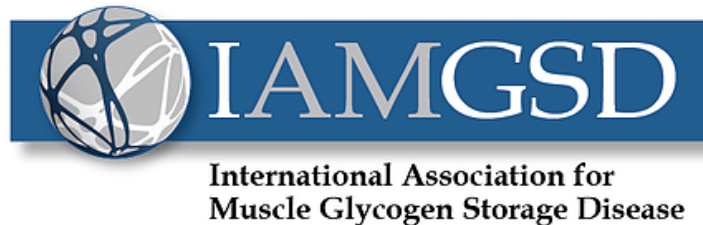


ADVOCACY AND MCARDLE DISEASE

AGSD US CONFERENCE SEPTEMBER 2019



Terri Chambers
Parent Advocate

WHO IS AN ADVOCATE?

- ▶ Health care professionals and organizations who work with patients and family members to navigate the health care system
- ▶ Family members or friends who accompany a patient to an appointment, ER visit or hospital admission
- ▶ Teachers, coaches, co workers: anyone who shows concern and support
- ▶ Most importantly....**YOU!**

THE ABC'S OF ADVOCACY

- ▶ **BE PREPARED** - Understand and be able to communicate what McArdle Disease is (and is not). The patient experience is valuable. Don't underestimate the value of **YOUR** knowledge and awareness.
- ▶ **ASK QUESTIONS** - You may likely be the first McArdle patient your clinician has ever seen. Don't assume they will understand the "ins and outs" of McArdles. There are no silly questions!
- ▶ **COMMUNICATE CONCERNS** - Don't be intimidated. Be polite yet assertive. If something doesn't seem right, speak up!

THE GOALS OF SELF-ADVOCACY

- ▶ To be heard and understood
- ▶ To build confidence and self sufficiency
- ▶ To get the support you need
- ▶ To be safe!

*Know yourself, know what you need,
know where to find it!*

WHEN MIGHT YOU HAVE TO ADVOCATE FOR YOURSELF OR FOR YOUR CHILD?

- ▶ At School
- ▶ At Work
- ▶ At Home
- ▶ At your doctors
- ▶ At the hospital
- ▶ With your Insurers
- ▶ While travelling - trains, planes, buses; hotels, etc.

SELF-ADVOCACY AND MCARDLES

- ▶ Understand and document your symptom history
- ▶ Make a list of all of the doctors you've seen
 - ▶ What tests you've had done
 - ▶ What recommendations and advice you've been given
- ▶ Request to be referred to an expert
 - ▶ Dr. Haller or other recognized clinician
- ▶ Ask for DNA confirmation
 - ▶ This may be necessary to enroll in a patient registry or to participate in research
- ▶ Be proactive! Suggest areas of support to your clinician - e.g.
 - ▶ Physical therapy evaluation/program, walking test, nutritional support, etc. (provide handouts and resources with guidance **specific** to McArdles)

ADVOCACY IN PRIMARY AND SECONDARY SCHOOL

- ▶ Set up a 504 accommodation plan (or IEP if appropriate)
- ▶ Go to <https://www2.ed.gov/about/offices/list/ocr/504faq.html> or speak with your school counselor for more information to set up accommodations such as:
 - ▶ Extra set of books to keep at home
 - ▶ Use of an elevator on campus if there are many stairs to climb
 - ▶ Additional time to complete writing assignments
 - ▶ Dismissal a few minutes early to allow time to get to class or bus safely
 - ▶ Excused absences/ability to make up work without penalty
 - ▶ PE accommodations or waiver
 - ▶ Pass to go to nurses office to rest when necessary
 - ▶ Water bottle in classroom

At school with McArdle's

Information for schools

A plain language guide to McArdle Disease and how to safely accommodate and support a pupil with this muscle disorder.

- McArdle's is an ultra rare inherited condition which restricts fuelling of skeletal muscles.
- Children with McArdle's cannot utilize the energy stored in the muscle.
- They have a serious shortage of energy early in any activity, and throughout all *intense* activity.
- They can develop cramping and pain. If activity continues, muscles become stiff, swollen, very painful and hospitalization may be required.
- They have a 'second wind' somewhat reducing their symptoms after about 10 minutes of careful activity.
- They need to keep active to keep their muscles functioning and healthy.
- They achieve a lot by utilizing practical and management techniques to protect themselves.
- Some simple adjustments and allowances by the school are an essential contribution to the child's safety and on-going improvement.



ADVOCACY IN COLLEGE AND UNIVERSITY

- ▶ Visit the campus Disability Resource Office to DOCUMENT your disorder and APPLY for services that may include:
 - ▶ Special parking
 - ▶ Mobility assistance (cart service)
 - ▶ Housing accommodations (i.e. dorm room on lower level)
 - ▶ Academic and personal counseling
 - ▶ Test accommodations
 - ▶ Priority registration

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ADVOCACY AT WORK

INFORMATION FOR YOUR EMPLOYER


Help your employer understand McArdles.
Take away their questions and concerns!
Focus on communicating what you CAN do
and where you may need simple
modifications.

Remember...hiding McArdles is STRESSFUL!
By staying silent, you just may be denying
yourself potential support as well as
putting yourself at risk for injury.

**At work
with McArdle's**
Information for employers

A plain language guide to McArdle Disease
and how to assist an employee to carry out
their duties safely and efficiently.

- McArdle's is an ultra rare inherited condition which restricts fuelling of skeletal muscles.
- People with McArdle's cannot utilise the energy stored in the muscle.
- They have a serious shortage of energy early in any activity, and throughout all *intense* activity.
- They can develop cramping. If activity continues, muscles become stiff, swollen and very painful.
- They have a 'second wind' somewhat reducing their symptoms after about 10 careful minutes.
- They achieve a lot by utilizing techniques to protect themselves, but for a desk-based role few techniques will be needed.
- They tend to develop determination and "stickability", making them good workers.
- Major adjustments in their role are *not* normally required, but some flexibility is helpful.

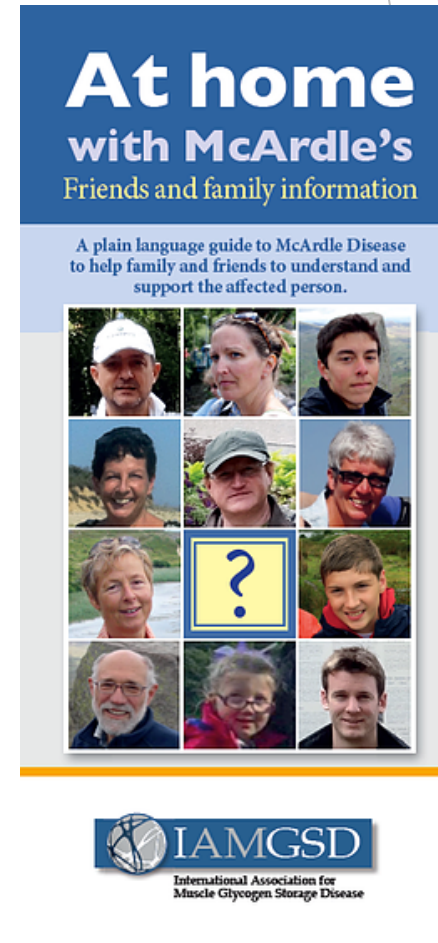
 **IAMGSD**
International Association for
Muscle Glycogen Storage Disease

ADVOCACY AT HOME

INFORMATION FOR FRIENDS AND FAMILY

Help friends and family understand this often misunderstood disorder. Looking healthy and “normal” can be confusing to others and make it difficult to appreciate the many serious challenges you face each day.

Let them know how McArdles affects your day-to-day life, and how they can help and support you.

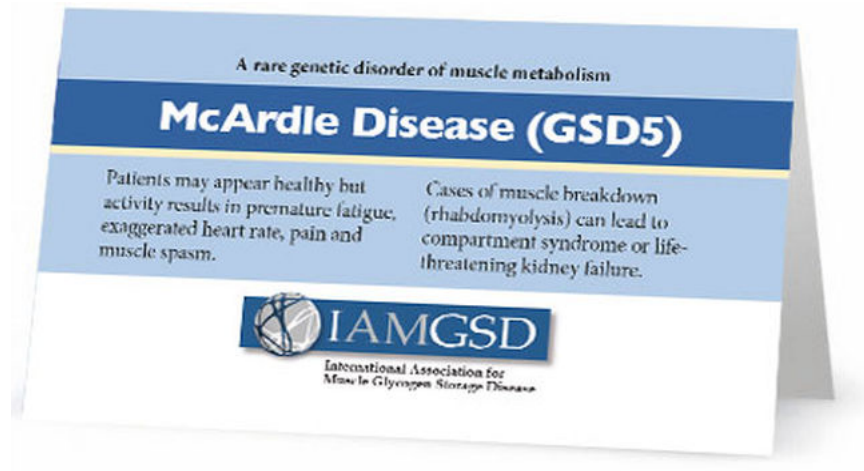


ADVOCACY WHEN RECEIVING CARE

- ▶ Come prepared to your appointment with questions
- ▶ Bring someone else with you that can be a “second ear”
- ▶ Offer new providers a copy of the MEDICAL OVERVIEW BOOKLET
- ▶ Take notes
- ▶ Temper your expectations when seeing a new doctor. You are the “expert” and likely will know more about McArdles than the provider you are seeing
- ▶ Share unsatisfactory care experiences with providers or medical facilities to the patient liaison or patient services department

ADVOCATING IN AN EMERGENCY BE PREPARED FOR THE UNEXPECTED!

Always carry your McArdle Emergency Card!



Guidance on what to do in an episode of **rhabdomyolysis**.

Most importantly, it has a brief note for emergency doctors. There is a space for you to write in a name and/or phone number of a doctor who should be contacted.

HOW TO BE AN EFFECTIVE ADVOCATE WITH INSURANCE COMPANIES

- ▶ Read your benefits
- ▶ Understand your rights
- ▶ Make a case -> include documentation from your doctor supporting the **NEED** to receive care for McArdles
- ▶ Be persistent! Don't take "no" for an answer
- ▶ Prepare to appeal!

<https://www.patientadvocate.org/wp-content/uploads/NavigatingInsuranceAppeals.pdf>

UNDERSTANDING THEIR LANGUAGE

- ▶ Medical Necessity
 - ▶ Without proper guidance you are in a medically compromised situation and can ultimately cost your insurer far more with the potential for repeated episodes of muscle injury and hospitalizations
- ▶ Muscle Energy Crisis with ACTIVITY
 - ▶ It is a common misconception in the medical community that McArdles only impacts “exercise” when in fact it affects all ACTIVITY
- ▶ Ultra Rare Condition
 - ▶ Most providers have never seen a patient with McArdles and do not understand the VITAL strategies that are necessary to manage exercise and activities of daily living (second wind, 6 second rule, 30 for 80)
- ▶ Specialized exercise testing (to recognize second wind)
 - ▶ Recognizing second wind is critical for someone with McArdle Disease. Standard exercise testing is not only inappropriate but dangerous for someone with McArdles

IAMGSD SUPPORT

- ▶ Familiarize yourself with the variety of McArdle Disease resources on <https://www.iamgsd.org> including:
 - ▶ At Home with McArdles
 - ▶ At School with McArdles
 - ▶ At Work with McArdles
 - ▶ 101 Tips for a Good Life with McArdle Disease
 - ▶ The McArdle Disease Handbook
 - ▶ Living with McArdle Disease
 - ▶ McArdle Disease Medical Overview
 - ▶ One Step At a Time: Walking with McArdle Disease
 - ▶ Videos, Presentations *and More!*

ADDITIONAL RESOURCES TO SUPPORT ADVOCACY

- ▶ Social media support groups
- ▶ Professional advocacy organizations
<https://www.patientadvocate.org>,
<https://rarediseases.org>
- ▶ Patient/Hospital Liasons
- ▶ Legislation <https://www.ada.gov/index.html>

POTENTIAL BARRIERS TO SELF-ADVOCACY

- ▶ Embarrassment
- ▶ Fear
- ▶ Denial
- ▶ Anxiety
- ▶ Depression
- ▶ Lack of confidence
- ▶ Lack of information

YOU ARE NOT ALONE....

GROUP ADVOCACY - GET INVOLVED!

- ▶ Sign up with lamGSD
- ▶ Become a member of AGSD US - help strengthen muscle GSD support in the US
- ▶ Participate in events (conferences, walking courses, global walking week, lamGSD fundraisers, future online workshops, etc)
- ▶ Volunteer! Check out the many opportunities on the lamGSD website: <https://www.iamgsd.org/volunteer>
- ▶ Register with the upcoming CoRDS Registry.
- ▶ Lend your support to others; tell your story, share your experiences!

HELP IAMGSD MEET OUR OBJECTIVES

- ▶ **Advocate** the patient viewpoint and needs to governments, patient organizations and medical professionals.
- ▶ **Disseminate** standards and best practice.
- ▶ Provide **support** for patients, organizations and medical professionals.
- ▶ Contribute to the planning of **research** projects and to support and assist those projects.
- ▶ Facilitate **communications** between organizations, especially internationally.
- ▶ Raise **awareness** amongst the medical profession, schools, sports clubs, and other relevant groups.
- ▶ Work towards reducing the average **age of diagnosis** to age 10.

ALL TOGETHER NOW...



TOGETHER WE ARE STRONGER!