Information for patients and the public: Exagamglogene autotemcel (exa-cel / Casgevy) for severe sickle cell

March 2025

Introduction

In January 2025, the National Institute of Health Excellence (NICE) approved Casgevy, a new gene therapy, for use in the NHS to treat people who live with severe sickle cell.

Casgevy (generic name exa-cel) is a new treatment to treat severe sickle cell in people aged 12 and over who do not have a matched donor for stem cell transplant, but who would have been well enough to tolerate a transplant. If successful, treatment with Casgevy effectively provides a cure for sickle cell. It is the first gene therapy available to treat sickle cell in the UK. Casgevy is also approved by NICE to treat transfusion-dependent β -thalassemia.

Sickle Cell Society and Anthony Nolan are grateful for all your support throughout the NICE appraisal of Casgevy as a new option for patients, and to make this a reality. We have jointly, and with clinical experts, pulled together key information that we hope will support you to understand Casgevy and will help you to have a conversation with your consultant about accessing this treatment.

Key Information & Frequently Asked Questions

Medical treatment process & access to Casgevy

Who is eligible to receive Casgevy?

Casgevy will only be available to a limited number of people because this is what has been shown to be safe and effective in clinical trials so far. To be able to access Casgevy, you will need to meet the following eligibility criteria:

- Be 12 years old and over
- Have had at least two vaso-occlusive crises, often known as “pain crises” per year during the last two years
- Have specific genotypes: βS /βS, βS /β+ or βS /β0
- A donor stem cell transplant would be appropriate (i.e., be fit enough for a stem cell transplant)
- You don’t have a well-matched stem cell donor

How do you know if you are eligible?

The best way to find out if you can access Casgevy is to talk to your sickle cell consultant. If you don't have a consultant who knows you well, you can also talk to the senior sickle cell nurse in your local treatment centre who you might know better.

If your consultant thinks you could be eligible, they will then put your forward to a national panel who will consider your case and discuss whether they think it will be safe for you to undergo this treatment.

Once you have expressed an interest in the treatment, you should be provided with more information and materials about the treatment process. It's a good idea to really think about if this is right for you and your family, and to have an open discussion with your medical team just like you would if you were considering a stem cell transplant.

How does Casgevy work?

To make Casgevy, stem cells are edited (by CRISPR/Cas9 technology) to produce more foetal haemoglobin, a type of haemoglobin mainly found in babies in the womb but also present in small amounts in adults. This foetal haemoglobin does not have the "sickle" red blood cell shape, and can therefore compensate for the lack of normal adult haemoglobin within the patient.

As a result, the modified stem cells, when injected into patients, can raise red blood cell levels in patients with beta-thalassaemia and prevent painful crises in patients with sickle cell disease.[1]

What is the treatment process?

The treatment process for Casgevy is not too different from going through a stem cell transplant including the need for conditioning medicines. These medicines are similar to undergoing chemotherapy and help your body take up the new Casgevy modified stem cells.

After receiving conditioning medicine, it may not be possible for you to become pregnant or father a child. **You should discuss options for fertility preservation with your healthcare provider before treatment.**

The treatment process is outlined below[2]:

Step 1: You stem cells will be collected using an apheresis machine at the hospital. It could take more than one visit to collect the amount needed.

Step 2: Your blood stem cells are then sent to a manufacturing site and used to make Casgevy. This step can take up to 6 months, as they need to be made and then tested before being sent back to the hospital. Once these have been received by your clinical team, you will be informed.

Step 3: You undergo conditioning treatment, similar to chemotherapy, in hospital. This clears your cells from the bone marrow so they can be replaced with modified Casgevy stem cells. You will remain in hospital once you have received your conditioning treatment and go onto the next step.

Step 4: You are transfused with your Casgevy modified stem cells, these are given to you via intravenous infusion (through your vein).

Step 5: You stay in hospital after infusion for about 4-6 weeks, and one or more vials of Casgevy stem cells will be given to you over a short period of time.

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[1] https://www.ema.europa.eu/en/medicines/human/EPAR/casgevy#product-details

Where is Casgevy available?

Casgevy is only available in a limited number of NHS specialist hospitals at the moment. This is because it is a complicated treatment, and the NHS needs to make sure the hospitals have the right equipment and people in place to treat sickle cell patients with Casgevy.

Currently there are four hospitals where Casgevy is available, and three more hospitals will be able to deliver it soon. Over time, the NHS will see how many people are being treated and increase capacity if needed in line with this new information. So far, the hospitals which will treat people with Casgevy include:

1. Barts Health NHS Trust (London)
2. Imperial College Healthcare NHS Trust (London)
3. University College London Hospitals NHS Foundation Trust (London)
4. Manchester University NHS Foundation Trust (Manchester)

About the NICE recommendation & Casgevy trial data

About NICE

The National Institute for Health & Care Excellence (NICE) is the independent public body that recommends whether or not new treatments should be funded by the NHS in England as a cost-effective use of NHS funds. Its decisions are usually also accepted by the NHS in Wales and Northern Ireland. Decisions about new medicines are made separately in Scotland, by the Scottish Medicines Consortium (SMC). The SMC is currently considering the use of Casgevy in Scotland for thalassaemia and plans to consider it for sickle cell shortly.

All information about the NICE recommendation for Casgevy is available publicly on the NICE website here: **https://www.nice.org.uk/guidance/indevelopment/gid-ta11249**

What is a "managed access scheme"?

A managed access scheme means that a new treatment is available through the NHS for a limited time only while further data is being collected about the impact of the treatment in real life outside of the controlled environment of a clinical trial.

The scheme means that Casgevy will be available for the next 5 years on the NHS. When the 5 years has ended, NICE, NHS England and the manufacturer will review the new data to see if the treatment is working as expected or if there are any problems. At this point NICE will decide whether it is beneficial to keep funding the treatment indefinitely through the NHS, and there would be no further review planned.

How effective is Casgevy?

The clinical trials for Casgevy were conducted on people aged between 12 and 35 years old, and are still ongoing. The trials found[2]:

- 28 out of 29 people did not have a severe or painful vaso-occlusive crisis for at least 12 months in a row after receiving Casgevy.
- 29 out of 29 people did not require hospitalisation for any painful crises for at least 12 months.
- People had increased levels of haemoglobin after Casgevy transfusion that stayed steady over time, for the period of the trial.

There are also trials ongoing for children aged 12 years old and under, so in the future Casgevy may be available for this age group too.

[2] https://www.casgevy.com/sickle-cell-disease/study-information

Why do we need to collect more data?

Additional data collection will help address the uncertainties there currently are in the clinical trial for Casgevy. We need more information about the long-term impacts of Casgevy as it is a very new treatment and people who were treated in the clinical trial have not had the treatment for long enough to tell us about the impact in, for example, 5-10 years. Therefore, more follow-up with patients needs to be done.

The NICE committee think the additional long-term follow up data will help us to understand more about:

- If people return to full or near full health, and for how long this lasts before people might need additional follow-up treatments
- If there are any long-term complications either relating to Casgevy itself, or the return of sickle cell symptoms
- To find out which types of people are more likely to be considered by clinicians as being eligible, and for which types of people the treatment works the best for, i.e., looking at age, sex, any prior complications or comorbidities

Where can I get more information about sickle cell or the transplant process?

Anthony Nolan helpline

Call the team on 0303 303 0303 or email **patientinfo@anthonynolan.org**
Our helpline is open 9am to 5pm, Monday to Friday (excluding bank holidays).

You can also read more about having a stem cell transplant on
https://www.anthonynolan.org/patients-and-families/having-a-stem-cell-transplant

The Sickle Cell Society

Visit the website on **www.sicklecellsociety.org** or find a contact on
www.sicklecellsociety.org/contact-details/

The Casgevy website for Sickle Cell

Visit the website on **https://www.casgevy.com/sickle-cell-disease**

You will be able to find lots more information to help inform your decision and help you to have conversations with your clinical team.