



Exagamglogene autotemcel for treating transfusion-dependent beta-thalassaemia in people 12 years and over

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Exagamglogene autotemcel (exa-cel; Casgevy) is available during the <u>managed access</u> period. It is a possible treatment for transfusion-dependent beta-thalassaemia in people 12 years and over:

- when a haematopoietic stem cell transplant (a blood and marrow transplant) is suitable, but
- a human leukocyte antigen-matched related haematopoietic stem cell donor is not available.

More evidence on exa-cel is being collected. After this, NICE will decide whether to recommend exa-cel for routine use on the NHS and update the guidance. It will be available with managed access until then.

Is this treatment right for me?

Your healthcare professionals should give you clear information, talk with you about your options and listen carefully to your views and concerns. Your family can be involved too, if you wish. See <u>our webpage on making decisions about your care</u>.

Questions to think about

- How well does it work compared with other treatments?
- What are the risks or side effects? How likely are they?
- How will the treatment affect my day-to-day life?
- What happens if the treatment does not work?
- What happens if I do not want to have treatment? Are there other treatments available?

Information and support

The NHS webpage on thalassaemia may be a good place to find out more.

These organisations can give you advice and support:

- Anthony Nolan, 0303303 0303
- UK Thalassaemia Society, 020 8882 0011

You can also get support from your local Healthwatch.

NICE is not responsible for the quality or accuracy of any information or advice provided by these organisations.

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