

# Cerdelga (eliglustat) FAQs



**Cerdelga**<sup>®</sup>  
(eliglustat) capsules

## **What is Cerdelga?**

Cerdelga is a new tablet for the long term treatment of adult patients with Gaucher Disease type 1. It has been approved for use by the European medicines agency and it is now available from the NHS for patients in England.

## **Can I take Cerdelga?**

Cerdelga can be used by most type 1 Gaucher patients if your specialist thinks this is the right treatment for you. There are a few additional important considerations –CYP2D6 status, other medication, pregnancy and diet.

## **What is CYP2D6 status?**

CYP2D6 is an enzyme in your liver that helps breaks down Cerdelga. The genetically determined activity of this enzyme will help decide what dose of Cerdelga is right for you. A small number of people have a form of CYP2D6 that breaks down the medication so fast that it will not work (known as “ultra-rapid metabolisers”), and currently Cerdelga is not recommended for these people. Another small group of people breakdown the drug so slowly that they need a less frequent dose, once per day, (known as “poor metabolisers”). Everyone else will need a twice daily dose (described as either “extensive “or “intermediate metabolisers”).

## **How is CYP2D6 status tested?**

CYP2D6 status is identified by a simple genetic blood test available from your specialist centre. You will not be eligible for treatment with Cerdelga without this blood test result.

## **What medication can I take with Cerdelga?**

Many other drugs are also broken down by the CYP2D6 and other related liver enzymes. This means that not all drugs can be taken whilst receiving treatment with Cerdelga. There are many complex ways in which drugs can interact with Cerdelga and you are advised to discuss your current medication (including over the counter preparations and herbal supplements/complementary therapies) with your specialist before considering starting treatment. Some drugs, including Cerdelga, potentially affect the electrical activity of the heart. For this reason you should have an ECG (heart tracing) before you start treatment. Once taking Cerdelga regularly, it is important to seek medical advice before starting any new medication. You may be provided with an alert card to carry which you can show to other healthcare professionals if you are unwell.

### **Can I take Cerdelga if I am pregnant?**

There is very little information about the safety of Cerdelga in pregnancy, therefore it is currently recommended to avoid Cerdelga during pregnancy. If you are considering pregnancy it is advisable to tell your specialist team, particularly when reviewing the treatment options available to you.

### **Do I have to follow any special diet?**

Unlike previous tablet treatments for Gaucher, Cerdelga does not require you to follow any special diet. However you should avoid grapefruit/grapefruit juice as this is broken down by some of the same liver enzymes as Cerdelga.

### **How do I take Cerdelga?**

Cerdelga is a tablet that needs to be taken regularly once or twice per day as recommended by your specialist. It comes in blister packs of 14 hard capsules. It doesn't matter whether you take it with or without food. Do follow the instructions in the tablet box and take the tablets regularly as prescribed. If you miss a dose, just continue again at the next scheduled time – do not take an extra capsule.

### **How will life be different on Cerdelga?**

Cerdelga is a first line treatment for Gaucher disease and therefore you are unlikely to need to continue treatment with enzyme replacement therapy. You will still receive medication deliveries from your homecare company but this is likely to only be once every 8 weeks. The drug needs to be stored safely in a cupboard at room temperature – no drug fridge is required. You will still need to attend your specialist centre at least once or twice per year as before to ensure the treatment is effective for you. You may be advised to carry an alert card notifying healthcare professionals of your Cerdelga therapy. You will be able to take Cerdelga away with you on holidays or when travelling.

### **Are there any side effects with Cerdelga?**

Side effects are commonly reported with many drugs and are not always directly related. The majority of side effects reported in the Cerdelga clinical trials were mild and short-lived. The side effects reported during more than 4 years of clinical trials were: tiredness, headache, gastrointestinal upset, joint pain and occasional fainting.

### **What if Cerdelga doesn't work for me?**

If you find that Cerdelga does not suit you for whatever reason, you may be advised to return to receiving enzyme replacement therapy.

### **What if I need more information?**

For further information regarding whether you can receive treatment with Cerdelga please contact your Specialist Clinical team. For general information about Cerdelga there is a helpful US website <http://www.cerdelga.com/> Further UK specific information is currently in development.

This information has been written independently by medical professionals on the request of The Gauchers Association