Resource impact summary report

Resource impact
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NICE has recommended etranacogene dezaparvovec with managed access as an option for treating moderately severe or severe haemophilia B (congenital factor IX [FIX] deficiency) in adults without anti-FIX antibodies. It is only recommended if the conditions in the managed access agreement for etranacogene dezaparvovec are followed.

Etranacogene dezaparvovec will be available to the NHS in line with the managed access agreement with NHS England. As part of this, NHS England and CSL Behring have a commercial arrangement (managed access agreement). This makes etranacogene dezaparvovec available to the NHS with a discount. The size of the discount is commercial in confidence.

It is estimated around 300 adults in England are currently living with severe to moderately severe or severe haemophilia B. The number of people eligible for treatment within this guidance is likely to be lower. Etranacogene dezaparvovec is delivered by a single intravenous infusion. There are capacity benefits relating to the one-off single administration of the technology versus current options requiring 6 to 8 weekly deliveries of FIX replacement therapy. This involves hospital homecare pharmacy administrative time for prescriptions and homecare delivery of blood products.

There is increased monitoring in the year people start treatment with etranacogene dezaparvovec, after this the number of appointments is the same as comparators.

When NICE recommends a treatment as an option for use within a managed access agreement, NHS England will make it available according to the conditions in the managed access agreement. This means that, if a person has moderately severe or severe haemophilia B (congenital FIX deficiency) without anti-FIX antibodies, and the doctor responsible for their care thinks that etranacogene dezaparvovec is the right treatment, it should be available for use in line with NICE's recommendations and the criteria in the managed access agreement.

The committee was satisfied that further data collection through a managed access arrangement could gather further evidence on treatment effectiveness and recognised that the ongoing HOPE-B trial could provide additional high-quality data to address some of the uncertainty about this innovative and complex treatment’s long-term durability. After this, NICE will decide whether or not to recommend it for use in the NHS and update the guidance.

This technology is commissioned by NHS England. Providers are NHS hospital trusts.