



Cerdelga▼
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Living well with Gaucher Disease

This booklet is for adults with Type 1 Gaucher Disease who have been prescribed Cerdelga.

The information in this booklet is not intended to replace the Patient Information Leaflet accompanying your medication and should act as further information. If you have any questions or concerns regarding your treatment, please consult your Patient Information Leaflet, or get in touch with your specialist healthcare team.

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Welcome!

How is life different with Gaucher Disease?

What does 'living well' mean to you?

This booklet is full of tips, advice, and support to help you to live well with Gaucher Disease.

From managing worries and setting goals to keeping on top of treatment, it might seem like there is a lot to cope with. However, there are lots of little things that you can do to make living with Type 1 Gaucher Disease easier.

You can find more information about getting the best out of life with Type 1 Gaucher Disease in the [Support Networks in Gaucher Disease](#) booklet.

A lot of things will impact how much your life is affected by your illness.



Anyone who's experienced a long-term illness knows just how important it is to have your own voice. Not everybody will understand immediately how your disease affects you unless it is explained to them. Therefore, take the time that you need to put your health first, consider your own needs and have those important conversations.

Occasionally, you will need to be your own source of strength – this is not something you should be afraid of. People often surprise themselves at how strong they become, particularly when they go through times of difficulty or pain.

There will be ups and downs, and keeping in good health throughout is important. It is good to look back at how far you have come in your journey when you are going through a difficult time.

To learn more about getting the most from the people that care about you (i.e. family and friends, your healthcare team), take a look at the [Support Networks Within Gaucher Disease](#) booklet.

Setting goals



Goals are designed to put you first and can be a good way to see how far you have come.

Health and treatment goals

Your healthcare team will have some goals in mind for you and your Cerdelga treatment. These may include:

- *Helping you become more independent*
- *Improving your symptoms of Type 1 Gaucher Disease*
- *Helping to address any side effects you may be struggling with*

Not every medicine works for every patient, and you may have to arrange to visit your healthcare team again to see how Cerdelga is working for you.

Your goals

Just as your healthcare team might set health and treatment goals for you, it can help to set your own personal goals too. Goals can be based on small every day improvements, or more aspirational targets. Aim for goals you can feel good about, but ones that are still achievable!



Setting goals



TIP: Try writing your goals on a piece of paper, and sticking them on your fridge – you'll be reminded of what you want to achieve every morning.

Keeping a Gaucher Disease diary

A Gaucher Disease diary is a place for you to write down any important information related to your treatment and health. It can help you:

- Keep track of any side effects to discuss with your healthcare team at your next appointment
- Monitor any symptoms of Type 1 Gaucher Disease you might be having which you can discuss with your healthcare team at your next appointment
- See how your health changes over time
- Look at your own personal goals and development



It is really important to use your healthcare team for any support you may need. You should always talk to them if you have any worries, including reporting any side effects you may be experiencing.

Keeping a Gaucher Disease diary

Suggested topics:

Side effects
.....
.....
Symptoms
.....
.....
Bone pain and other serious events
.....
.....
Wellness and achievements
.....
.....
Concerns
.....
.....

Keeping a diary can help you keep track of everything to do with your illness, in one place.

Personal goals and aspirations
.....
.....
Important contacts, addresses and dates
.....
.....
Questions to ask at your next appointment
.....
.....
Anything else related to your wellness and Gaucher Disease
.....
.....

TIP: Get yourself a diary or planner that you like, that suits **your** needs. What about a portable, pocket sized diary planner that you can take everywhere? Or, you may prefer a big A4 notepad to keep at home, that you can stick appointment letters and results in, with lots of space to write or draw.

How to deal with worries

Getting to grips with a long-term illness may often mean working out a few worries along the way.

Although they are not nice, having worries is not always a bad thing. Worries are an important way of our body and brain telling us that something isn't right. They can help you to:

- **Improve something you are not happy with**
- **Speak to a doctor when you may not have done so otherwise**
- **Encourage you to speak to others to solve your problems**

If you cannot change a situation, try your best not to worry about it. Worrying about things you cannot change is very tiring, and rarely leads to a good solution. In these situations:

- **Try to distract yourself**
- **Do something positive, or help others – this can help to make your worries seem smaller**
- **Speak to friends, family, or a support group**
- **Talk to others that might understand what you are going through**

Managing your worries and dealing with new or concerning things is part of life, especially if you are living with a long-term illness. Finding the balance between listening to your gut feelings and not over-thinking a situation is important.

For more information on getting help from others, have a look at the **Support Networks in Gaucher Disease** booklet.

Worries about side effects

Sometimes when you worry, you can end up looking for signs that something might go wrong. Thoughts like this can sometimes make you feel worse or change how you act.

If you are worried about side effects, you should consult the Patient Information Leaflet that comes with your medicine.

If you are still concerned, it's best to talk to your healthcare team, and write your concerns down (in your Gaucher Disease diary) to discuss with them. They will be able to reassure you and make sure everything is okay – they may also be able to carry out some tests to help put your mind at ease.

How to deal with worries

Feeling 'different'

Any kind of news that will affect the rest of your life, will always take time to accept and adjust to. This includes being told you have Type 1 Gaucher Disease.

Hospital visits and needing to take time off a little more often than others are likely going to be part of your life story. Take time to think about how you are coping with this, and reflect on if it is changing how you see yourself compared to others.

Everybody is unique. You may find accepting this new part of your life easy, but others will find it a little more difficult. It is really important to remember that although your illness is part of you, it does not have to take over.

For more information on managing worries, take a look at the [Support Networks in Gaucher Disease](#) booklet, or visit the Gauchers Association website at: www.gaucher.org.uk

Tips for keeping on top of treatment

Setting a treatment routine that works for you

Making sure you have a good treatment routine could be one of the most important things you can do to help you stay as healthy as possible:



- **Use something that you do at the same time every day already (like brushing your teeth) to build your treatment routine around.** Taking your medicine at the same time as another part of your daily routine can really help you remember to do it

- **Always try and plan ahead.** Where are you going to be at the weekend? Will this change when you take your medication? By planning ahead of time, you can often avoid forgetting to take medication with you



- **Set a daily reminder on your phone**
- **Place reminders anywhere you might find helpful (on the fridge, or your bedside table)**
- **Remember, practice makes perfect!**

It is normal to make mistakes. If you do, don't worry and focus on how to get back on track.

Long-term planning

If you have been managing your Type 1 Gaucher Disease for a long time, sometimes you may find that new challenges appear. Big changes may mean that you find it more difficult to take your treatment or talk to your healthcare team in the future.

- Next time you find yourself feeling a bit tired about treatment, take a moment to consider why this might be.
- Is there something else going on in your life that may be a distraction?
- Do you need to make some changes to when you take your treatment?
- It is quite normal for you to feel like you have fallen off the track. Try to focus on what you have been doing well and any new changes you might need to put in place.

Travelling with Cerdelga

Travelling with your medication, particularly if you are leaving the country, will require a little bit of planning ahead.

Store it in a safe place

Cerdelga needs to be stored in a dry place at room temperature. Make sure to keep it out of reach of any children. It's also best to keep your medication in its original packaging, so that others know what it is.

Pack enough for emergencies

Make sure you take enough supply of your medicine to cover any emergencies or getting home later than expected. You may need to talk to your healthcare team to get more medicine ahead of time.

Going abroad

- Take a copy of your prescription with you, in case you need to prove it is your medicine
- You may need to declare your medicine at customs/passport control
- Check before you book your trip as to whether your medication is allowed at your destination

Myths and misconceptions about medicine

It can be tough with any treatment to know whether what people say is true or not.

Some common myths associated with taking medication:

- x When I feel better, I can stop taking my treatment**
As someone living with a long-term illness, feeling better is a sign that your medicine is working. If you stop taking it without talking to your healthcare team you may start to feel worse.
- x If I miss a dose, I can take more to make up for it**
Taking more than your recommended dose of medicine can increase your chances of side effects and should be avoided, even if you have missed a dose. Doubling up after a missed dose will not make up for the time your body has been exposed to less medicine, and can carry risks.
- x I will still need infusions at the hospital**
As long as your healthcare team recommends you take Cerdelga, you will not need infusion-based therapy for the treatment of Type 1 Gaucher Disease (this is known as enzyme replacement therapy or ERT).
- x My doctor can tell if my treatment is not working**
Although your healthcare team will need some test results to assess your progress, it takes more than this to know if your treatment is working. It's important to tell them how

you have been feeling, including if you might have been experiencing any side effects.

- x It doesn't matter how you swallow your capsule, as long as you take it**
Although this is true for some medicines, Cerdelga needs to be swallowed whole. The capsules are specially coated to carry the medicine through your stomach, so that they can dissolve in your digestive tract at the right time. Breaking the capsules open could result in a reaction in your mouth, throat or stomach, leading to you having the wrong amount of medicine in your system.
- x Natural supplements are harmless, and won't affect my medicine**
This is a common misconception about natural products. Natural is not equivalent to harmless or safe. Some natural supplements can also interact with Cerdelga, so it is important to tell your healthcare team if you take ANY natural supplement or herbal medicines.

If you are not sure about anything relating to your treatment speak to your healthcare team next time you see them, or if you need to speak with them more urgently pick up the phone or send them an email. You should always consult your healthcare team before making any changes in treatment.


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You can refer to this booklet at any point in your treatment journey, for information on your journey with Gaucher Disease and Cerdelga.

If you find you have more questions, there is a Patient Information Leaflet included in the packaging of your Cerdelga tablets. You can also speak to your doctor, nurse or pharmacist.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects that you may get. In the UK, see www.mhra.gov.uk/yellowcard for how to report side effects. In Ireland, see www.hpra.ie for how to report side effects.