



## Information for patients and the public: NICE appraisal of exagamglogene autotemcel (exa-cel) for sickle cell

March 2024

### Background

Exa-cel is a new gene therapy for the treatment of sickle cell disorder. You may have seen it in the news under the name of 'Casgevy'.

The National Institute for Health and Care Excellence (NICE) is considering whether to fund this treatment on the NHS in England.

On 14 March NICE published a draft decision not to recommend exa-cel for NHS funding. This document provides more information on this decision and on next steps. We're working together with the Anthony Nolan charity on this, to combine resources and increase our impact.

**The key action for members of the patient and clinical community is to respond to the NICE consultation which is open until 11 April 2024. [You can respond on this webpage](#) or email your comments to [info@sicklecellsociety.org](mailto:info@sicklecellsociety.org) or [public.affairs@anthonymolan.org](mailto:public.affairs@anthonymolan.org).**

It is very important that NICE has the opportunity to hear from as many voices in the community as possible ahead of their next meeting to discuss exa-cel. The key things to share with NICE are:

- The negative impact that severe sickle cell has on the day to day life of people living with the condition
- The worsening impact that sickle cell has over time
- The impact of sickle cell on life expectancy and the risk of mortality

### Key information and Frequently Asked Questions

#### About exa-cel

Exa-cel (brand name Casgevy) is a new treatment which is licensed by the MHRA to treat severe sickle cell in people aged 12 and over who do not have a matched sibling donor for stem cell transplant, but who would have been well enough to tolerate a transplant.

Sickle Cell Society 54 Station Road London NW10 4UA

020 8961 8346

[info@sicklecellsociety.org](mailto:info@sicklecellsociety.org)

Charity no. 1046631

#### Anthony Nolan

2-3 Heathgate Place,  
75-87 Agincourt Road,  
London NW3 2NU

[anthonymolan.org](http://anthonymolan.org)

0303 303 0303





If successful, treatment with exa-cel effectively provides a cure for sickle cell. It would be the first gene therapy available to treat sickle cell in the UK.

Exa-cel is manufactured by the pharmaceutical company Vertex. Anthony Nolan and the Sickle Cell Society are not privy to the proposed cost of exa-cel to the NHS, but it is likely to be one of the most expensive medicines ever considered by NICE.

### **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).

### **About the draft NICE decision**

On 14 March NICE published a draft decision not to recommend exa-cel for use in sickle cell. The draft decision is available to view [here](#).

NICE have said that, based on the information provided to them by Vertex and clinical and patient experts so far, exa-cel would not be a cost-effective use of NHS resources. They have also said that there is too much uncertainty in the evidence about exa-cel they have received. In particular, NICE are not certain about the severity of sickle cell or the magnitude of the long-term benefits to patients of exa-cel.

### **Is this a final decision?**

No, this is not a final decision. NICE has opened a public consultation to seek the public's views on the draft decision. The consultation is open until 11 April 2024 and can be accessed here:

<https://www.nice.org.uk/guidance/indevelopment/gid-ta11249/consultation/html-content-5>.

Once the consultation closes, NICE will meet again and will then issue its final decision. The date for a final decision is as yet unknown.

**Sickle Cell Society 54 Station Road London NW10 4UA**

**020 8961 8346**

**[info@sicklecellsociety.org](mailto:info@sicklecellsociety.org)**

**Charity no. 1046631**

**Anthony Nolan**  
2-3 Heathgate Place,  
75-87 Agincourt Road,  
London NW3 2NU

**anthohnolan.org**  
0303 303 0303



Anthony Nolan is a registered charity no 803716/SC038827 and registered as a limited company no 2379280 in England and Wales. Registered address: Royal Free Hospital, Pond Street, London NW3 2QG.





Saving lives through stem cells

### What do we need to do next?

Anthony Nolan and the Sickle Cell Society are asking sickle cell patients, families, clinicians and anyone with experience of sickle cell to share their views on making exa-cel available to the NHS.

### How can I respond to the consultation?

You can respond directly at this link: <https://www.nice.org.uk/guidance/indevelopment/gid-ta11249/consultation/html-content-5>

You'll need to create a NICE account to respond.

This video from NICE shows how to use their online commenting system:

<https://www.youtube.com/watch?v=DILcZkxVPCY>

If you are unable to respond you can send your comments directly to [info@sicklecellsociety.org](mailto:info@sicklecellsociety.org) or [public.affairs@anthohnolan.org](mailto:public.affairs@anthohnolan.org) and we will send them on to NICE on your behalf.

In your response, please consider including your views on:

- The negative impact that severe sickle cell has on the day to day life of people living with the condition
- The worsening impact that sickle cell has over time
- The impact of sickle cell on life expectancy and the risk of mortality

### How does this relate to other NICE decisions about exa-cel?

NICE is also considering exa-cel for the treatment of transfusion-dependent beta thalassaemia.

On 6 March NICE announced that Vertex and NHS England have entered into commercial and managed access discussions for exa-cel in this indication.

The final NICE recommendation will depend on the outcome of those negotiations. The NICE decision on thalassaemia is being made separately from the one on sickle cell, although it is the same committee considering both indications. More information about the thalassaemia appraisal is available here

<https://www.nice.org.uk/guidance/indevelopment/gid-ta11250>.

**Sickle Cell Society 54 Station Road London NW10 4UA**

**020 8961 8346**

**[info@sicklecellsociety.org](mailto:info@sicklecellsociety.org)**

**Charity no. 1046631**

#### Anthony Nolan

2-3 Heathgate Place,  
75-87 Agincourt Road,  
London NW3 2NU

[anthohnolan.org](http://anthohnolan.org)

0303 303 0303



Anthony Nolan is a registered charity no 803716/SC038827 and registered as a limited company no 2379280 in England and Wales. Registered address: Royal Free Hospital, Pond Street, London NW3 2QG.



Registered with  
FUNDRAISING  
REGULATOR





## Where can I get more information about gene therapy and stem cell transplant for sickle cell?

### The Sickle Cell Society

Call the helpline on 020 8963 7794 or [helpline@sicklecellsociety.org](mailto:helpline@sicklecellsociety.org)

For more information about sickle cell disorder and treatments, visit the website on [www.sicklecellsociety.org](http://www.sicklecellsociety.org)

### Anthony Nolan helpline

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

You can also read more about sickle cell and stem cell transplant [here](#).

Sickle Cell Society 54 Station Road London NW10 4UA  
020 8961 8346  
[info@sicklecellsociety.org](mailto:info@sicklecellsociety.org)  
Charity no. 1046631

**Anthony Nolan**  
2-3 Heathgate Place,  
75-87 Agincourt Road,  
London NW3 2NU

[anthonymolan.org](http://anthonymolan.org)  
0303 303 0303



Anthony Nolan is a registered charity no 803716/SC038827 and registered as a limited company no 2379280 in England and Wales. Registered address: Royal Free Hospital, Pond Street, London NW3 2QG.

