

Key contact information

Write your key Gaucher Disease contacts here:		
	Name/Role:	
	Contact information:	
	Name/Role:	
	Contact information:	
	Name/Role:	
	Contact information:	

▼ 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.

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Welcome to your Cerdelga patient booklet!



Cerdelga is a medicine for the treatment of your Type 1 Gaucher Disease



Take Cerdelga whole, once daily, with or without food, at around the same time every day. Do not crush, dissolve, or chew the capsule before you swallow it, and swallow it with water



Avoid grapefruit/grapefruit juice, as it affects the levels of Cerdelga in your body



Don't stop taking Cerdelga until your healthcare team tells you to do so



Several medicines influence the amount of Cerdelga in your blood, and Cerdelga can influence the amount of other medicines you are taking. This includes herbal medicines, traditional Chinese medicines, over-the-counter remedies, vitamins and medicines. It is important that you tell your doctor if you have used, or are using, any other medicines of any kind. A full list of the medicines that can interact with Cerdelga are found on the back pages of this booklet

What is Cerdelga and how does it work?

Cerdelga is a medicine for the long-term treatment of your Type 1 Gaucher Disease.

Gaucher Disease occurs when there isn't enough of an **enzyme** called β -glucosidase. Without this enzyme to break things down, a fatty substance called **glucosylceramide** (GL-1) builds up in cells within your body causing them to become bloated and dysfunctional, leading to the symptoms of Gaucher Disease.

Cerdelga works by blocking the enzyme that creates GL-1, meaning there is less GL-1 in your body. This may help with reducing the symptoms of Gaucher Disease.

There are two types of treatment for Gaucher Disease – enzyme replacement therapy (ERT) and substrate reduction therapy (SRT). ERT helps the body recycle more waste (GL-1), while SRT helps the body produce less of it to begin with.

Cerdelga is an SRT.



Enzyme: A biological molecule that helps speed up chemical reactions in your body.

Gaucher Disease: A lysosomal storage disorder.
There are three different types of Gaucher Disease;
Type 1, Type 2 and Type 3. Each type is slightly different in terms of what symptoms they are associated with and when they occur.

β-glucosidase: An enzyme in your body that breaks down GL-1.

Glucosylceramide (GL-1): A type of fat that is used as a building block to help make the cells in your body.

How to take Cerdelga (once daily)

What to do if you miss a tablet (dose)

You have been prescribed Cerdelga tablets once daily, as your liver breaks down Cerdelga more slowly than some people. This means that you only need one capsule of Cerdelga a day.



You can take Cerdelga with or without food.



The only food you need to avoid is grapefruit/ grapefruit juice. This is very important as it may increase the amount of Cerdelga in your body.



You should take Cerdelga at the same time every day. To help you remember it may be helpful to do it with an activity you do every day, for example:

- Before or after brushing your teeth
- When you eat
- Before bed
- Before you leave for work



Cerdelga must be taken whole. Do not crush, dissolve, or chew the capsule before swallowing. Swallow Cerdelga with water.



Keep Cerdelga in a dry place at room temperature, away from children.

It's important that you don't miss a dose. If you do forget to take a capsule, do not take a double dose. Carry on as usual and take your next dose at your usual time.



If you are missing doses a lot, try setting a reminder on your phone or try asking a reliable person to remind you to help you get into a routine. If this still isn't helping you to remember, talk to your healthcare team. They can help you make the most of your Cerdelga treatment.

If you are missing doses because you are having problems swallowing the capsules, try:

- Taking them with plenty of water
- Taking a few breaths and relaxing before you take each capsule

If you are still struggling to swallow your capsules it is important you talk to your doctor so that they can help you. They can work out different ways to make taking the capsule easier, or look at prescribing you a different medication.

What to do if you take too many capsules

If you take more capsules than you were prescribed, or a double dose, it's important that you speak to your healthcare team straight away. Make sure you tell them how you are feeling. Too much Cerdelga may:

- Make you feel sick
- Make you feel dizzy
- Cause a loss of balance
- Cause you to have low blood pressure
- Slow your heartbeat

Please seek urgent medical advice if anyone else swallows your Cerdelga.

Side effects

Like all medicines, Cerdelga can cause some side effects, although not everybody has them.

Common side effects of Cerdelga can include:

- Headache
- Dizziness
- Dysgeusia
- Palpitations
- Throat irritation
- Dyspepsia
- Abdominal pain upper
- Diarrhoea
- Nausea
- Constipation
- Abdominal pain
- Gastroesophageal reflux disease

- Abdominal distension
- Gastritis
- Dysphagia
- Vomiting
- Dry mouth
- Flatulence
- Dry skin
- Urticaria
- Arthralgia
- Pain in extremity
- Back pain
- Fatigue



Some people may experience fainting or feeling faint. If this happens, tell your healthcare team straight away.

Managing side effects

When you visit the Gaucher centre, you may have a number of tests:

- How your heart, kidney and lungs are working
- Whether you are pregnant (if you're a woman)
- Your blood levels to check if you are anaemic

As part of these visits you will also be asked if you have had any suspected side effects. It is very important that you remember to mention anything you have experienced so that your healthcare team can assess you appropriately.



It's not unusual to worry about any side effects you are having. Keeping a diary and then talking about these with your doctor can help to work out what is going on.



If you do start getting new symptoms, don't wait until your next appointment to get help. Contact your healthcare team for advice.

Will my other medications be affected?

There are certain medications you cannot take if you are on Cerdelga. This is because they may cause **drug-drug** interactions (when two or more drugs interact with one another). Therefore it is very important to:

- Tell your doctor if you are on any other medication BEFORE starting Cerdelga
- Make sure that if you are told to start taking any new medications AFTER you have started Cerdelga, such as by your GP or pharmacist, that they know you are on Cerdelga
- Carry your patient card with you at all times and show it to any professional before accepting a new medication

Medicines that may increase the amount of Cerdelga in your body:

- Antidepressants
- Antiarrhythmia medicines (irregular heartbeat)

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Antibiotics

Will my other medications be affected?

Medicines that may decrease the amount of Cerdelga in your body:

- Antibiotics
- Anti-epileptics

Cerdelga may also increase the levels of other medications in your body. These include:

- Anticoagulants (making your blood thinner)
- Anti-epileptics
- Antipsychotics



Your healthcare team will be able to give you a full list of all the medicines that may either affect or are affected by Cerdelga. This includes herbal medicines, traditional Chinese medicines, over-the-counter remedies, vitamins, and medicines prescribed by a doctor for a different health issue, even if it seems to be unrelated to Type 1 Gaucher Disease.





You can refer to this booklet at any point in your treatment journey, for information on 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.

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