

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Appraisal

Idecabtagene vicleucel for treating relapsed and refractory multiple myeloma in people who have received at least 3 prior therapies

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of idecabtagene vicleucel within its marketing authorisation for treating multiple myeloma.

Background

Multiple myeloma is a form of cancer that arises from plasma cells (a type of white blood cell) in the bone marrow. Myeloma cells produce large quantities of an abnormal antibody, known as paraprotein. Unlike normal antibodies, paraprotein has no useful function and lacks the capacity to fight infection. Myeloma cells suppress the development of normal blood cells that are responsible for fighting infection (white blood cells), carrying oxygen around the body (red blood cells) and blood clotting (platelets). The term multiple myeloma refers to the presence of more than one site of affected bone at the time of diagnosis. People with multiple myeloma can experience bone pain, bone fractures, tiredness (due to anaemia), infections, hypercalcaemia (too much calcium in the blood) and kidney problems.

In 2017, 5,034 people were diagnosed with multiple myeloma in England.¹ It is most frequently diagnosed in older people, with 43% of new cases in England in people aged 75 years and over.² Multiple myeloma is more common in men than in women and the incidence is also reported to be higher in people of African family origin.³ The 5-year survival rate for adults with multiple myeloma in England is about 52%.⁴

Multiple myeloma is an incurable disease. Therapy aims to prolong survival and maintain a good quality of life by controlling the disease and relieving symptoms. Some people may be eligible for stem-cell transplants. If the disease progresses after initial treatment, the choice of subsequent therapy is influenced by previous treatment and response to it, the duration of remission, comorbidities and patient preference.

For people who have had at least 2 prior therapies:

- NICE technology appraisal guidance [171](#) recommends lenalidomide in combination with dexamethasone as a treatment option for people who have received 2 or more previous therapies.
- NICE technology appraisal guidance [380](#) recommends panobinostat in combination with bortezomib and dexamethasone as a treatment option for people who have received at least 2 previous therapies including bortezomib and an immunomodulatory agent.
- NICE technology appraisal guidance [505](#) recommends ixazomib citrate in combination with lenalidomide and dexamethasone for use within the Cancer Drugs Fund after 2 or 3 previous therapies.

Final scope for the appraisal of idecabtagene vicleucel for treating relapsed and refractory multiple myeloma in people who have received at least 3 prior therapies

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For people who have had at least 3 prior therapies:

- NICE technology appraisal guidance [427](#) recommends pomalidomide in combination with low-dose dexamethasone as a treatment option after 3 previous therapies including both lenalidomide and bortezomib.
- NICE technology appraisal guidance [510](#) recommends daratumumab monotherapy for use within the Cancer Drugs Fund after 3 previous therapies.

The technology

Idecabtagene vicleucel (brand name unknown, Celgene, a BMS company) is a chimeric antigen receptor (CAR) T-cell therapy that changes a patient’s T cells to target a protein called BCMA, which is highly expressed on myeloma cells. Idecabtagene vicleucel is administered as a single intravenous infusion.

Idecabtagene vicleucel does not currently have a marketing authorisation in the UK for multiple myeloma. It is being studied in clinical trials for relapsed and refractory multiple myeloma in adults who have received at least 3 previous treatments (a single-arm study) and 2 to 4 previous treatments (a randomised study), including a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 antibody.

Intervention(s)	Idecabtagene vicleucel
Population(s)	People with relapsed and refractory multiple myeloma who have had at least 3 prior therapies
Comparators	<ul style="list-style-type: none"> • Pomalidomide in combination with dexamethasone • Panobinostat in combination with bortezomib and dexamethasone
Outcomes	<p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> • overall survival • progression-free survival • response rates (for example overall response and complete response) • adverse effects of treatment • health-related quality of life.

Economic analysis	<p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p> <p>The availability of any patient access schemes for the intervention, comparator technologies and subsequent therapies (such as treatment for cytokine release syndrome) will be taken into account.</p>
Other considerations	<p>Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.</p>
Related NICE recommendations and NICE Pathways	<p>Related Technology Appraisals:</p> <p>Daratumumab monotherapy for treating relapsed and refractory multiple myeloma (2018) NICE technology appraisals guidance 510. Review date November 2020.</p> <p>Ixazomib with lenalidomide and dexamethasone for treating relapsed or refractory multiple myeloma (2018) NICE technology appraisals guidance 505. Review date December 2019.</p> <p>Pomalidomide for multiple myeloma previously treated with lenalidomide and bortezomib (2017) NICE technology appraisals guidance 427. Review date 2020.</p> <p>Terminated appraisals:</p> <p>Pomalidomide with bortezomib and dexamethasone for treating relapsed or refractory multiple myeloma (2019) NICE technology appraisal guidance 602. (Terminated appraisal).</p> <p>Elotuzumab for treating relapsed or refractory multiple myeloma (2017) NICE technology appraisal guidance 434. (Terminated appraisal).</p> <p>Bortezomib for treating multiple myeloma after second or subsequent relapse (2017) NICE technology appraisal guidance 453. (Terminated appraisal)</p> <p>Daratumumab with lenalidomide and dexamethasone for treating relapsed or refractory multiple myeloma (2017) NICE technology appraisal guidance 454 (Terminated appraisal).</p>

	<p>Appraisals in development (including suspended appraisals):</p> <p>Selinexor with low-dose dexamethasone for treating refractory multiple myeloma. NICE technology appraisals guidance [ID1535]. Publication expected January 2021.</p> <p>Isatuximab with carfilzomib and dexamethasone for treating relapsed or refractory multiple myeloma. NICE technology appraisals guidance [ID1620]. Publication date to be confirmed.</p> <p>Isatuximab with pomalidomide and dexamethasone for treating relapsed or refractory multiple myeloma. NICE technology appraisals guidance [ID1477]. Publication expected October 2020.</p> <p>Belantamab mafodotin for treating relapsed or refractory multiple myeloma after 3 therapies. NICE technology appraisal guidance [ID2701]. Publication date to be confirmed.</p> <p>Carfilzomib with daratumumab and dexamethasone for treating relapsed or refractory multiple myeloma. NICE technology appraisal guidance [ID2709]. Publication date to be confirmed.</p> <p>Pembrolizumab for previously treated multiple myeloma. NICE technology appraisals guidance [ID1139]. Suspended August 2017.</p> <p>Plitidepsin in combination with dexamethasone for treating relapsed or refractory multiple myeloma. NICE technology appraisals guidance [ID1081]. Suspended April 2018.</p> <p>Related Guidelines:</p> <p>Haematological cancers: improving outcomes (2016) NICE guideline 47</p> <p>Myeloma: diagnosis and management (2016) NICE guideline 35. Last updated October 2018.</p> <p>Related Quality Standards:</p> <p>Haematological cancers (2017) NICE quality standard 150</p> <p>Related NICE Pathways:</p> <p>Myeloma (2018) NICE pathway</p>
<p>Related National Policy</p>	<p>The NHS Long Term Plan, 2019. NHS Long Term Plan</p> <p>NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019) Blood and marrow transplantation services (adults and children) [section 29, page 98], specialist cancer services (adults) [section 105, page 274].</p> <p>Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 1, 4, 5.</p>

	https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017
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References

¹ Cancer Research UK. Available from: '[Myeloma incidence by sex and UK region](#)'. Accessed September 2020.

² Office for National Statistics. Available from: '[Cancer registration statistics, England](#)'. Accessed September 2020.

³ National Cancer Institute. Available from: '[SEER Cancer Statistics Review, 1975-2008](#)'. Accessed September 2020.

⁴ Cancer Research UK Available from: '[Myeloma survival](#)'. Accessed September 2020.