Information for patients and the public: NICE appraisal of Exagamglogene autotemcel (Exa-cel/ Casgevy) for beta-thalassaemia

August 2024

Background

Today, Exa-cel (Casgevy) has been approved by NICE to treat transfusion-dependent beta-thalassaemia in people aged 12 years and over.

Exa-cel is a new gene therapy that has been going through the medicine appraisal process for the treatment of sickle cell disorder and transfusion-dependent beta-thalassaemia. The National Institute for Health and Care Excellence (NICE) has been considering whether to fund this treatment on the NHS in England.

The NICE decision on thalassaemia was made separately from the one on sickle cell, although it is the same committee considering the therapy for both of these conditions. The manufacturer of the therapy ; Vertex, NHS England and NICE remain in discussion on the approval of Exa-cel to treat sickle cell.

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 and transfusion-dependent β-thalassaemia 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 Exa-cel effectively provides a cure for sickle cell or thalassaemia. It would be the first gene therapy available to treat sickle cell in the UK, and is now the first gene therapy to treat beta-thalassaemia in the UK.

Exa-cel is manufactured by the pharmaceutical company Vertex. The “list price” to the NHS of exa-cel is £1,651,000 per course of treatment, although it is our understanding that a confidential discounted price has been agreed between Vertex and the NHS. It is likely to be one of the most expensive medicines ever considered by NICE.

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About the NICE decision for beta-thalassemia

NICE's final draft guidance published today (8 August 2024) recommends Exagamglogene autotemcel ("Exa-cel", also called Casgevy and made by Vertex), the world's first CRISPR-based gene therapy and also the first gene therapy available in Europe for treating transfusion-dependent beta thalassaemia.

Exa-cel is recommended for use in the Innovative Medicines Fund (IMF) as an option for treating transfusion-dependent beta-thalassaemia in people 12 years and over when a haematopoietic stem cell transplant (sometimes called a bone marrow transplant) is suitable but no donor is available.

Its use in the IMF allows more data about Exa-cel's clinical and cost-effectiveness to be collected. It is expected that the treatment will start to be delivered in the next few weeks and that around 460 people with thalassaemia in England and Wales are eligible for the treatment.

This is a positive outcome that we hope will help influence the decision NICE makes regarding the use of Exa-cel to treat sickle cell.

More information about the thalassaemia appraisal is available here: https://www.nice.org.uk/guidance/indevelopment/gid-ta11250.

About the draft NICE decision for sickle cell

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.

However, a consultation was opened to gather further information on the impacts of sickle cell on patients, which you helped contribute to in April 2024. The NICE committee then met again to discuss these responses, and remain in negotiations with Vertex and NHS England to spend time to understand the data and new information.

The date for a final decision on sickle cell is as yet unknown, and we will update you when further details are available.

We wanted to reassure you that we continue to work with NICE, Vertex and NHS England to advocate on your behalf to help get exa-cel approved.

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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 beta-thalassemia

In the UK beta thalassaemia mainly affects people of Pakistani, Indian and Bangladeshi ethnic origin. It is an inherited blood disorder caused by a genetic mutation that reduces or prevents production of healthy red blood cells and haemoglobin (the protein found in red blood cells that carries oxygen around the body).

People with the most severe type of beta thalassaemia need regular blood transfusions. Transfusion-dependent beta-thalassaemia can cause delayed growth, bone problems, problems with endocrine development and affect quality and length of life. It has significant effect on work, family and friends, and the intense blood transfusions have associated side effects and complications.

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

The Sickle Cell Society

Visit the website on www.sicklecellsociety.org

Call the helpline on 020 8963 7794 or helpline@sicklecellsociety.org Monday-Friday 10am – 5pm

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 sickle cell and stem cell transplant here.

public.affairs@anthonynolan.org or info@sicklecellsociety.org

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