FOI 23/897

Dear

Thank you for your request for information dated 17 November 2023, where you asked:

> *"[…] transfusion dependent thalassemia major. I wanted to know when this treatment will be available and made available to a wider audience. Whether it will be possible to access it also in Italy or only in England and what it will cost. […]"*

Our response:

Casgevy 4-13 × 10^6 cells/mL dispersion for infusion was authorised by the MHRA on 15 November 2023, and is indicated (can be used in the treatment of):

"Transfusion-dependent β-thalassemia
Transfusion-dependent β-thalassemia Casgevy is indicated for the treatment of transfusion-dependent β-thalassemia in patients 12 years of age and older for whom haematopoietic stem cell transplantation is appropriate and a human leukocyte antigen matched related haematopoietic stem cell donor is not available. Sickle cell disease Casgevy is indicated for the treatment of sickle cell disease in patients 12 years of age and older with recurrent vasoocclusive crises who have the βS /βS , βS /β+ or βS /β0 genotype, for whom haematopoeitic stem cell transplantation is appropriate and a human leukocyte antigen matched related haematopoietic stem cell donor is not available.

Sickle cell disease (**another indication for the same product**)
Sickle cell disease Casgevy is indicated for the treatment of sickle cell disease in patients 12 years of age and older with recurrent vasoocclusive crises who have the βS /βS , βS /β+ or βS /β0 genotype, for whom haematopoeitic stem cell transplantation is appropriate and a human leukocyte antigen matched related haematopoietic stem cell donor is not available."

In terms of your questions related to when the treatment will be available or if it will be available to a wider audience, this is beyond the MHRA's remit. The Medicines and Healthcare products Regulatory Agency (MHRA) is responsible for regulating all medicines and medical devices in the UK by ensuring they work and are acceptably safe. This means that when we approve a medicine such as Casgey, we issue a marketing authorisation that grants a company the right to sell the product in the UK or in individual UK territories. That company and other UK healthcare bodies (such as NICE and the NHS) may then collaborate to discuss availability on the NHS to patients.

In terms of your questions related to if access will also be possible in Italy or only England, and the cost, we can only comment that this conditional marketing authorisation was for Great Britain only. The European Medicine Agency (EMA) may be able to confirm if they hold an application for this product to authorise, for

example, Casgevy in all European member states. Alternatively, the product could become authorised on a national basis in Italy via an application to the national competent authority (regulator of medicines). Please find the contact details for the EMA and AIFA below.

EMA contact details:
Send a question to the European Medicines Agency | European Medicines Agency (europa.eu)

Contacts | Italian Medicines Agency (aifa.gov.it)

Some further general information on access to medicines.
Access to medicinal products | Italian Medicines Agency (aifa.gov.it)

Relevant to the UK only
The National Institute for Health and Care Excellence (NICE) provides guidance to the NHS in England on the clinical and cost effectiveness of selected new and established technologies. NICE carries out appraisals of health technologies at the request of the Department of Health and Social Care. In summary, NICE suggest which treatments should be available on the NHS in England by examining the evidence for each medicine. They may be able to advise on Casgevy at the following NICE contact details:
Contact us | Get involved | NICE

The company that markets Casgevy (Vertex Pharmaceuticals (Europe) Ltd, and CRISPR therapeutics) could also be approached as they may wish to share their future intentions in terms of marketing their product.

In terms of some further information about the product, the patient information leaflet is available here.

We trust that you will have found this information of use. However, if you disagree with how we have interpreted the Freedom of Information Act 2000 in answering your request, you can ask us to review our actions and decisions by writing to: info@mhra.gov.uk, and requesting an internal review.

Please note that your internal review request must be in a recordable format (email, letter, audio tape etc.), and that you have 40 working days upon receipt of this letter to ask for a review. We aim to provide a full response to your review request within 20 working days of its receipt. Please quote
the reference number above in any future communications.

If you are not content with the outcome of the internal review, you would have the right to apply directly to the Information Commissioner for a decision. Please bear in mind that the Information Commissioner will not normally review our handling of your request unless you have first contacted us to conduct an internal review. The Information Commissioner can be contacted online via an electronic form: https://ico.org.uk/make-a-complaint/foi-and-eir-complaints/foi-and-eir-complaints/

Or in writing to:
Information Commissioner's Office,
Wycliffe House,
Water Lane,
Wilmslow,
Cheshire,
SK9 5AF

Yours sincerely,

HQA FOI Team