will the treatment take to work? When and how will I know if it is working? What happens if the treatment doesn't work?
- What are the potential side effects from treatment?
- Can my disease return, even after successful treatment?

About Prescription Drug Treatment

- Has the drug been approved by the U.S. Food and Drug Administration (FDA) for treating my disease?
- How do I take the medicine? How often do I need to take it?
- How long will I need to take the medicine? When can I expect the treatment to start working?
- What are the common side effects of this medicine? What are the most severe side effects? What can be done to control them? Do you have any tips for staying on the treatment plan?
- What are the long-term side effects of this medicine?
- How much does it cost? Is it covered by my insurance?
- Can this prescription drug cause interactions, or side effects with any other prescription drugs, over-the-counter medicines, or supplements that I take?

For more questions to ask healthcare providers, visit the “Questions Are the Answer” site www.ahrq.gov/questions. This website is maintained by the Agency for Health Research and Quality.

You can create a customized list of questions to take with you to your next doctor's visit. In the months and years to come, keep track of how well your treatment plan is working. Note your symptoms and side effects. Review the plan with your healthcare providers regularly to see if it can be improved.

Find a Patient Advocacy Organization

Patient advocacy is an area of specialization concerned with advocacy for patients, survivors, and caregivers. Patient advocacy organizations exist for many diseases, even rare diseases. These organizations may have information about your disease. They may be able to connect you to other patients who understand what it is like to live with your disease. Patient advocacy organizations may help develop legislation to improve systems or processes for patients. You can search the internet for organizations that focus on your rare disease. Or you can visit any of the following reliable online resources for rare disease patients.

Global Genes Project www.globalgenes.org

The Global Genes Project mission is centered on increasing rare disease awareness, public and physician education, building community through social media, and supporting research initiatives to find treatments and cures for rare and genetic diseases.

Health Hotlines www.healthhotlines.nlm.nih.gov

This is a database of toll-free numbers from the National Library of Medicine (NLM) with descriptions of more than 14,000 biomedical information resources, including organizations, databases, research resources, etc.

In Need of Diagnosis, Inc. (INoD) www.inod.org

This website provides help and support to individuals with undiagnosed conditions.

National Library of Medicine www.nlm.nih.gov

This website has reliable and detailed information on many diseases. It has a simple search tool on the main page. From this site, you can also use MedLinePlus (www.medlineplus.gov), a website for the general public that is maintained by the National Institutes of Health.

National Organization for Rare Disorders (NORD) www.rarediseases.org

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service. The NORD current member list is a good place to find patient advocacy organizations for more than 225 rare diseases: <http://rarediseases.org/patient-orgs/current>

Finding Trustworthy and Reliable Information on the Internet

There is a lot of information on the Internet. In a way, it can be like trying to get a sip of water from an open fire hydrant. How do you know what to trust? You'll want to find and use only accurate and current information. Reliable health information comes from scientific research that has been conducted in government, university, or private laboratories, and reliable websites will tell you where the information came from and how it has been reviewed.

Information that you find on a website should not replace your doctor's advice. Your doctor is the best person to answer questions about your personal health. If you read something on the web that doesn't agree with what your doctor has told you, ask him or her about it. Use these tips for finding good and reliable information on the Internet:

Is it clear who sponsors the website?

The web address can help you learn about the type of site you are visiting:

- A government agency has .gov in the address.
- An educational institution is indicated by .edu in the address.
- A professional organization such as a scientific or research society and patient advocacy organizations will be identified as .org.
- Commercial sites identified by .com will most often identify the sponsor as a company, such as a pharmaceutical firm.

Is there a way to contact the website sponsor?

Trustworthy web sites provide contact information. Many even have toll-free numbers to call.

Is there an editorial board that reviews the information?

Click on the "About Us" page of a website to see if there is an editorial or medical advisory board that reviews the information before putting it online. Government websites may not have this page.

Is the information current?

New research findings can make a difference in making smart choices regarding your treatment for your rare disease, so you'll want to look carefully to find out when the web page was last updated. Older information isn't useless, however. Many websites provide older articles so readers can get a historical view of the information.

Is your privacy protected?

You want to be as certain as possible that your personal information is not shared with other lists or companies. Take time to identify and read the website's policy - if the website includes something like, "We share information with companies that can provide you with products," that's a sign you information isn't private. Never give out your Social Security number. If you asked for personal information, be sure to find out how the information is being used by contacting the website sponsor by phone, mail, or the "Contact Us" feature on the website.

Could the claims be too good to be true?

Be careful of claims that various remedies will offer dramatic cures or that offer to sell you something to treat your disease. Talk with your healthcare team about all treatment claims, and before buying and trying products you may find on the Internet. Here are two reputable sources of drug and treatment information:

MedlinePlus www.medlineplus.gov

This website offers a wealth of information about health topics, drugs, and supplements. It also provides videos and interactive tutorials. The website is maintained by the National Institutes of Health. It includes a tutorial that teaches you how to evaluate the information you find on the internet.

Drugs@FDA www.FDA.gov

This website, developed by the U.S. Food and Drug Administration (FDA), provides detailed information about all FDA-approved drug products. You can easily search by generic or brand name. To find this site, go to www.fda.gov and search for Drugs@FDA.

Learn About Your Treatment Options

When the time comes to make a decision about treatment, use reliable information—along with input from your healthcare providers, family, friends, and others. Weigh the pros and cons of each treatment. Think about how well it might work, side effects, and costs. Consider how it might affect your lifestyle. Then, work with healthcare providers to develop a care plan you want to do, and feel you can do. Remember, you have the right to choose or refuse treatment.

What is a treatment plan?

A treatment plan is like a road map that a patient will follow on his or her journey through treatment. It outlines the progress of disease treatment or therapy. A treatment plan may be highly formalized in a medical record, or may consist of loosely handwritten notes. No two treatment plans are going to be the same, everyone's journey with rare disease is different. Keep in mind, the treatment plan is always subject to change as medications, therapies, or your rare disease progresses.

Thinking of Participating in a Clinical Trial?

If you have a rare disease, you may want to consider taking part in a clinical trial, also called a research study. Clinical trials are used to test new treatments before they're made available to the public. If the new treatment proves to be better than the current treatment, the new treatment can then be approved for general use by the U.S. Food and Drug Administration (FDA). Be an advocate before you participate -- get your questions answered

Join a clinical trial. Find out if there are any free or low-cost clinical trials to participate in. If trial-related costs are of concern to you, the study doctor or nurse can answer questions about what is covered and what costs you or your insurance will be expected to pay.

The doctors and nurses running the trials will let you know about the study plan.

Make sure to ask about:

- The medicines to be used and the dosage
- How the treatment will be given
- Any lab tests that will be done
- Known side effects of the treatment
- Possible benefits of the treatment

Visit www.clinicaltrials.gov to get up-to-date information about clinical research that is being carried out all over the world for a wide range of diseases and conditions.

You may also consider contacting the National Institutes of Health (NIH). NIH might have a clinical trial that is right for you. If you meet the requirements and are accepted into a clinical trial, they will usually cover medical expenses related to participating in the trial. To find out more, call toll free: (800) 411-1222.

Keep an Eye on Your Care

Healthcare providers are only human—mistakes can happen. As an informed patient, you can keep an eye out to make sure you get the right treatment every time—in the office, at the lab, and at the hospital. Try these ten tips provided by the U.S. Agency for Health Research and Quality:

Ten Tips for Preventing Medical Mistakes

1. Be an active member of your healthcare team. Take part in every decision about your health. Research shows that patients who are more involved get better care.
2. Make sure your doctor knows all the medicines and supplements you take.
3. Make sure your doctor knows about any allergies or adverse reactions you have had to medicines.
4. When your doctor writes you a prescription, read it. If you can't read it, your pharmacist might not be able to either.
5. Ask for information about medicines and treatments in terms you understand—both when your medicines are prescribed and when you receive them.
 - What is this medicine for?
 - How am I supposed to take it?
 - What side effects are likely? What do I do if they occur?
 - Is this medicine safe to take with other medicines or dietary supplements I am taking?
 - What food or drink should I avoid while taking this?
6. When you pick up a medicine or get a treatment at a medical center, ask: Is this the medicine/treatment that my doctor prescribed?
7. Make sure that all the healthcare providers involved in your care have important information about you. Don't assume that everyone knows what they need to know.
8. Ask a family member or friend to be there with you and be an advocate.
9. Know that more is not always better. Ask why a test or treatment is needed and how it can help. You might be better off without it.
10. If you have a test, don't assume that no news is good news. Find out the results.



Need help paying for healthcare provider visits or treatments?

What if your health insurance doesn't cover seeing specialists or certain treatments? Read below to find out where you can get help.

Patient Advocate Foundation www.patientadvocate.org. Call (800) 532-5274 to see if they can help with fees. Ask about their Co-Pay Relief program.

Chronic Disease Fund www.cdfund.org. This non-profit organization provides financial help to seriously ill Americans who have insurance, but cannot afford their medicines. Call (972) 608-7141, or toll-free at (877) 968-7233

Department of Health and Human Services (DHHS) www.hhs.gov.

The Department of Health and Human Services (DHHS) has information on assistance in paying for medical care for low-income families. Go to the "Families & Children" section of the website. Call (202) 619-0257 or toll-free (877) 696-6775.

Insure Kids Now www.insurekidsnow.gov. Insure Kids Now is a resource where you can find free or low cost health insurance for children and teens.

Find a Health Center www.findahealthcenter.hrsa.gov. You can find federally funded free or low cost medical and dental care by using the "HRSA Find a Health Center" tool.

National Organization for Rare Disorders (NORD) www.rarediseases.org

The NORD website includes information on medication assistance programs and networking programs, a resource guide and links to other online resources. Contact NORD directly at (800) 999-6673 (voicemail only) or (203) 744-0100 or by email, orphan@rarediseases.org.

RxAssist www.rxassist.org has a complete database of patient assistance programs, as well as practical tools, news, and articles.

Search State Insurance and Co-Pay Assistance Programs.

Go to the Families USA Program Locator at www.familiesusa.org/resources/program-locator for listings of health insurance assistance programs by state. These programs handle many questions about Medicare, Medicaid, and private insurance and may be able to refer you to low-cost or free healthcare (which may include prescription drug assistance).



Gather and Organize

Once you've gathered all the information you can on treatment options, read it and organize it. Then share what you've learned with healthcare providers, family, and friends.



Summary

Take the time to learn about your disease and your treatment options. Remember, knowledge is a powerful part of self-advocacy. The more you know, the better you will be able to make informed choices about your care.

As you gain health advocacy skills, you may find it helpful to take this patient guide with you to your doctor visits. Some patients find reading the questions from the list helps them stay on track as they seek answers from their healthcare providers.

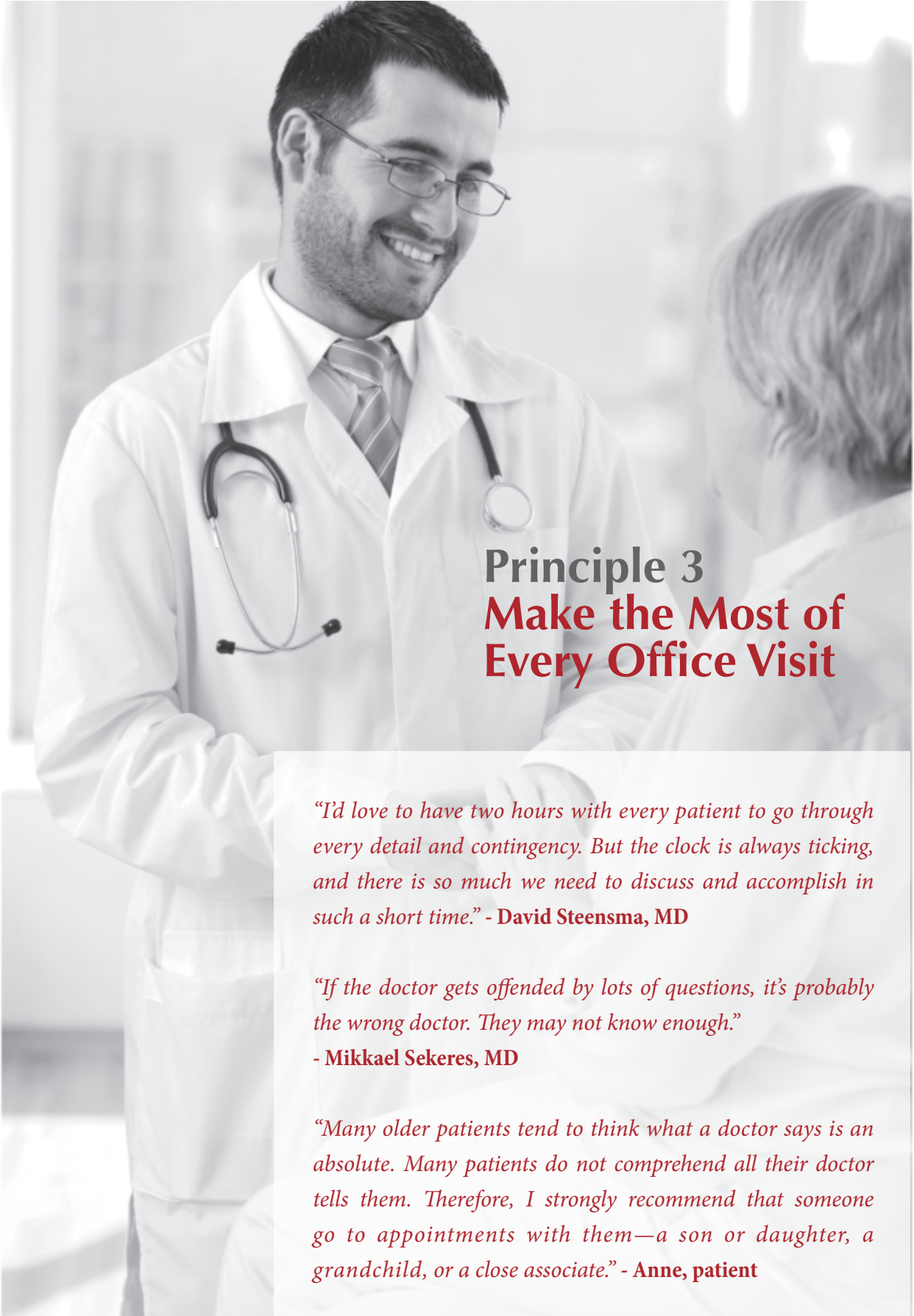


Skills Checklist

Use this checklist to assess your skills in learning about your disease and available treatment options:

- I have sought up-to-date information about my disease by talking to specialists and doing my own research.
- I seek information only from reliable sources on the Internet.
- I ask my healthcare team about treatment options.
- I know whom to contact for help paying for medicines or treatment.
- I feel able to take the ten steps to help prevent medical mistakes.





Principle 3 Make the Most of Every Office Visit

“I’d love to have two hours with every patient to go through every detail and contingency. But the clock is always ticking, and there is so much we need to discuss and accomplish in such a short time.” - David Steensma, MD

*“If the doctor gets offended by lots of questions, it’s probably the wrong doctor. They may not know enough.”
- Mikkael Sekeres, MD*

“Many older patients tend to think what a doctor says is an absolute. Many patients do not comprehend all their doctor tells them. Therefore, I strongly recommend that someone go to appointments with them—a son or daughter, a grandchild, or a close associate.” - Anne, patient

Principle 3 **Make the Most of Every Office Visit**



Prepare for Each Visit

Office visits may be shorter than you hoped. To be a strong advocate for yourself, it is critical that you do everything you can to make the most of every visit. You'll want to do some work in advance.

Set an Agenda For the Visit

Ask yourself, "What do I want to get out of this visit?" Write down the top three things you want to discuss. Focusing on your agenda will help you make the most of your time.

Ask For the Time You Need

When you make an appointment, let the staff know if you have special concerns that might require a little extra time with your doctor. If, after your appointment, you still need more time, find out how you can call or email your doctor with concerns.

Do Your Homework

Learn all you can about your condition and the available treatments.

Bring Information With You

Bring your medical information to the visit. This information should include:

- A list of questions in priority order (see page 22 for questions to consider)
- All current and past illnesses, as well as any chronic conditions you may have
- A list of prescription and over-the-counter medicines, vitamins, herbs, and supplements you have used
- Notes on symptoms and side effects you are having
- Test results

Form a Good Relationship With Your Healthcare Team

Establishing friendly relationships and showing appreciation for your doctor and the staff is important. When you work well with your healthcare providers, it enables them to focus on your condition and to make the best use of the time they have with you.

Bring a Member Of Your Personal Support Team

Going to the doctor can be overwhelming. Asking a family member or friend to come along can help. This person can help you ask questions, write down answers, and ensure you get answers you understand because four ears are always better than two. A family member or friend can also provide emotional support. For more on building a strong personal support team, see pg 45.

Ask Questions, Get Answers

Clear communication is an integral part of good healthcare. In order to make smart choices about treatment and follow your care plan correctly, you need to fully understand your disease and treatment options. These are the things that empowered patients do to make sure they get the best care and treatment.

Ask the Most Important Questions First

Be sure to bring a list of questions and put them in priority order. You may have just a few minutes to ask your questions, so be sure to ask the most important ones first. For a list of disease and treatment related questions to consider, see “Questions to Ask Your Healthcare Team” on page 22.



Ask for Clarification

Don't worry about offending your healthcare provider. Healthcare providers tend to share more information with patients who pay attention and ask questions. Asking questions shows healthcare providers that you are actively involved in your own care and want to stay informed. Let your doctor know if the information is confusing or if there is anything you don't understand.

If you have trouble understanding your healthcare provider

- Ask the healthcare provider to say things again or use simpler words until you understand it.
- Ask a family member or friend to listen with you.
- Ask for brochures, drawings, or models.
- Ask the provider to write down answers.

Take Notes

After the visit is over, it may be hard to recall exactly what your provider said. Take notes, ask a family member or friend to take notes, or use an audio recorder. Keep these notes with your medical information.



Remember:

- Your healthcare provider is your paid consultant and part of the team you need to organize to obtain the best care possible.
- Be respectful, but do not be intimidated.
- Always consult with your doctor prior to making any medical care changes.
- You are always entitled to a second opinion.

Summary

Have you ever left your doctor's office not sure of what you need to do next? Not quite sure how to take your medicine? You're not alone. There is a lot of information to take in at each visit. Using the steps outlined in this section can enable you to get the most from your office visits. While not always easy, asking questions until you get answers you understand is a central part of becoming an empowered patient.



Skills Checklist

Use this checklist to assess your skills in preparing for office visits and making sure you get your questions answered in a way you can understand:

- I take steps to prepare for each visit by writing a list of questions beforehand and bringing them along with me.
 - I ask my healthcare team questions.
 - I make sure to get answers I understand.
-



Principle 4 **Keep Careful Track of Health Information**



“Since I take copies of my lab work with me when I travel, I always make photocopies to leave at home in case I lose this important paperwork. I would suggest making a couple of photocopies of all important medical papers. In my view, being able to put my hands on my health records at a moment’s notice is a critical part of self-advocacy.”

- Harvey, patient

Principle 4 **Keep Careful Track of Health Information**



An Organized Patient is a Powerful Patient

Keeping all your medical information organized and in one place makes it easier to track changes in your health and share information with your healthcare provider. Read this section to learn how to keep track of your health information.

Gather Information

Collect information about your health, office visits, and treatment in one place. Don't feel that you must gather all of your health information at once. The next time you visit the doctor, simply ask for recent records, and do so each time you visit a healthcare provider. Here are some of the items you'll want to hold onto:

Health Information

- Allergies
- Immunizations you have had
- Notes on symptoms
- Personal health history and family health history

Office Visit Information

- Dates of office visits
- Notes on what providers tell you
- Questions to ask healthcare providers

Treatment Information

- List of medicines and other treatments, vitamins, herbs, and supplements you have used
- Notes on side effects
- Treatment instructions

Financial and Legal Information

Insurance claims—paid and unpaid (You can appeal claims that are denied by your insurance company. Call your insurance company for steps to take to appeal.)

Contact Information

- Family and friends
- Other patients
- Pharmacy—note when they are open
- Healthcare providers—note when they have office hours and how to reach them after hours during an emergency
- Insurance—note when they are available to take calls from members

Ask for Copies of Health Records at Each Visit

It can be difficult to get your health records weeks or months after a doctor's visit. Different states have different rules. Sometimes, you need to complete an application, and sometimes, there is a fee. It is a good idea to ask for key health records at the end of each visit—ask for test results and transfusion records.

A federal law, the HIPAA Privacy Rule (available at www.hhs.gov/ocr/privacy/index.html), gives you the right to see, get a copy of, and correct your medical records. Most states also have laws that give you access to your records.

Take Notes

Keep a log of changes in how you feel. Some of these changes may be symptoms of your disease. Others may be side effects of treatment. This is key information that only you can provide.

Keep a running list of questions to ask providers. And record your healthcare providers' answers. Include what they say over the phone and via email, as well as during office visits.



Find an Organization System that Works for You

Now that you have all this information, you need to figure out the best way for you to keep it organized and in one place. Here are some tools other patients have found useful:

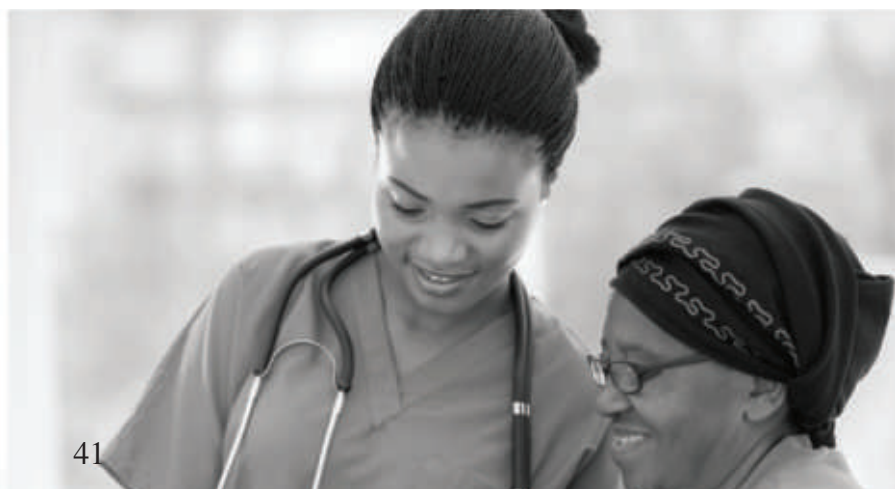
- 3-ring binder with dividers
- Accordion folder
- Manila file folders
- Notebook or journal
- Electronic files and spreadsheets on a laptop or desktop computer, tablet, flashdrive, or smartphone
- Online tools such as a personal electronic health record.

Remember—there's no right way to compile or organize. Talk to other patients to learn what they do to keep their health information organized.

Consider Creating Your Own Complete Personal Health Record (PHR)

If you don't have one, now may be the time to create a complete personal health record, or PHR for short. The PHR is a tool that you can use to collect, track, and share all your past and current health information. A complete PHR can help you share your health history with healthcare providers, avoid unneeded tests and procedures, and lower the chance of medical errors. To start your personal health record, you will need to request a copy of your health records from all of your healthcare providers, including your primary care doctor, eye doctor, dentist, and any other specialists you have seen.

Remember, you're in charge of making choices about your health. A PHR can help you do that. For detailed instructions on how to create your own PHR, go to www.myPHR.com. This website is a service of the American Health Information Management Association (AHIMA).



Share Information

Share your medical information with your healthcare providers, family, and friends. It will help them stay in the loop—so they can assist and support you better.

Take Medical Records With You When Traveling

Are you going on a long trip? Be sure to take your medical information with you. You never know when you might need it. It is also a good idea to have a current medication list in your wallet or purse in case of emergency. Just make sure to make photocopies and leave a set at home in case you lose any of this information.

If you are going on a trip, you may want to locate hospitals, pharmacies, and other medical facilities near your destination in advance. You can include this information in your healthcare files.

Summary

Keeping track of your medical records and health information can be quite a task. Yet being able to put your hands on health information you need with a moment's notice can be very helpful as you advocate for your health. While it may not come naturally to you to ask for your health records, gaining this assertiveness skill can help you feel in control of your health.

Feeling overwhelmed by paperwork? Read Principle 5 on page 45 to see how your personal support team may be able to help out.

Skills Checklist



Use this checklist to assess your skills in organizing key health information:

- I use a system for organizing my health information.
 - I can put my hands on health information when I need it.
 - I ask for key health records at the end of each visit.
 - I take my health information with me when I travel and make sure I have a second copy at home.
 -
-



Principle 5 **Build a Strong Personal Support Team**



“I encourage my wife to stay active—even when she’s feeling tired. I remind her to take a nap. I try to be helpful and supportive and ask how I can help her.”

- Roger, husband of patient

“Joining support groups has been helpful for me because I’ve been able to hear new information. Plus, I’ve been able to share with people who have been recently diagnosed.”

- Jill, patient

Principle 5 Build a Strong Personal Support Team



You Don't Have To Do It Alone!

Having a rare disease can be a heavy burden. But you don't have to carry it alone. There are people in your life—and people you don't know yet—who would be glad to help you cope with the physical and emotional demands of your disease. With their support, you can become a strong self-advocate.

Get Support from Family and Friends

Family and friends can be a tremendous source of strength. They can help you with daily tasks and give you emotional support. It can be hard to accept the fact that you need support—but it is important for your health. Try these tips:

Make a list of tasks

Keep a list of tasks you could use help with. For instance, you might ask family and friends to:

- Cook, clean, and shop for you
- Give you rides
- Go to healthcare provider and hospital visits with you or look after your children
- Organize your medical information
- Research your disease and treatment options
- Talk with you or listen while you talk

Ask for help

Family and friends often want to support you, yet they may not know how. Feel free to tell them what you need. Having trouble? Try these conversation starters:

- “I could use a hand with ...”
- “It would be really helpful if you would ...”
- “I'm not feeling very well today. Would you mind ...”
- “I sure could use some help with ...”
- “I'd really appreciate it if you ...”

Keep them in the loop

Family and friends want to know how you're doing. But answering their questions can be tiring. Think about asking one family member or friend to give updates on your behalf. Or post updates on a health social networking website like www.caringbridge.org or www.patientslikeme.com.

Know your limits

Having a rare disease takes a lot out of you. Know your limits, and feel free to say “no” to outings and activities planned by family and friends.



Get Support from a Health Advocate

“My older sister and I have always been very close. She lives in an assisted living home near me. She has a rare disease and was always an excellent advocate for herself until recently. Lately, Janine is feeling overwhelmed. I see how hard it is to cope with a chronic health condition, so I asked Janine if I could go with her to visits and be in contact with her doctor and nurses. She actually seemed relieved to have someone help her through this difficult treatment round.

I heard it varies from state to state, but I became Janine’s official patient advocate (PA). I had to sign a state-approved form to accept this role. Being her PA allows me to review medical changes in any type of healthcare facility.

I know my sister will be back in the swing of things once she is feeling better. For now, I think it works well for both of us that I am taking a role to help her out.”

- Robert, brother of patient

A health advocate is a family member, friend, trusted coworker, or paid professional who can help you advocate for your best care. This person can go with you to your doctor visits, ask questions, and write down information. You may also want your health advocate to speak up for you when needed.

Ask a Family Member or Friend To Be Your Health Advocate

A friend or family member may be able to serve as a patient advocate. You may want to ask a few people to share that role. Each person can take on different tasks. One person may be better able to assist you with a doctor's visit, while another might be able to assist you with insurance issues.

Or choose a professional patient advocate

The Patient Advocate Foundation has professional case managers who provide a variety of services to help patients to settle issues with access to care, medical debt, and job retention related to their illness. Their services may be free. To learn more, call them at (800) 532-5274. Or, go to www.patientadvocate.org. Also, many hospital, community health centers, and long-term care facilities have professional patient advocates on staff. They go by different titles. Some of the more common titles include:

- Care manager or case manager
- Health advocate
- Healthcare or nurse navigator or health advisor
- Patient advocate, patient advisor, patient educator, or patient navigator
- Social worker



Why Should You Use a Health Advocate?

“Research shows that quality healthcare means taking an active role in decisions about your care. If you’re facing a difficult medical decision, it’s a good idea to bring someone with you who can help you take an active role in your care when you’re not fully up to it.

As a doctor and a patient, I’ve seen how valuable it is to have ‘another set of ears and eyes’ in the exam room. Having an advocate at medical appointments or during a hospital stay can ensure that you get the information you need to manage your health. ...

Who makes a good health advocate? Someone who is calm, pays attention to details, and can ask questions and state information clearly.”

Carolyn M. Clancy, MD
Former Director of the U.S. Health and Human Services
Agency for Healthcare Research and Quality/www.ahrq.gov/consumer

Health advocates can:

- Ask questions or voice concerns to your doctor for you.
- Ask the “what’s next” questions, such as: “If this test is negative, what does it mean? If it’s positive, will more tests be needed?”
- Compile or update your medicine list.
- File paperwork or assist with insurance matters.
- Help arrange rides.
- Help you follow treatment instructions, including asking questions about your follow-up care.
- Remember your medicine schedule.
- Research treatment options, procedures, doctors, and hospitals.

Get Support From Other Patients

More than anyone else, people living with your rare disease know what you’re going through. They can let you know you’re not alone. Here are some ways to connect with them:

- Join a support group. If you can’t find one specific to your disease, join a related one that addresses a similar health topic.
- Look for groups of patients with your disease on social media sites such as Facebook.

Get Support From Your Healthcare Team

You may be able to get emotional support from a member of your healthcare team. This healthcare provider may be a counselor or social worker. Or it may be a nurse or doctor who goes above and beyond their professional duties.

Get Support From Non-Profit and Patient Advocacy Organizations

Visit the patient advocacy organizations identified in Principle 2 (pg 23) and the non-profit organizations (pg 56) for additional sources of support.

Stand Up for Your Health—Today!

Standing up for your health is a process. It's something that happens over time, as you learn more about your disease and gain confidence. Refer to this patient guide when you want to remind yourself of the core skills needed to become an empowered patient. We hope it will help you take steps to become a stronger self-advocate.

Summary

For many of us, asking for help is not easy. Yet, empowered patients know that help from others can help them get the healthcare services and treatment they need. Some of us have family members or close friends who live nearby and are willing and able to serve as part of our support team. Others choose a professional patient advocate. And some of us find joining online groups or forums offers the level of support we need.

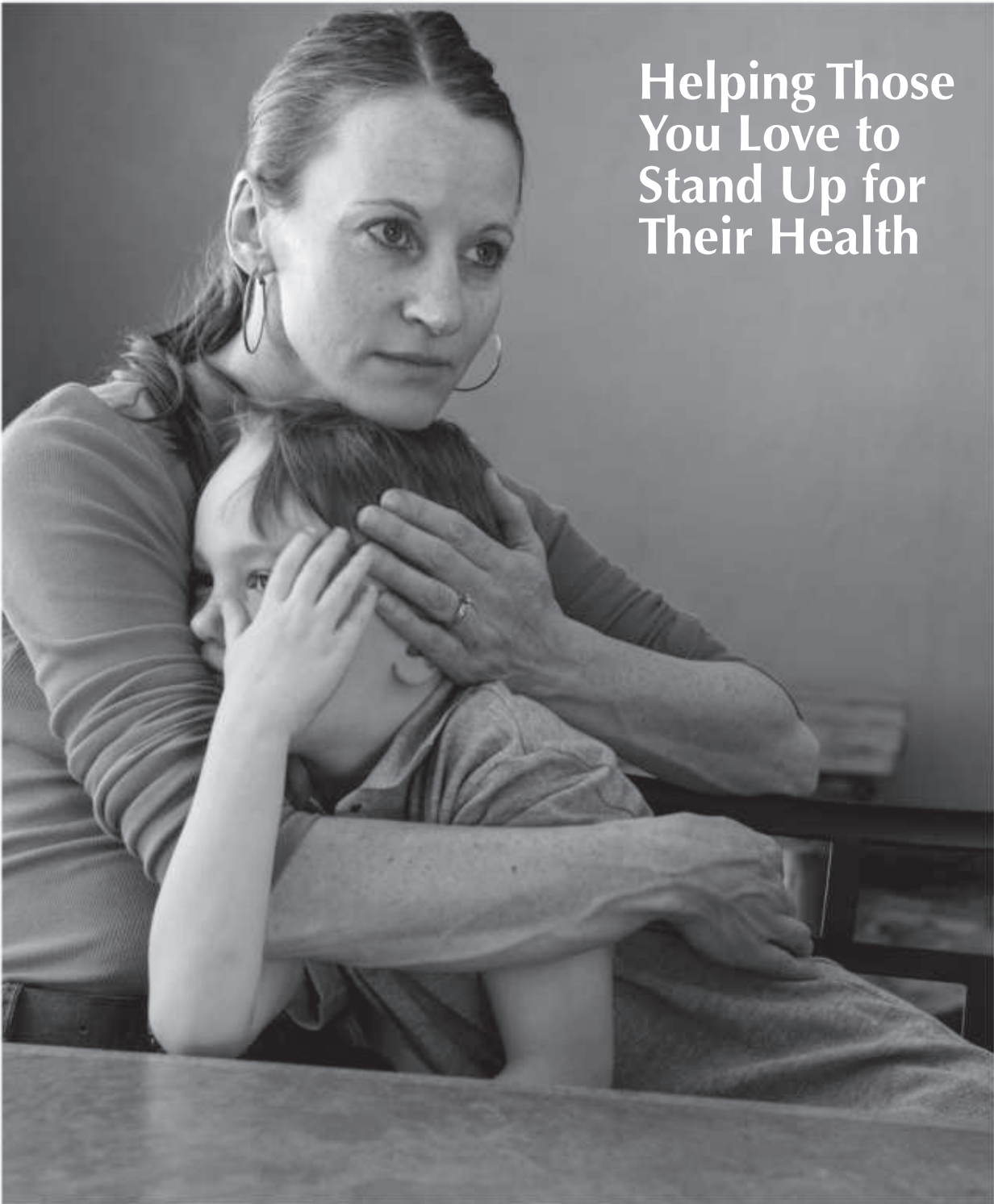
Having a chronic and rare disease can be very difficult. Knowing that someone is in your corner can help you get through the ups and downs.



Skills Checklist

Use this checklist to assess your skills in building a strong personal support team:

- I ask friends, family, or trusted coworkers to help out when needed.
 - I get support by connecting with other patients.
 - My healthcare team offers support.
 - I get support from a professional advocate.
-



Helping Those
You Love to
Stand Up for
Their Health

Helping Those You Love to Stand Up for Their Health



Having a child, teenager, or parent with a rare disease presents the same unique challenges for those who love and care for them, as it can for adult patients with rare diseases. The first five principles described in this guide all apply to caregivers who are managing the care of a loved one. We have added some specific suggestions to empower parents and adult children of patients with rare diseases.

Standing Up for Your Child's Health

There is nothing more devastating to a parent than to see your child suffer from a serious disease. For every parent who has heard these words, “Your child has a rare disease,” it is a moment frozen in time and a life changing event. Whether you’ve just learned your child has a rare disease, or you have been managing your child’s disease for many years, this section provides tips, information, and resources that can help.

Is Your Child Recently Diagnosed?

If your child was recently diagnosed, it is likely your days have suddenly become filled with medical tests, doctor visits, and, no doubt, worrying. This process can be confusing and overwhelming. You might feel depressed or even angry, and ask, “Why did this happen to us?” While those feelings are normal, it’s important to know that you’re not alone. The first five principles in this guide can all be used to help you be a strong parent advocate to stand up for your child’s health, to ensure that they, and you, get the support you need.

Supporting Students With Rare Diseases

Sending your child back to school after treatment for any disease can be scary and overwhelming. Speak with your child’s teacher, doctors, and school nurse. Explain any limitations or precautions that might be necessary to have your child safely return to his or her classroom.

Take Care of Yourself

As any parent of a sick child knows, this is a very real dilemma. On the one hand, your focus needs to be on your child and helping him or her get healthy. On the other hand, your child needs you to be healthy, strong, and in control. And you can only be this kind of model for your child if you take care of yourself by eating right, exercising, and getting plenty of sleep. If you feel you don’t have time to do this, eliminate other things from your life and make the time. Your mental, physical, and spiritual health is critical to your child’s health.

Find Time to Have Fun

Your child's illness is serious. Sometimes, you may feel like every second of your life revolves around medication schedules and trips to the doctor. That's why it's vital for you to find relief by not taking every moment of the day too seriously. Make time to do things you enjoy – even if you need to sandwich fun times between appointments. This could be as simple as taking a walk, playing cards with a friend, or doing a craft project or going to the park. Take a minute now to make a list of five things you have fun doing – and commit to doing one this week. You will find you feel more refreshed when you do.

Adolescents Transitioning to Adult Care

As parents, we do our best to prepare our children for the adult world. We teach them right from wrong, social skills, how to do their laundry and how to drive a car, but do we prepare them to transition from pediatric medical care to adult medical care?

“Transition” in the patient care field is defined as the process of moving from pediatric health care to adult health care. The process can often present challenges for youth with a rare disease and their families. The transition generally begins once your child is a teenager, but don't wait to get your child involved. The sooner your child learns about their illness, the better prepared they are to begin taking charge and stand up for their health.

How do parents begin the process of preparing their children to assume responsibility for their own medical care and make the best choices? Below are some suggestions on how to begin the process.

1. Identify a transition team. These are the key people who will help your child with transitioning from pediatric care to adult care
2. Establish a transition plan to identify your child's specific needs.
3. Create a transition record, including a notebook summarizing medical information, including the patient's medical insurance and history of hospitalizations, procedures, and tests.
4. Complete this skills assessment check list. This will help you and your child identify their strengths and in what areas they need assistance.
5. Seek recommendations from your child's pediatrician for adult care physicians. Schedule an appointment to meet the physicians so you can help your child in the selection process.

Pediatric to adult healthcare transition can be a scary process for parents, especially when your child has a chronic illness. Ask your child's doctor about resources that can help with making the move from pediatric care to adult care successful.

Standing Up for Your Parent's Health

Standing up for your health is important for every patient with a rare disease. It becomes more so, and more complicated as parents age. They may have other illnesses or disabilities that must also be considered when being a strong advocate for their health. You and/or your parent may need to be more proactive with his or her healthcare providers.

These first five principles to standing up for your health may be new to your parent and might take some coaching or assistance from you. The goal is to empower your parent to stand up for their health. That includes encouraging them to do all that they are capable of doing. If you want them to be more engaged in their healthcare decisions, it is important that you not assume the role of primary advocate for them unless it is clear that they cannot adequately stand up for themselves. Many adult children step in prematurely to “help” their parent without first assessing the parent's true capabilities.

How you answer the questions below will help determine to what degree, if any, you should partner with your parent as their health advocate. Regardless of whether your parent is a strong self-advocate or does so with your assistance, the five principles described in this guide suggest practical ways to ensure that your parent gets the quality healthcare he or she deserves.

Here are several important questions you can ask yourself to better assess whether your parent needs for you to be his or her health advocate, if they just need a little support to be able to stand up for their own health:

- What is your parent's overall health status? Are there vision or hearing impairments that would make communication with healthcare providers difficult or confusing? Does your parent exhibit any cognitive difficulties such as memory loss or confusion?
- Is your parent expressing a desire for help?
- Is she or he easily overwhelmed by details about her or his rare condition or other health issues?
- Does she or he typically ask pointed questions of healthcare providers to get the necessary information? Is the information she or he provides about her symptoms, medical history, or other details of her or his rare disease and overall health accurate and thorough?

Standing Up for Your Health: Online Resources for Empowering Patients With Rare Diseases



Standing Up for Your Health: Online Resources for Empowering Patients With Rare Diseases



Use the information and organizations in this resource list as you seek information to help you become an empowered patient.

Federal Government Website Resources

Agency for Healthcare Research and Quality (AHRQ)

www.ahrq.gov (301) 427-1104

The Agency for Healthcare Research and Quality's (AHRQ) mission is to produce evidence to make health care safer, higher quality, more accessible, equitable, and affordable, and to work within the U.S. Department of Health and Human Services and with other partners to make sure that the evidence is understood and used. This branch offers resources for healthcare consumers at www.ahrq.gov/consumer.

Clinical Trials www.clinicaltrials.gov

This website provides up-to-date information about clinical research that is being carried out all over the world for a wide range of diseases and conditions. It tells the purpose of each trial, who may participate, locations, and contact numbers. It also describes the clinical trial process and reports recent results.

Food and Drug Administration (FDA) www.fda.gov (888) 868-4000

The FDA is the federal agency in charge of regulating drugs, medical devices, and biologics. This website contains information on drugs, blood products, recalls, and safety alerts. The FDA also has a website called Drugs@FDA. This site provides detailed information about all FDA-approved drug products. You can easily search by generic or brand name. To find this site, go to www.fda.gov and search for Drugs@FDA.

Healthfinder www.healthfinder.gov

Healthfinder offers links to hundreds of sites that provide reliable healthcare information and support to consumers.

Medline Plus www.medlineplus.gov

This website offers a wealth of information about health topics, drugs, and supplements. It also provides videos and interactive tutorials.

National Institutes of Health (NIH) www.nih.gov

The National Institutes of Health (NIH), a part of the U.S. Department of Health and Human Services, is the nation's medical research agency—making important discoveries that improve health and save lives. The NIH is the research arm of the U.S. Department of Health and Human Services.

PubMed www.pubmed.gov

PubMed comprises more than 24 million citations for biomedical literature from MEDLINE, life science journals, and online books. Citations may include links to full-text content from PubMed Central and publisher web sites.

Non Profit Websites

Caring Bridge www.caringbridge.org

CaringBridge website allows people to easily get updates and offer support and encouragement.

Families USA Program Locator www.familiesusa.org/resources/program-locator

This website includes listings of health insurance assistance programs by state. These programs handle many questions about Medicare, Medicaid, and private insurance and may be able to refer you to low-cost or free healthcare (which may include prescription drug assistance).

My Personal Health Record www.myPHR.com

This website is a complete resource for anyone interested in creating their own personal health record (PHR). It describes the benefits of creating a PHR, includes tools and tips for creating a PHR, and provides stories from patients about the benefits of using a PHR. Created by the American Health Information Management Association (AHIMA).

National Organization for Rare Disorders (NORD) www.rarediseases.org

The National Organization for Rare Disorders (NORD) provides advocacy, education and other services to improve the lives of all people affected by rare diseases. NORD maintains a patient organization database to help search for organizations that help people with rare diseases.

Orphanet www.orphanet.net

Orphanet is the reference portal for information on rare diseases and orphan drugs, for all audiences. Orphanet's aim is to help improve the diagnosis, care and treatment of patients with rare diseases.

Patient Advocate Foundation www.patientadvocate.org (800) 532-5274

This group can help you find professional case managers who provide a variety of services to help patients to settle issues with access to care, medical debt, and job retention related to their illness. This group also provides resources for how to be your own advocate.

RareConnect www.rareconnect.org

RareConnect was created by EURORDIS (European Rare Disease Organisation) and NORD (National Organization for Rare Disorders) to provide a safe space where individuals and families affected by rare diseases can connect with each other, share vital experiences, and find helpful information and resources.

RxAssist www.rxassist.org

This website has a complete database of patient assistance programs run by drug makers. These programs can provide free or low cost drugs to patients that qualify. They may also be able to help you work with your insurance company to get your drug covered. The web site also has practical tools, news, and articles.



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STANDING UP 
for your health
SELF-ADVOCACY FOR PATIENTS WITH RARE DISEASES

