

Appendix B

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Appraisal

Lenalidomide for the maintenance treatment of newly diagnosed multiple myeloma after autologous stem cell transplantation

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of lenalidomide within its marketing authorisation for the maintenance treatment of multiple myeloma after autologous stem cell transplantation.

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 ability 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 1 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 2016, 4,731 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 and Wales is about 47%.⁴

Therapy aims to prolong survival and maintain a good quality of life by controlling the disease and relieving symptoms. NICE technology appraisal 311 recommends bortezomib in combination with dexamethasone, or with dexamethasone and thalidomide for the induction treatment of adults with previously untreated multiple myeloma, who are eligible for high-dose chemotherapy with haematopoietic stem cell transplantation. After stem cell transplantation, all patients receive ongoing monitoring and supportive care. In some patients, maintenance treatment may be considered to stimulate the immune system and slow or stop cancer cell growth. However, there is currently no standard maintenance treatment used in the NHS.

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The technology

Lenalidomide (Revlimid, Celgene) is a structural analogue of thalidomide. Its mechanism of action includes anti-neoplastic, anti-angiogenic, pro-erythropoietic, and immunomodulatory properties. Lenalidomide inhibits proliferation of certain haematopoietic tumour cells, enhances T cell- and Natural Killer (NK) cell-mediated immunity and inhibits production of pro-inflammatory cytokines. Lenalidomide is administered orally.

Lenalidomide as monotherapy has a marketing authorisation for the maintenance treatment of adult patients with newly diagnosed multiple myeloma who have undergone autologous stem cell transplantation.

Intervention(s)	Lenalidomide
Population(s)	People with newly diagnosed multiple myeloma who have had autologous stem cell transplantation.
Comparators	<ul style="list-style-type: none">Established clinical management without lenalidomide maintenance therapy (including monitoring and follow up)
Outcomes	The outcome measures to be considered include: <ul style="list-style-type: none">overall survivalprogression-free survivaltime to relapse or progressionadverse effects of treatmenthealth-related quality of life.

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<p>Economic analysis</p>	<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 commercial arrangements for the interventions, comparator and subsequent treatment technologies will be taken into account.</p>
<p>Other considerations</p>	<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>
<p>Related NICE recommendations and NICE Pathways</p>	<p>Related Technology Appraisals:</p> <p>Lenalidomide plus dexamethasone for multiple myeloma after 1 treatment with bortezomib (2019) NICE technology appraisal 586. Review date 2022</p> <p>Lenalidomide plus dexamethasone for previously untreated multiple myeloma (2019) NICE technology appraisal 587. Review date 2022</p> <p>Bortezomib for induction therapy in multiple myeloma before high-dose chemotherapy and autologous stem cell transplantation (2014) NICE technology appraisal 311. On static list</p> <p>Appraisals in development (including suspended appraisals)</p> <p>Daratumumab in combination for untreated multiple myeloma when stem cell transplant is suitable. In development ID1510. Expected publication date to be confirmed.</p>

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	<p>Bortezomib for induction and consolidation therapy after autologous stem cell transplantation for the treatment of multiple myeloma ID529. Suspended.</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>
Related National Policy	<p>The NHS Long Term Plan, 2019. NHS Long Term Plan 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. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017</p>

References

- ¹ Cancer Research UK. Available from: '[Myeloma incidence by sex and UK region](#)'. Accessed October 2019.
- ² Office of national statistics. Available from: '[Cancer registration statistics, England](#)'. Accessed October 2019.
- ³ National cancer institute. Available from: '[SEER Cancer Statistics Review, 1975-2008](#)'. Accessed October 2019.
- ⁴ Cancer Research UK Available from: '[Myeloma survival](#)'. Accessed October 2019.

Draft scope for the appraisal of lenalidomide for the maintenance treatment of multiple myeloma after autologous stem cell transplantation

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