



Strong Connections

When it comes to Pompe disease, every person has a different story to tell, but there is a shared understanding that requires no words to explain.

There is also great strength — and unity — when people who share a common bond come together, even though they may live worlds apart.

This is the essence of **Pompe InCommon**.

Pompe InCommon is a new and exciting initiative from Genzyme that will bring you useful information and insightful programs that are designed to help you every day. First up is this publication, which is all about becoming a strong advocate for yourself — or for someone you care for.

And much more is coming!



Send a picture of you with your balloon to share with the **Pompe InCommon** community

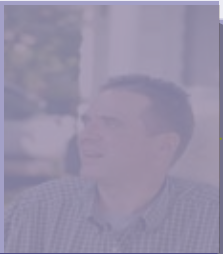
Along with this publication, you received a globe balloon that symbolizes how **Pompe InCommon** connects diverse stories, experiences, and insights from people who are living with Pompe. We will feature pictures of community members holding their **Pompe InCommon** balloons in upcoming publications

(if you have respiratory challenges, ask a family member or friend to help you blow up your balloon). To participate, you can upload your photo at www.pompe.com/incommon or send your photo via mail to:

Pompe InCommon
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You are your best advocate

"If you want to get anything done, you have to advocate for yourself," says Mark L., who has been living with Pompe since 1996. From financial assistance to medical assistance, Mark doesn't back down easily, even when people tell him "no." When asking people for any type of assistance, Mark suggests, "You have to explain to them why seeing things the way that you see them would be a benefit to everyone involved."



Advocating is really about taking care of yourself — or your loved one. "If you won't fight for yourself, other people are not as inclined — even though they are of a good nature — to fight for someone who's not willing to fight for themselves," adds Mark.

"To be empowered, you must have access to the information you need to make informed decisions, about your health or anything else. Informed patients and caregivers are the best self-advocates," says Sharon F. Terry, president and chief executive officer of Genetic Alliance.

Here are some tips to help you:

Learn as much as you can about Pompe.

A great place to start is by visiting www.pompe.com (click on the "Patients & Caregivers" link in the upper right corner) or by contacting a Pompe patient organization (several are listed on page 7).

Know your team players. Your — or your loved one's — medical team likely includes several different specialists. Understanding the roles these health care professionals play in the care of Pompe may help you have more

productive conversations. “The Health Care Team” page at www.pompe.com (<http://www.pompe.com/en/patients/managing-pompe/comprehensive-care/health-care-team.aspx>) can help you get started. Keep an updated list of the members of your medical team — including key staff at doctors’ offices — so family members can quickly access this information to assist you in case of an emergency. This contact information is an important part of your medical record.

Think ahead. It’s easy to feel nervous when talking to health care providers or people in positions of authority. Write down questions ahead of time so you don’t forget what’s most important to you. An example might include: “Is there anything I (or my loved one) can do to keep muscles strong?”

Take notes. Bring a notebook to each doctor visit to keep track of what’s discussed. If writing is too difficult, ask if you can bring a tape recorder. Having a family member or trusted friend as an “extra set of ears” can also help. Dating your notes can help you assess your health over time. You can also ask a family member or friend to take pictures that you can keep in your notebook to visually chart your progress over time (or use a video camera).

Stay organized. An expanding folder is great for storing important contacts, notes from phone calls, upcoming appointments, medical reports, insurance statements, etc. You can bring it with you to doctor visits so that everything is at your fingertips.

Read the fine print. Knowing what is — or isn’t — covered under insurance plans may save time and frustration. Highlight areas you don’t understand and bring your questions to a Genzyme Case Manager. Genzyme Case Managers have great expertise in this area. Another resource is the **US Department of Health & Human Services** website (www.healthcare.gov), which provides information for individuals and families with children. It also has a section about understanding the Patient Protection and Affordable Health Care Act (PPAA), the new law that makes several changes to the US health insurance system (most of which will take effect between now and 2014).

Express yourself. If there’s something you don’t understand, you have a right to ask questions and express your concerns. If this feels uncomfortable for you, it may get easier with practice. “If Doctor A is not giving you any response, there’s always Doctor B,” adds Mark. If you prefer not to question doctors or insurance companies, you can ask a family member or friend to call these offices on your behalf as your legal designate.

Seek support. No one understands your situation better than someone who’s already been through it. Contact a Genzyme Case Manager, or a Pompe patient organization, to help put you in touch with another person living with Pompe who may be able to share their personal experiences, including how they have approached different issues.

Hello, can you help me?



When getting information over the telephone, always ask for the person’s first and last name at the beginning of the call. Be sure to note the date and time of the call in your records. State your name and why you’re calling (“Hi my name is...and I have a question about my insurance policy”). Make your requests for action brief and clear (“Can you give me an answer before my doctor visit next week?”). If a person cannot help you right away, ask when they can get back to you and jot down a date if a follow-up call is needed. Remaining calm and courteous may make the person on the other end of the line more willing to help you, and saying “thank you” is always appreciated.

Genzyme Case Managers are available to help you by working closely with your physician and your insurance company. Upon request, they can make important telephone calls along with you. Call 1-800-745-4447, Option 3, Monday-Friday, 8AM-6PM EST.

Even though Krystal H.'s daughter attends her early childhood school only three days a week, Krystal is well aware of the challenges that parents face with school systems. "Some of her teachers do understand what Pompe is, just from me telling them, I guess. And some of them know what they are supposed to do with Haley, but they still might not understand the whole background."

The first step for advocating for your child at school is to arm yourself with the right information. But where do you start? "When you have a child with special needs, all of the general information goes out the window. You need your own specialized information and information sources," says Sarah Escowitz, Information Coordinator for the **National Dissemination Center for Children with Disabilities (NICHCY)**, a clearinghouse of information that is funded by the Office of Special Education Programs of the US Department of Education. "A disability organization, whether it be specifically for Pompe, or for a broader range of diseases, is a very important resource," she adds.

One resource Escowitz points to is **parent training and information (PTI) centers**, which are located in most states (you can find them on the NICHCY website at www.nichcy.org/Pages/StateSpecificInfo.aspx). The Parent Training and Information Project is a US Department of Education program that ensures parents of children with disabilities receive training and information to help improve results for their children. Other resources Escowitz notes are **Parent to Parent USA** (www.p2pusa.org), a nonprofit organization that provides parent-to-parent support programs, and **Family Voices**, a national organization for children with special needs. Family Voices has a section on their website (www.familyvoices.org) titled "Advocating for your child."

"These organizations give families information about educational issues, support groups, and other types of resources that might be available in the community," adds Escowitz. Information changes quickly, and she advises staying in touch with these organizations regularly over time.

Other points to keep in mind:

You're a great knowledge source. It is very likely that your child's teachers, and the school nurse, may not be familiar with Pompe. Try to be patient with them as they learn along

Helping your child get the most out of school



with you. You may also need to decide how much — or how little — you want teachers to share about your child's situation with other children in the class. This is a personal decision, but the school psychologist may be able to provide guidance on the best approach. The **Muscular Dystrophy Association (MDA)** website offers information on this subject for teachers that may help guide you (www.mda.org/publications/tchrdmd/what_should_i_say.html). It's a topic that can be discussed when meeting with school staff (see "Meeting everyone face-to-face" below).

If your child is too young for school, consider setting up a pre-enrollment appointment with school administrators and nursing staff 3 to 6 months before your registration date.

Think positive partnership. Ask school staff to work with you to create an action plan for your child. The needs of your child always come first, but fostering good working relationships may go a long way in getting those needs met.

What's their policy? Your child may be eligible to receive special services and assistance at no cost, according to the Individuals with Disabilities Education Act (IDEA). Individual states and schools, however, may add details to the IDEA definition of a

child with a disability, which can affect a child's eligibility. To read the IDEA definition of disability, visit idea.ed.gov/explore/home, type "definition of disability" in the search box, and click on the "Sec. 300.8(c)" link.

Seek out the school nurse. Discuss needs, issues, and concerns, including what to do during emergencies. Provide a list of important phone numbers as well as any other medical information the nurse should know. "Especially in school systems, or anywhere public, they should be very conscientious about other children being sick and that's been our biggest thing about school," says Krystal. "Some days, if the other kids are sick, I just won't send her."

Ask Genzyme for help. Genzyme Case Managers and Patient Education Liaisons (PEL) are available to help educate school staff about all aspects of Pompe, including how to better understand disability resources and Individualized Education Program (IEP) procedures. In some cases, they have even met with school staff at the parents' request.

Start a support circle. Call a Genzyme Case Manager, or contact a Pompe patient organization (see page 7), to ask if they can connect you with parents who have a child who is your child's age, and parents of a child who is a few years older than yours. This way you'll have someone to share with — and someone to learn from.

Meeting everyone face-to-face

A group meeting is a great way for school staff to get information first-hand from you. Try to get everyone in the room at the same time, including your child's teacher(s), the school nurse, principal, school psychologist, and special education coordinator; and for older children, the guidance counselor.

Some topics you can consider discussing include:

How Pompe affects your child.

If your child does not have any visible symptoms, you may need to explain that he/she still has special needs. Discuss accommodations as they are needed, such as curriculum modifications due

to absences, tutoring, use of mobility devices, bathroom assistance, and use of special computers/keyboards.

The fatigue factor. Let the school staff know that your child may tire more easily than other children, have difficulty with some activities, and may need added rest times. Older children may need more time to get from class to class. Discuss options that can allow your child to participate in activities if he/she is able.

Risk of respiratory infection. Stress that something as simple as a common cold can lead to more serious problems like bronchitis or pneumonia. "They need to make sure they do their hand

washing and use sanitizer. I'm very fanatical about that because I'm scared Haley's going to catch something," says Krystal.

Question and answer time.

Providing teachers with printed information about Pompe (such as pages from www.pompe.com or the Pompe patient organization websites listed on page 7) can help them address questions from your child's classmates.

If your child is college-bound, view "The Road to College" presentation on the MDA website.

www.mdausa.org/services/webinar.html

Spreading your advocacy wings



If you have a desire to help others in the Pompe community, you may want to get involved with these organizations that are committed to helping:

The Acid Maltase Deficiency Association (AMDA) promotes public awareness of Pompe and works to raise funds to support research in Pompe. In addition, the AMDA seeks to provide direct patient advocacy and support through a variety of channels, including: newsletters, webinars, and one-on-one support. You can learn more by visiting www.amda-pompe.org. You can also reach the AMDA at 210-494-6144.

United Pompe Foundation (UPF) sponsors two to three regional patient meetings each year that patients and caregivers are encouraged to participate in. “We try to bring in sometimes a drug company, researchers, physical therapists, and patients that speak to their own experiences,” says David Hamlin, president of UPF. If you are interested in holding a meeting in your general area, you can call him directly at 559-227-1898 or visit www.unitedpompe.com. “We’re open

to listen to any ideas that people have that would better serve the Pompe community, whether it’s meetings or anything else.” If you would like to help promote public awareness of Pompe, Hamlin suggests talking to your local newspaper or asking your doctor to arrange a meeting with resident doctors who are doing their training or internships at a nearby hospital. “Most of these doctors have never met another Pompe patient in their life — or they might meet one 10 or 15 years down the road — and something from the personal interaction with the patient may bring something to mind where they will pick up on it better,” says Hamlin.

Association for Glycogen Storage Disease (AGSD) also works to provide public awareness and raise funds to support a broad range of research endeavors. Learn more at www.agsdus.org or by calling 563-514-4022.

Muscular Dystrophy Association (MDA) has an advocacy page on their website (www.mda.org) where you can sign up to become an MDA advocate, participate in their current advocacy campaigns, and find numerous resources on government and advocacy (including educational advocacy, employment and medical benefits, research advocacy, and more). You can call MDA at 800-572-1717.

Genetic Alliance, a network of thousands of organizations, including many disease-specific advocacy groups, focuses on collaboration in public health and public policy. Sign up for their *Weekly Bulletin*, with news from the genetics, health, and advocacy community. For more information, visit www.geneticalliance.org or call 202-966-5557.



To learn about Pompe:

- **Visit the Pompe Community at www.pompe.com.** This Genzyme website provides comprehensive information about Pompe.
- The **International Pompe Association (IPA)** website (www.worldpompe.org) offers downloadable brochures about a range of topics related to living with Pompe. Click on the “Publications” link, then look for the “Pompe Connections Brochure” link.

To learn from others who are managing Pompe:

- Consider joining the **GSDNet**, an online support community. You can subscribe to this electronic chat room through the **Association for Glycogen Storage Disease (AGSD)** website (www.agsdus.org/html/emailotherswithgsd.htm).

To contact a Genzyme Case Manager:

- Genzyme Care Coordination is a team of Genzyme Case Managers who offer confidential, personalized support and services to people living with Pompe, including assistance with insurance and reimbursement challenges. Their services are completely voluntary and offered at no cost to patients and caregivers. You can reach a Genzyme Case Manager in your area by calling 1-800-745-4447, Option 3, Monday-Friday, 8AM-6PM EST.

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