NICE National Institute for Health and Care Excellence

Resource impact summary report

Resource impact
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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 transfusion-dependent beta-thalassaemia in people 12 years and over:

- when a haematopoietic stem cell transplant (HSCT) is suitable, but a human leukocyte antigen-matched related haematopoietic stem cell donor is not available
- only if the conditions in the managed access agreement for exa-cel are followed.

This recommendation is not intended to affect treatment with exa-cel that was started in the NHS before this guidance was published. People having treatment outside this recommendation may continue without change to the funding arrangements in place for them before this guidance was published, until they and their NHS healthcare professional consider it appropriate to stop. For children or young people, this decision should be made jointly by the healthcare professional, the child or young person, and their parents or carers.

Eligible population for exa-cel

Table 1 shows the population eligible for exa-cel and the number of people expected to have exa-cel in each of the next 5 years (based on NICE estimates), including population growth.

Table 1 Population expected to be eligible for and have exa-cel in England (NICE estimates)

Eligible population and uptake	Current practice	2024 to 2025	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029
People eligible for exa-cel	475	480	484	489	494	498

Eligible population and uptake	Current practice	2024 to 2025	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029
Market share for exa-cel (%)	0	3	5	7	9	11
People having treatment each year (change in number of people)	0	14	10	10	10	10

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

- 85.00% of people do not have a human leukocyte antigen-matched related haematopoietic stem cell donor available
- 62.15% of people aged over 18 years have regular transfusions

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 beta-thalassaemia includes blood transfusions and iron chelation therapy to remove excess iron in the blood. If people who have regular blood transfusions are well enough, an HSCT is an option. Exa-cel is a possible cure when an HSCT is suitable but there is no human leukocyte antigen-matched stem cell donor.

The patient and clinical experts from the committee commented that red blood cell transfusions are typically given every 3 to 4 weeks, equating to between 13.0 and 17.3 per year. A midpoint of 15 blood transfusions per year for people having standard care is used in the resource impact template.

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.

Evidence from an indirect comparison showed that exa-cel reduced the need for transfusions compared with standard care. In the long term, the resource benefits of this could be considerable when compared with the number of years people have regular

blood transfusions, and the associated capacity impact avoided.

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

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 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.

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 Key capacity impacts (activity) in England using NICE uptake estimates

Capacity impact	Current practice (without exa-cel)	2024 to 2025	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029
Mobilisation administrations (hospital bed days)	0	345	236	240	245	249
Post-treatment hospital stay (hospital bed days)	0	403	275	280	286	291

Capacity impact	Current practice (without exa-cel)	2024 to 2025	2025 to 2026	2026 to 2027	2027 to 2028	2028 to 2029
Appointments with specialist (for stem-cell harvesting, treatment infusion and follow up)	0	113	150	207	264	322
Blood transfusions avoided from people having transfusion independence	0	0	0	(137)	(139)	(142)

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)	Authorised treatment centres
Pathway position	When a 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 NICE's technology appraisal guidance on exagamglogene autotemcel for treating transfusion-dependent beta-thalassaemia in people 12 years and over and should be read with it. See terms and conditions on the NICE website.

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