

Pompe Disease: Engaging Patients in Shared Decision-making



What Is Shared Decision-making?

SDM is a key component of patient-centered care and is a process in which patients with LOPD and healthcare providers work together to decide what is best for the health of the patient. Optimal healthcare decision-making occurs when evidence-based information about available management options, provider knowledge and experience, and patient values, goals, and preferences are considered. By engaging in the SDM process, patients with LOPD, their families, and their caregivers are more likely to identify management options that will work best for all those involved.

The SHARE Approach

The Agency for Healthcare Research and Quality endorses a 5-step process known as the SHARE Approach that can be used to engage patients with LOPD in SDM. Use the QR code below to learn more about the SHARE Approach and access SHARE Approach resources, including webinars and a toolkit.

5 Essential Steps of Shared Decision Making

- 1 SEEK** your patient's participation
- 2 HELP** your patient explore & compare treatment options
- 3 ASSESS** your patient's values & preferences
- 4 REACH** a decision with your patient
- 5 EVALUATE** your patient's decision

Scan the QR code for more resources
including webinars and a toolkit!



Real-World Examples of SDM with LOPD Management



PATIENT 1

Doctor: You have been on standard ERT for 16 years now. Compared with the assessments made when you started taking ERT, now you are less able to walk great distances and you have more difficulty rising from and sitting down in a chair. Overall, your ability to function independently is declining along with your lung function. We've spoken about clinical trials with new treatments in the past. These agents are now approved. How do you feel about trying a new ERT?

Patient: Let's try this new therapy doctor, as I could not [join] any clinical trial!

3 Months After Initiation of Second-Generation ERT

Doctor: How are you feeling? Tell me about the types of activities you have been doing.

Patient: All fine, doctor. This gave me back some energy. My fatigue is better. I try to walk more, and I have lost weight! AND I can do the infusion now at my home!

BE SURE TO ASK...

Have I explained the differences with the available therapies in a way that is understandable to you?

This is a great question to ask patients and caregivers to ensure that you have effectively communicated with them and that they can make informed decisions.

PATIENT 2

Doctor: Let's review your ERT. We started you on a standard-of-care dose of alglucosidase alfa. There was an initial benefit followed by a period of plateau and a subsequent clinical decline. You have been receiving ERT infusions every 2 weeks for the last 4 years. Have you been able to leave the house and meet your friends? Have you been able to volunteer lately? I know you like to read. How much time have you spent reading this week?

Patient: My energy is declining, and I am concerned about what I can safely do. I spend most of my time watching TV. I've done some reading, but I just don't have the energy to get up and do the things I want to do. I am not volunteering anymore because it requires too much effort on my part.

Doctor: There are recently approved treatments for Pompe disease that I want to talk with you about. They are better able to deliver the enzyme you need to your skeletal muscles. This may help to stabilize your disease.

12 Months After Initiation of Second-Generation ERT

Doctor: It is good to see you. What activities have you been enjoying lately?

Patient: I'm now almost leading a normal life. I am going to church again. This past week we just had a Christmas party in my house with people [from] Bible study. I [go out to] breakfast sometimes with [friends] roughly every week or so. I've been doing things to reach out and use my energy. I am looking for things to keep me active at home. I can dress myself, shave, and shower. Everything takes longer than it used to, but I have a new ray of hope about what the future can bring; how long I can be productive.

Doctor: That is wonderful to hear. Let's do some testing, too, so we can measure how well you are doing.

Abbreviations: ERT: enzyme replacement therapy; LOPD: late-onset Pompe disease