8th August 2024

UKTS Press release

Outcome of National Institute for Health and Care Excellence (NICE) Technology Appraisal for Exagamglogene autotemcel (Exa-cel) for treating transfusion-dependent beta thalassaemia [ID4015] in individuals 12 years and over.

To our thalassaemia community,

We are delighted to announce that the NICE committee has taken the decision to recommend that Exa-cel (Gene Therapy) can be offered as a treatment option for individuals 12 and over living with transfusion dependent thalassaemia under the Managed Access Agreement. It is estimated by NHS England that approximately 600 individuals could be eligible for this treatment.

This announcement is a major breakthrough for the future of thalassaemia treatment following more than 20 years of research in the field. We would like to thank everyone who has worked tirelessly to make this a positive result.

UKTS and the thalassaemia community were represented by experts; Dr. Clare Samuelson, Dr. Emma Drasar, Romaine Maharaj, Gabriel Theophanous and Roanna Maharaj.

Dr. Clare Samuelson (Consultant Haematologist) said "*This is excellent news for patients with thalassaemia and their families, who have waited a very long time to have access to an innovative curative therapy. We now need to ensure that this novel treatment is available to all patients who will benefit from it.*"

Romaine Maharaj (UKTS) Executive Director said " *The availability of gene therapy via the NHS managed access agreement for individuals living with thalassaemia is a monumental milestone for our community. We are pleased that the NICE committee recognised how debilitating and challenging living with thalassaemia can be for individuals and their families. Exa-cel has the potential to transform lives by eliminating the need for regular transfusions and to slow down or reduce the development of secondary conditions. This is yet another victory in our ongoing battle to secure treatment options for all individuals living with thalassaemia, enabling them to live fuller, healthier and happier lives.*"

What does this mean for you?

To access this treatment, NICE has set out a strict criterion.

- when a haematopoietic stem cell transplant (HSCT) is suitable, but a human leukocyte antigen-matched related haematopoietic stem cell donor is not available (**i.e. if you are healthy enough to undergo a HSCT / Bone Marrow transplant but do not have a matched donor**).

- only if the conditions in the managed access agreement for exa-cel are followed.

If you would like to find out if this treatment option is suitable for you or a family member, we advise you to please contact your thalassaemia team.

If you would like to find out more about the general process of gene therapy we have two resources for you.

An article on the mechanisms of Exa-cel which can be accessed here: https://ukts.org/clinical-trails/gene-home/gene-editing-2/

A webinar by our Clinical Expert; Dr. Clare Samuelson which can be accessed here: https://www.youtube.com/watch?v=L4URSnay_zc

How will Exa-cel be funded?

Exa-cel will be the first of it's kind for thalassaemia to enter the NHS England's Innovative Medicine Fund (IMF), following a commercial deal being reached between NHS England and the treatment's manufacturer, Vertex Pharmaceuticals.

Through the IMF, Exa-cel will be funded by the NHS and offered to patients within months at seven specialist centres in England (London, Birmingham and Manchester). NHS England have committed to looking at the demand for this treatment a year after access becomes available to determine if more centres should be invited to offer treatment. However, if there is a greater demand in less than a year after the guidance has been issued, NHS England will review sooner.

Please note: Exa-cel has not been recommended for use in routine commissioning. According to NICE in its final draft guidance, " *This is because of the uncertainty in exa-cel's long-term effects and impact on quality of life, and in the outcomes for people on standard care. Some of the most likely cost-effectiveness estimates are higher than what NICE normally considers an acceptable use of NHS resources, even when accounting for exa-cel's potential impact on health inequalities. So, exa-cel is not recommended for routine use in the NHS.*
Collecting more data through a managed access agreement may resolve some uncertainty in the evidence. So, exa-cel is only recommended for use with managed access."

The managed access agreement will run for a period of up to 4 years to gather the various data highlighted by the committee. It is expected that the relevant data will be submitted to NICE in the summer of 2028.

If you would like to read NICE'S Final Draft Guidance (FDG) and/ or other NICE related documents please follow this link:
https://www.nice.org.uk/guidance/indevelopment/gid-ta11250/documents