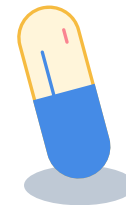


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About Cerdelga® ▽ (Eliglustat) Once Daily

About Cerdelga® (Once Daily)

What is it?



▼ 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. By reporting side effects you can help provide more information on the safety of this medicine.

Overview of Cerdelga®

Cerdelga® is an oral medication used for the treatment of adults with Gaucher disease type 1. It's a capsule you take once a day, every day.

This may be the first medication you have had to treat your Gaucher disease type 1, or it may be an alternative treatment for you from infusions of enzyme replacement therapy (ERT).



How does Cerdelga® work?

Gaucher disease type 1 occurs when a person has a faulty version of a gene in their DNA, responsible for making an enzyme called acid β -glucosidase which breaks down a fatty substance called glucosylceramide that is naturally produced by the body.

- ▶ Without enough of this enzyme, the fatty substance builds up in special cells of the body causing them to become bloated and dysfunctional.

Think of your faulty gene as a spelling mistake in the instructions which tell things how to work in your body. If one letter is wrong in a word, it can cause it to work incorrectly, much like a faulty gene in your DNA. This could make a sentence take on a whole different meaning, for example 'You cannot exit here' becomes 'You cannot exist here'. With the addition of the letter 's' the sentence loses its original purpose. In a similar way, your faulty gene stops the functioning of the acid β -glucosidase, therefore it cannot break down glucosylceramide.



How does Cerdelga® work? (Continued)

This causes the symptoms of Gaucher disease type 1 that you may have been experiencing prior to diagnosis or treatment. If you've ever had treatment for Gaucher disease type 1, chances are you've had intravenous ERT infusion.

ERT works by replacing the broken enzyme in affected Gaucher cells, allowing the body to break down the fatty substance itself.

Cerdelga works in a different way to ERT. It falls under a category of treatments called “substrate reduction therapy” (SRT). SRT reduces the production of the fatty substance, so that it doesn't build up in the first place.

Try thinking of it like this:

If your dustbin is getting full on a weekly basis, there are two ways in which you could solve it. One would be to get your rubbish taken away more regularly, which is the equivalent of ERT breaking down the fatty substances in your body. The other would be to put less rubbish in the bin to start with, which is the equivalent of SRT, which stops the fatty substances being produced.

Effectiveness of Cerdelga® in the treatment of Gaucher Disease Type 1

If you are moving from ERT to Cerdelga you may wonder if a tablet can be as effective as an infusion.

In clinical trials, Cerdelga® was shown to be effective at treating the main symptoms of Gaucher disease type 1. This was proven in patients who had previously been treated with ERT as well as those patients who had not been on treatment for Gaucher before.

In people who had previously been treated with ERT, Cerdelga® showed comparable effectiveness in maintaining:

- ▶ **Stability of haemoglobin** and platelet levels in the blood
- ▶ **Spleen and liver** volume

In people who had never been treated for Gaucher disease type 1, Cerdelga® helped to improve (vs. placebo):

- ▶ **Levels** of haemoglobin and platelets
- ▶ **The size** of the liver and spleen
- ▶ **Levels of Gaucher cells** in the bone marrow (for most patients)
- ▶ **Bone density** (for most patients)

About Cerdelga® (Once Daily)

How do you take it?



Establishing a treatment routine

Your specialist doctor or nurse will have explained to you that Cerdelga® should be taken once a day. As Cerdelga® can be taken with or without food, you can fit it into your daily routine in a way that suits you best. However, it is important that you take Cerdelga® at the same time every day and it is important not to skip a dose. In the front of your Cerdelga® pack you can find stickers to place on your capsule blister pack to help you remember when to take your medication. If you require extra stickers please request these from your specialist doctor.

Some people find that it's easier to remember to take medication when it's bound up with another part of your daily routine such as:

- ▶ **At mealtimes**
- ▶ **Brushing your teeth**
- ▶ **With the morning and evening news**
- ▶ **When you leave for and get back from work**

Important tips on how to take Cerdelga®

Cerdelga® can be taken with or without food, and should be taken with water to enable easy consumption.

Cerdelga® and grapefruit

Grapefruit (either whole or as juice) has an effect on many drugs, including Cerdelga®. In the case of Cerdelga®, grapefruit has the effect of increasing your body's exposure to the active ingredient in the capsule, which can therefore increase the risk of side effects.



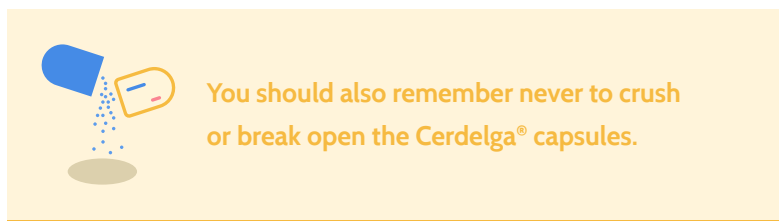
One key thing you need to remember is to avoid grapefruit.

Cerdelga® Capsules

The capsules have been designed to carry the active ingredient through your stomach into your digestive system, where the capsule is broken down and the drug is released at the right time, in the right place, and in the right quantity. If you crush your capsule, or break it open and just eat the contents, this process can't happen.

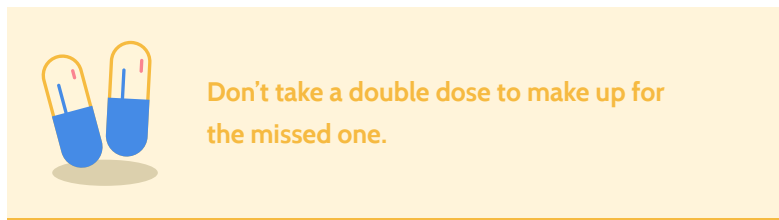
The effect can be that you get too much or too little of the active drug, you may absorb it at an unpredictable rate, and you may get reactions to the drug in your mouth, throat and stomach.

If you feel you need to crush or break the capsules because you find it difficult to swallow them whole, please talk to the specialist doctor who prescribed Cerdelga® for you as soon as you can – they can then work to find ways to make taking the capsule easier, or look at prescribing you an alternative therapy.



Cerdelga® and double dosing

It is important not to skip doses of Cerdelga®, however, if you forget a dose, just carry on as normal and take the next dose at your usual time.



How to alleviate potential side effects

Like all medicines, Cerdelga® can cause side effects, although not everybody gets them. Most side effects associated with Cerdelga® are not serious, and generally get better over time. The most common side effect people on Cerdelga® report is diarrhoea.

Other side effects you might notice include:

- ▶ **Headache**
- ▶ **Feeling sick** (nausea)
- ▶ **Stomach pain**
- ▶ **Wind** (flatulence)
- ▶ **Joint pain** (arthralgia)
- ▶ **Tiredness** (fatigue)
- ▶ **Sore throat** (oropharyngeal pain)

If you experience these side effects, talk to your specialist doctor before taking any medications for them. Your specialist doctor will be able to advise you on effective options that are OK to take alongside Cerdelga®.

A small proportion of people who take Cerdelga® can experience "syncope", which is fainting or feeling faint. If this happens to you, tell your specialist doctor straight away.

Reporting side effects

As well as telling your specialist doctor about any side effects you may experience, you are also encouraged to report side effects through the Yellow Card Scheme. This is a scheme which collects information on suspected problems or incidents to help ensure medicines are made safer in the future.

Any medicine can cause side effects, but sometimes rare adverse reactions aren't known until many people have used a medicine for a long time.

If you think your medicine is causing symptoms that aren't listed as a side effect in the patient information leaflet, it's best to report it, even if you're not sure.

There are a few different ways you can report a side effect through the Yellow Card scheme



Complete an online Yellow Card form at

<http://www.yellowcard.mhra.gov.uk/yellowcards/reportmediator>



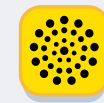
Pick up a Yellow Card form from your GP surgery or your local pharmacy

Once completed, send it to the freepost address on the form



Call the Yellow Card free phone hotline

0808 100 3352, Monday to Friday, 10am to 2pm



Use the new Yellow Card app to report any suspected side effects

You can also use the app to view any previous reports you've submitted

Managing worries about side effects

Sometimes if we are a bit worried about something that is new this can lead to us overthinking or focusing too much on what might happen. These thoughts sometimes make us feel worse unnecessarily or affect what we do. If you think you have been experiencing any potential side effects then it is important to write these down to discuss with your specialist doctor.

Giving your specialist an accurate picture of what is going on helps them to give you the best type of advice and support.

Remember to note down what you think has happened, when, how often and any other relevant information (e.g., changes to your routine or how you felt).

By talking things through, you and your specialist doctor will be able to work out what's really going on. They will be able to help you find ways to manage some side effects, carry out any tests that might be necessary, and may even be able to put your mind at rest about any niggling worries.

Will your other medication be affected?

There are certain medications that can't be taken if you are on Cerdelga®. They can cause what are known as drug-drug interactions, or DDIs. If you are on ANY medication, you must tell your specialist doctor about it BEFORE starting Cerdelga®.

If you are prescribed another medication AFTER you have started on Cerdelga® (such as by your GP, or another specialist), you **MUST** tell the prescribing doctor that you take Cerdelga® (eliglustat) so that they can assess your needs. Ensure that you carry round your patient safety card, found at the front of this pack, and show this to any healthcare professional before accepting a prescription for another medication to take alongside Cerdelga®.

Cerdelga® can interact with other medications in different ways

Certain drugs may increase the amount of Cerdelga® in your body, meaning that you'll get too much.

This includes some:

- ▶ **Antidepressants**
- ▶ **Antiarrhythmia medicines**
- ▶ **Antibiotics**

Other drugs may decrease the amount of Cerdelga® in your body, making it less effective.

These include some:

- ▶ **Antibiotics**
- ▶ **Anti-epileptics**

Cerdelga® can also sometimes increase the levels of other medications in your body.

These include some:

- ▶ **Anticoagulants**
- ▶ **Anti-epileptics**
- ▶ **Antipsychotics**

Your specialist doctor will be able to access a comprehensive list of medications that either affect Cerdelga® or are affected by Cerdelga®. This is why it's important to talk to a specialist doctor before combining Cerdelga® with any other medication – that includes herbal medicines, traditional Chinese medicines, over-the-counter remedies, vitamins, and medicines prescribed by a specialist doctor for a different health issue, even if it seems to be unrelated to Gaucher disease type 1.

Can I take Cerdelga® if I am pregnant, or planning to become pregnant?

The effects of Cerdelga® on an unborn baby are not known. For this reason, you should not take Cerdelga® if you are, or are planning to become, pregnant. If you are taking Cerdelga® and then discover that you are pregnant, stop taking Cerdelga® and contact your specialist doctor immediately.

If you are planning to become pregnant or you are breastfeeding, talk to your specialist doctor before taking Cerdelga®

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Travelling with Cerdelga®



Store it sensibly

Cerdelga® doesn't need to be stored in any particular way. Just ensure it is kept in a dry place, at room temperature, and well out of the reach of children. Always keep Cerdelga® in its original packaging so it can be identified easily. This also acts as a reminder of how to take it.

Ensure you have enough medication for your trip

When travelling, ensure that you have enough Cerdelga® with you to last for the entire trip. It's also a good idea to have a plentiful supply with you in case of any unforeseen delays such as flight cancellations. If you are planning a trip, ensure that you contact your specialist doctor or nurse as far in advance as possible to be sure they can get you the additional medication you may require.

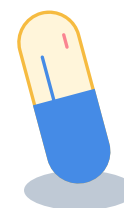
Going abroad

If you are travelling abroad, you may need to take extra precautions. Make sure you carry a copy of your prescription with you, in case you need to prove that it is your medication. You may need to declare your medication at customs or passport control – different countries have different rules about which medicines you can bring in.

It's a good idea to check before you book your trip whether your medication is allowed into the destination country

About Cerdelga® (Once Daily)

Taking your medication as recommended



Taking your medication as recommended

If you miss one dose, don't worry. Just skip that dose and take your next capsule at the usual time. Don't take extra Cerdelga® to make up for a missed dose. If you are regularly missing doses however, Cerdelga® can't work at its best.

If you find that you're missing doses a lot, there are a few things you can try:

- ▶ **If it's a case of always forgetting, try:**
 - › Setting reminders or alarms (e.g. on your phone)
 - › Asking someone reliable to remind you until you set up a routine
 - › Making Cerdelga® part of your normal daily routine, so it becomes a habit (e.g take it after brushing your teeth)
 - › Using the blister pack stickers found within this pack to help remind you when you have taken your medication
- ▶ **If it's a case of finding it physically difficult to swallow the capsules, try:**
 - › Taking the capsule with plenty of water
 - › Talking to your specialist doctor for advice
 - › Being distracted – do it whilst watching TV or when someone is talking to you

What happens if you take too many capsules of Cerdelga®?

If you take Cerdelga® as recommended by your specialist doctor, you will get the maximum benefit with no risk of overdosing. If you take more tablets than recommended, or don't leave long enough between doses, then the risk rises sharply.

If you accidentally take too much, you may experience some of the symptoms of Cerdelga® overdose. These include:

- ▶ **Dizziness**
- ▶ **Loss of balance**
- ▶ **Low blood pressure**
- ▶ **Abnormally slow heart beat**
- ▶ **Vomiting**

If anyone else swallows your Cerdelga® - adult or child - seek medical advice urgently

If at any time you take too much Cerdelga®, seek medical advice urgently – even if you feel fine. You may need to have medical supervision and be treated for overdose

How will your use of Cerdelga® be monitored?

At each visit to a Gaucher centre, you may have a number of tests to check:

- ▶ *Your heart, kidney and lung function*
- ▶ *Whether you are, or are planning on becoming, pregnant – if you're a woman*
- ▶ *Your blood, and the presence or levels of particular substances e.g. platelets*

You may also have an MRI scan or x-ray to determine how your bones, liver and spleen look.

You may have all, some or none of these tests on any particular visit. You may also be asked about any other medications you've been taking, and whether you've noticed any new or changing symptoms or side effects.

If you develop symptoms or side effects that you are finding hard to manage, get in contact with your specialist team for advice – you may not have to wait until your next official appointment to get help.





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