NICE National Institute for Health and Care Excellence

Resource impact summary

Resource impact
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Resource impact summary report

This summary report is based on the NICE assumptions used in the resource impact template. Users can amend the 'Inputs and eligible population' and 'Unit costs' worksheets in the template to reflect local data and assumptions.

Recommendation

NICE has recommended exagamglogene autotemcel (exa-cel) with managed access as an option for treating sickle cell disease (SCD) in people 12 years and over:

- who have:
  - recurrent vaso-occlusive crises (VOCs) and
  - a β^S/β^S, β^S/β^+ or β^S/β^0 genotype, and
- when haematopoietic stem cell transplant (HSCT) is suitable, but a human leukocyte antigen-matched related haematopoietic stem cell donor is not available.

It is only recommended:

- for people who have had at least 2 VOCs (as defined in section 3.4 of the guidance) per year during the 2 previous years and
- if the conditions in the managed access agreement for exa-cel are followed.

Eligible population for exa-cel

Table 1 shows the population who are eligible for exa-cel and the number of people who are expected to have exa-cel in each of the next 5 years.

Table 1 Population expected to be eligible for and have exa-cel in England

Eligible population and uptake	Current practice	2025-26	2026-27	2027-28	2028-29	2029-30
People eligible for exa-cel	1,794	1,811	1,829	1,846	1,864	1,882

Eligible population and uptake	Current practice	2025-26	2026-27	2027-28	2028-29	2029-30
People selected for treatment each year (intention to treat population)	0	29	72	90	96	96
Percentage of people who complete treatment	81%	81%	81%	81%	81%	81%
People who will complete treatment	0	23	58	73	78	78

Abbreviations: Exa-cel, exagamglogene autotemcel.

The following assumptions have been used to calculate the eligible population:

- approximately 70% of people with sickle cell disease have the genotypes β^S/β^S, β^S/β^+ or β^S/β^0
- 48% of people with genotypes β^S/β^S, β^S/β^+ or β^S/β^0 have at least 2 VOCs per year during the 2 previous years
- 54% are fit for the procedure as per the company submission, but people may want to review this assumption locally
- 85% do not have a human leukocyte antigen-matched related haematopoietic stem cell donor available.

The market share for exa-cel is based on NICE estimates and is for illustrative purposes. A low market share is anticipated because a lengthy hospital stay is needed for the process involved. NHS organisations should review the estimates and amend locally.

Treatment options for the eligible population

Standard care for SCD includes hydroxycarbamide, blood transfusions and iron chelation therapy to remove excess iron in the blood. People who are well enough can have an HSCT if available. When an HSCT is suitable but there is no available human leukocyte antigen-matched donor, exa-cel is a potential cure.

The company submission assumes that red blood cell transfusions are typically given every 6 weeks, equating to 8.7 times per year. Of the treated population, it is estimated that 16% will be having red blood cell transfusions and that 64% will be having hydroxycarbamide.

The exa-cel treatment process involves collecting blood stem cells from the person having exa-cel and sending them to a manufacturing facility. There, the CD34+ cells are isolated. Then, the CRISPR associated protein 9 is used to edit the BCL11A gene before the cells are frozen. The edited cells are returned to the body in a single infusion. This involves several steps needing healthcare resource use.

For more information about the treatments, such as dose and average treatment duration, see the resource impact template.

Financial resource impact (cash items)

The company has a commercial arrangement. This makes exa-cel available to the NHS with a discount.

Users can input the confidential price of exa-cel and amend other variables in the resource impact template.

The payment mechanism for the technology is determined by the responsible commissioner and depends on the technology being classified as high cost.

For further analysis or to calculate the financial impact of cash items, see the resource impact template.

Capacity impact

While there are additional treatment costs and significant capacity impacts for people having exa-cel, these are experienced over the year of treatment. These are alleviated by the longer-term benefits from reduced need for regular transfusions. This can be assessed in the local resource impact template. Additionally, a reduction in VOC events will reduce hospital admissions and reduce healthcare resource use.

Table 2 shows the impact on capacity activity in each of the next 5 years based on NICE estimates for people taking up exa-cel.

Table 2 Capacity impact (activity) in England

Capacity impact	Current practice	2025-26	2026-27	2027-28	2028-29	2029-30
Mobilisation administrations (hospital bed days)	0	493	1,225	1,531	1,633	1,633
Post-treatment hospital stay (hospital bed days)	0	658	1,633	2,041	2,177	2,177
Blood transfusions avoided from people having transfusion independence	0	0	(33)	(147)	(362)	(686)

For further analysis or to calculate the financial capacity impact from a commissioner (national) and provider (local) perspective, see the resource impact template.

Key information

Table 3 Key information

Time from publication to routine commissioning funding	Treatment is available according to the conditions in the managed access agreement.
Programme budgeting category	3 'Disorders of Blood'
Commissioner(s)	NHS England
Provider(s)	Secondary care - acute. Limited to authorised providers only
Pathway position	Sickle cell disease with recurrent vaso-occlusive crises and a β^S/β^S, β^S/β^+ or β^S/β^0 genotype when haematopoietic stem cell transplant is suitable, but a human leukocyte antigen-matched related haematopoietic stem cell donor is not available.

About this resource impact summary report

This resource impact summary report accompanies the NICE technology appraisal on exagamglogene autotemcel for treating sickle cell disease and should be read with it.

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