The difference between regulatory and market access decisions on treatment availability for new drugs in six common cancers across Australia, Canada, and Europe

Jan Kendrick,¹ Bill Malcolm,² Kate Sheahan,³ Ioannis Katsoulis,¹ Xirong Song,¹ Jeannv van Loon¹

¹PRMA Consulting Ltd, Fleet, UK; ²Bristol-Myers Squibb Pharmaceuticals Ltd, Uxbridge, UK; ³Gillings School of Global Public Health, University of North Carolina at Chapel Hill, Chapel Hill, NC, USA

Background and objectives

• Cancer is associated with high levels of mortality, morbidity, and economic burden.
• Innovation: Unapproved/least-licensed medicines are subject to health technology assessment (HTA) or pricing and reimbursement (P&R) processes that have evolved in response to the significant budgetary challenges that medical advances may bring.
• Assessments may restrict access to medicines to a subset of the population covered by the regulatory license. Thus, discrepancies can arise between the clinically eligible population (under the license) and the population for which treatment is funded through reimbursement.
• The study objective was to identify discrepancies between regulatory and reimbursement decisions across 13 countries and quantify their impact on patients – both the number of clinically eligible patients without access and the associated years of life lost (YLL).

Methods

• Six common cancers were identified (breast, kidney, lung, melanoma, multiple myeloma, and prostate). New oncology therapies granted a first license by the European Medicine Agency (EMA), Health Canada (HC), or the Australian Therapeutic Goods Administration (TGA) between January 2006 and June 2016 were identified (Figure 1) along with any relevant follow-on indications.

Results

• Scenario analyses demonstrate the impact of assumptions around missing assessments (i.e., resulting either in complete restriction [high-restriction scenario] or no restriction [base case, low-restriction scenario]) and the estimated size of the affected patient population.
• No systematic differences in access restrictions were identified across the six cancer types (Figure 3). The highest estimated rate of patients without access to publically reimbursed treatments was for lung cancer; multiple myeloma was associated with the highest YLL burden.

Discussion and conclusions

• Patient access to cancer drugs is restricted to varying degrees through national reimbursement assessments in Europe, Canada, and Australia. Scenario analyses suggest that, although results depend differing assumptions, the base case analysis may underestimate the impact of restrictions in some countries.
• Country-specific differences in P&R decision-making criteria play a pivotal role in determining the patient population eligible for reimbursement, as does the specificity of the licensed indication and the strength of the evidence base, although these are more consistent across countries.
• The nature and extent of restrictions are not consistent across countries, resulting in inequitable access to new cancer medicines, which affects patients, families, caregivers, and clinicians.
• Variation in access across countries is expected in part because of different willingness-to-pay thresholds for oncology medicines. Thresholds are influenced by healthcare system characteristics such as healthcare system structure, funding sources, and government prioritization of healthcare financing.
• Our results are directly relevant to health policy initiatives in Europe that aim to improve access to medicines and reduce differences across member states.

References

1. 2018 population estimates from the World Bank online database

Footnotes

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Methodology overview

HTA or P&R decisions identified from national sources (2006–February 2017) Restrictions on reimbursed populations were classified as:
• Full restriction: available to all patients within the regulatory license
• Partial restriction: available to subpopulation(s) of patients within the regulatory license
• Complete restriction: not available to any patient within the regulatory license

Number of drug/indication combinations (HTA or P&R decisions identified from national sources (2006–February 2017)) Restrictions on reimbursed populations were classified as:
• Full restriction: available to all patients within the regulatory license
• Partial restriction: available to subpopulation(s) of patients within the regulatory license
• Complete restriction: not available to any patient within the regulatory license

Based on published assessments only. * Nine drug/indication combinations approved by EMA were not licensed in Canada or Australia.

Table 1: Estimated impact of reimbursement national restrictions by cancer type across Australia, Canada, and 11 European countries

Discussion and conclusions

Patient access to cancer drugs is restricted to varying degrees through national reimbursement assessments in Europe, Canada, and Australia. Scenario analyses suggest that, although results differ depending on assumptions, the base case analysis may underestimate the impact of restrictions in some countries.

Country-specific differences in P&R decision-making criteria play a pivotal role in determining the patient population eligible for reimbursement, as does the specificity of the licensed indication and the strength of the evidence base, although these are more consistent across countries.

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Our results are directly relevant to health policy initiatives in Europe that aim to improve access to medicines and reduce differences across member states.