



Cerdelga ▽
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Talking to my specialist doctor or nurse

Information for patients with Type 1 Gaucher disease
who have been prescribed Cerdelga

Talking to your specialist doctor or nurse

Preparing for an appointment

We know that you may have to travel a long distance for your appointment with your specialist doctor or nurse, therefore it is important you get the most from it.

This information should help you make the most from your appointment.



▼ This medicine is subject to additional monitoring. This will allow for quick identification of new safety information. You can help by reporting any side effects you may experience. If you get any side effects talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. You can also report side effects directly via the Yellow Card Scheme at www.mhra.gov.uk/yellowcard.

Why should you attend your appointment?

It can be tempting to skip appointments if you're feeling well or are too busy. There's nothing your healthcare team can do for you at the moment, so why bother?

However, it is important that you DO go to all the appointments that are made for you, for several reasons:

- ▶ **Even if you're feeling well**, it is important that your doctor tracks your progress to quickly pick up any issues should they occur. This will enable them to act quickly, and adjust your care accordingly to ensure you stay healthy in the long term, and minimise the impact of Gaucher disease type 1 on your life.
- ▶ **You may feel OK**, but when asked there may be a side effect you hadn't really noticed before, or there may be a new symptom that you have put down to something else. It's best to let a specialist doctor or nurse give you a good check-up.
- ▶ **This is your time to talk** about anything that has been bothering you, or things that are going well. You are the focus of the appointment, so unlike talking to friends and family, you can talk about ANY aspect of your Gaucher disease or your Cerdelga® treatment, in as much depth or detail as you wish, and if you have any questions you can be sure of a well-informed answer.

Informing your pharmacist

Cerdelga® cannot be taken with certain other medications.

For this reason, you must tell your pharmacist that you are taking Cerdelga® if you have a prescription for a medicine or you buy something over the counter for either your type 1 Gaucher disease or any other condition, even if totally unrelated. This is so they can check that it is safe for you to take other medicines, or offer any advice you might need.

The Cerdelga® Life pack contains a patient safety card with further information and can be given to your pharmacist.



Setting goals for your treatment

Clinical goals

The goals of Cerdelga® treatment are largely:

- ▶ *To reduce the symptoms of Gaucher disease type 1*
- ▶ *To cause minimal side effects and make them manageable until they pass*
- ▶ *To give you independence, and the ability to manage your own treatment*

If you feel that Cerdelga® is not achieving any of these goals, it's important to get in touch with your healthcare team to discuss ways to get you closer to a good outcome.

Starting out

Starting a new treatment for a long-term condition like Gaucher disease type 1 can be exciting and daunting at the same time. You don't know what to expect, or how well it will work for you, so it's important to be prepared and not every medication will suit every patient.

Your healthcare team will talk to you when you are prescribed Cerdelga® about:

- ▶ *What it does*
- ▶ *How you might feel physically*
- ▶ *What side effects you might get*

Follow-up

To see if Cerdelga® is working for you, you may have to go back for a follow-up visit. If you've switched from ERT, you might begin taking Cerdelga® from the very next day. If this is the case, you shouldn't notice a huge difference in your symptoms.

If your symptoms DO get worse, or you notice new symptoms, get in touch with your healthcare team straight away.

Personal goals to be checked by your nurses

It can be helpful, when you start on a new treatment, to take some time to think about what you would like to get out of your treatment and what your health goals might be. Having something to aim for helps you assess your progress, and it can be motivating to find that you are able to do something that you were unable to do 6 or 12 months ago.

Your goals might be practical or aspirational – so long as it's something that feels achievable and will motivate you. Ideas might include:

Practical Goals



To remember to take your medication every day



To take up a new healthy habit e.g. eating more vegetables or doing (appropriate) exercise



To keep a Gaucher diary of symptoms, side effects and experiences

Aspirational Goals



To travel abroad



To get a job or into further/higher education



To complete a walk, run or similar event



To become a Gaucher patient advocate (see the section later in this booklet)



To take up a new hobby or join an evening class

Talking to your specialist doctor or nurse

What are the goals for my treatment?

Use the next pages to note down the goals for your treatment. If you're struggling, have a chat to your nurse about what these could be, or use this book to give you some ideas.





The goals I want to achieve are...

Achieve by...



The goals I want to achieve are...

Achieve by...



The goals I want to achieve are...

Achieve by...



The goals I want to achieve are...

Achieve by...

What questions will your specialist doctor or nurse ask you?

In addition to any clinical tests that your doctor/nurse may carry out, they are also likely to ask you questions about your health since they last saw you. It can be helpful to make notes at home, and bring your notes with you at your next appointment, as it's easy to forget how something felt, or when exactly something happened when you're asked about it weeks, or even months, later.

Things you may be asked about might include:

- ▶ **How you have felt** in general
- ▶ How any **Gaucher-related symptoms** have been for you
- ▶ Whether you've noticed **any side effects** of Cerdelga®
- ▶ How you're getting on with **taking Cerdelga®** and whether you've had any problems with sticking to the regimen as prescribed

It's important to answer these questions as honestly as you can, so you can get any appropriate help that you need. When your specialist doctor or nurse gives therapy as an intravenous infusion, they can see for themselves that you have received the right treatment at the right dose, every time. When you leave their clinic and care for yourself at home, they rely on you to tell them exactly what's been happening. Nobody would want you to struggle on silently if there's something they can do to help. Even if you've missed a number of doses, it's essential to tell your healthcare team about it – this is so that they can assess whether any improvements or problems have been due to the Cerdelga® or not.

Questions you might want to ask your specialist doctor or nurse

Don't be afraid to ask your healthcare team about any aspect of Gaucher Disease type 1 or your treatment. Even if we have answered them in this book, you might have more questions on what you have read. If it matters to you, it will matter to them.

Questions you might be interested in finding the answers to could include:

- ▶ *How will taking Cerdelga® make me feel physically?*
- ▶ *Will I get any side effects?*
- ▶ *If I do get side effects, what can I do about them? And how long will they last?*
- ▶ *Can I drink alcohol/take supplements while taking Cerdelga®?*
- ▶ *How long will I have to take Cerdelga® for?*
- ▶ *How long will it be before I feel the benefit of Cerdelga®?*
- ▶ *Do you have any other patients that have taken Cerdelga®? What do other people say about it?*
- ▶ *What are the main benefits and drawbacks of taking Cerdelga®?*
- ▶ *What should I do if I have trouble swallowing the capsules?*
- ▶ *What does a Cerdelga® capsule look like? How big is it?
How big is the packet?*
- ▶ *How do I store Cerdelga®?*
- ▶ *What should I do if I run out of Cerdelga®?*

Getting the most out of your appointments

Use these simple steps to get the best out of visits to your specialist doctor or nurse:

- ▶ Write down a **list of key questions** you want to ask
- ▶ Note down **which of these are priorities** if you run out of time. You could even ask your specialist doctor or nurse if you could send these across prior to your appointment if you have their email address
- ▶ Remember to **bring any notes** about how you have been feeling
- ▶ **Take a friend or family member** along with you to be a second ear
- ▶ Ask your specialist doctor or nurse to **explain things** in a different way or in more detail if you need to. Don't worry about asking questions – they are likely to have been asked before, and if something is troubling you, your doctor will be glad to address it

Travelling to your appointment

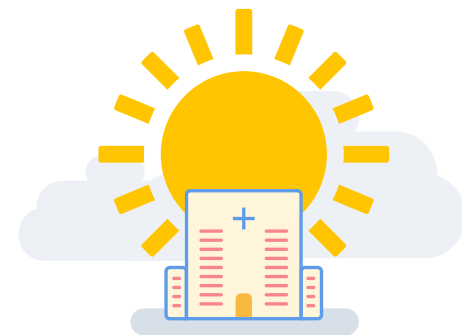
Preparations you'll need to make for your appointment will depend on what your healthcare team intend to do that day. It may be a good idea to wear loose, comfortable clothing with minimal amounts of metal (zips, jeans buttons, poppers, jewellery etc.) in case you need to have an MRI scan or x-ray, or if you need to roll your sleeves up to have blood taken.

It can be helpful to bring with you any medication you take:

This includes vitamins, herbal supplements, traditional Chinese medicine, over-the-counter medicines and medicines prescribed by a specialist doctor or nurse (tablets, liquids or inhalers).

Talking to your specialist doctor or nurse

Who To Contact



UK Treatment Centres

Birmingham

Queen Elizabeth Department of Inherited Metabolic Diseases
Heritage Building
Queen Elizabeth Hospital
Mindelsohn Way
Edgbaston,
Birmingham
B15 2TH



Telephone

+44 (0) 121 627 1627 Ext 51592

Cambridge

Addenbrookes Lysosomal Storage Disease Unit
Addenbrookes Hospital
Hills Rd
Cambridge
CB2 0QQ



Telephone

+44 (0) 1223 274 634

Salford

The Mark Holland Metabolic Unit
Salford Royal NHS Foundation Trust
Stott Lane
Salford
M6 8HD



Telephone

+44 (0) 161 701 2137

Cardiff

The Inherited Metabolic and Lysosomal Disease Service
University Hospital of Wales
Heath Park
Cardiff
CF14 4XW



Telephone

+44 (0) 29 2074 3275

Belfast

Belfast City Hospital
Genetics Department
Lisburn Rd
Belfast
BT9 7AB



Telephone

+44 (0) 2890 240 503 Ext 3874

Notes

UK Treatment Centres (Continued)

London

National Hospital for Neurology and Neurosurgery
Metabolic Disease (Adult Inherited)
Charles Dent Metabolic Unit
Box 92
Queen Square
London, WC1N 3BG



Telephone

+44 (0) 207 829 8778

London

The Royal Free Lysosomal Storage Disease Units
Pond St,
London
NW3 2QG
+44(0)207 472 6409



Telephone

+44 (0) 207 472 6409



