Long-Term Fiscal Implications of MEPACT in the Treatment of High-Grade Nonmetastatic Osteosarcoma: A Budget-Impact Model and a Lifetime Tax Perspective

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BACKGROUND

- Nonmetastatic osteosarcoma is a type of bone cancer that primarily affects children, adolescents, and young adults.
- It is an ultra-orphan disease, a term used to describe very rare diseases.
- MEPACT (melphalan) is indicated for use in children and adults aged between 1 and 65 years of age for the treatment of high-grade resistant nonmetastatic osteosarcoma after macroscopically complete surgical resection to remove the tumour.
- MEPACT is used in combination with postoperative multiagent adjuvant chemotherapy.
- The largest ever completed phase 3 randomized trial in osteosarcoma (METASTASI013 study, N = 842) comparing add-on MEPACT to three- or four-drug-adjusted regimen using multiagent adjuvant chemotherapy with the addition of MEPACT to chemotherapy, a statistically significant increase in 5-year overall survival from 70% to 76% (P = 0.03).
- The average characteristics in the model were based on a generalizing accounting framework described by Cardarelli et al. (2009) and used by Connolly et al. (2009) to model direct financial transactions between an ultra-orphan health (IVF) conceived singleton and the UK government over the projected lifetime.
- To estimate the long-term financial impact on the government, introducing MEPACT.
- The annual budget impact for the NHS in England and Wales is estimated to be £23,040,285 in 2011, rising to £46,048,095 in 2015.
- There are no potential resource savings through the introduction of MEPACT.
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- The additional budget impact due to MEPACT is mainly due to the unmet cost of the drug.
- Given the young age at which the average patient may be treated for high-grade resistant nonmetastatic osteosarcoma, the societal impact may be significant.
- From the tax calculations, we conclude that investment in MEPACT does not negatively impact the long-term fiscal budget of the UK government.
- Consequently, by taking a broader government perspective over an average lifetime, a surviving patient returns a positive net value to the state.
- On average, a patient who receives MEPACT has repaid all costs of treatment by the age of 41 years and then goes on to make significant positive contributions to society.

METHODS

Budget-impact Model Overview

• A previously developed budget impact model was developed to evaluate the trajectory of spending over 1- to 5-year time horizons in a hospital setting for a specific subset of patients in a 1-year period with nonmetastatic osteosarcoma from an NHS perspective.

• The model is based on a 1-year period with nonmetastatic osteosarcoma in a 1-year period based on prevalence data from the Automated Childhood Information System and population data from the Office of National Statistics, UK (Table 1).

• The model assumes 80% of all cancer patients are newly diagnosed, nonmetastatic, and resistant.

• Patients received the average number of infusible doses administered in a 1-year period (i.e., 34 doses).

Budget Impact Model Input Parameters

• The model was populated with clinical and economic data obtained from a variety of sources, including:
  - Clinical trial data for MEPACT
  - Published literature
  - Treatment guidelines
  - Other information from published and unpublished sources

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RESULTS

The additional budget impact due to MEPACT is mainly due to the unmet cost of the drug.

CONCLUSIONS

• Treatment with MEPACT improves survival outcomes for nonmetastatic osteosarcoma patients.

• There are no potential resource savings through the introduction of MEPACT.

REFERENCES


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