1584O Health technology assessment (HTA) in England, France and Germany: What do we know about variations in cancer-related HTA outcomes?

R. Schaefer, D. Hernández, M. Schlander

Division of Health Economics, German Cancer Research Center, Heidelberg, Germany

Background: Variations in HTA outcomes can primarily be explained with reference to institutional context and administrative rules. Here we focus on the German Federal Joint Committee (Gemeinsamer Bundesausschuss, GBA), the French Haute Autorité de Santé (HAS) and the British National Institute for Health and Care Excellence (NICE) to identify matched drug pairs as a basis to compare HTA outcomes and to analyze cancer-related HTA results in detail.

Methods: Data were extracted from all published GBA resolutions from January 2011 — when the AMNOG legislation was introduced in Germany — to June 2018, as well as all publicly available HAS reports and NICE single technology appraisals during this period. We compared HTA outcomes of matched pairs overall, and separately for non-cancer and cancer drugs. Then, the potential role of additional attributes with regard to cancer drugs was explored, such as orphan drug designation in Germany, impact of reimbursement rates in France and consideration of end of life (EoL) criteria in England. The relationship between HTA outcomes and attributes was tested for statistical significance with a chi-square test or, if required, Fisher's exact test.

Results: By pairwise comparison, HTA outcomes (cancer, 58/102; non-cancer, 44/102) showed higher congruence for GBA/HAS (total, 67%; cancer, 72%; non-cancer, 59%) than for GBA/NICE and HAS/NICE (total, 54%; cancer, 57%; non-cancer, 50%). NICE recommended 85/102 (cancer, 42/58; non-cancer, 43/44) technologies, whereas GBA and HAS reported added benefit for 72/102 (cancer, 49/58; non-cancer, 23/44) and 60/102 (cancer, 37/58; non-cancer, 23/44) medicines, respectively. When we tested NICE cancer recommendations for the consideration of EoL criteria, no significant impact was found ($p>.05$, Fisher's test). In contrast, GBA cancer appraisals are associated with an orphan designation ($p<.05$, chi-square test), and cancer-related findings for the HAS indicate that the reimbursement rate correlated with the assessment of clinical added value ($p<.05$, chi-square test).

Conclusions: Our results confirm that variations in HTA outcomes frequently exist. However, cancer-related HTA results seem to be less divergent compared to non-cancer results.

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1586MO Pivotal trial endpoints of drugs for rare and non-rare cancers in the US and Europe

K.N. Vokinger[1], P. Daniore[1], A.S. Kesselheim[2]

[1]Institute of Law, University of Zurich, Zurich, Switzerland; [2]Program on Regulation, Therapeutics, and Law (PORTAL), Brigham and Women's Hospital and Harvard Medical School, Boston, MA, USA

Background: A key clinical outcome for new cancer drugs is improvement in overall survival (OS), defined as time from the date of randomization to the death from any cause. Surrogate endpoints, such as progression-free survival or overall response rate, can provide misleading information about efficacy. However, for rare cancers, as outlined by Rare Cancers Europe (RCE), a campaign developed by ESMO, surrogate endpoints may be of value or even the only way to show improvements timely. Furthermore, the FDA and EMA introduced orphan drug designation to encourage drug development for rare conditions. We categorized pivotal trial endpoints for approved cancer drugs by the FDA and EMA (OS vs. OS surrogates) and evaluated the correlation with orphan drug designations by the FDA and EMA as well as rare cancers as defined by the RCE.

Methods: We identified new cancer drugs FDA-approved between 2009 and 2019 that were indicated to treat solid and hematologic tumors in adults and that had also been approved by the EMA by December 2019. Fisher's exact tests were conducted to assess the association between pivotal trial endpoints (OS vs. non-OS) and orphan drug designation by the FDA and EMA, as well as between pivotal trial endpoints and rare cancers as defined by RCE.

Results: 76 drugs were approved by the FDA and EMA during the study period. In the US, 39 (51%) were approved based on OS by contrast to 49 (64%) in the EU; 50 (66%) drugs were designated orphan status by the FDA, 17 (22%) by the EMA. There was an association between rare cancers as defined by the RCE and drug indications designated with orphan status by the FDA ($p=0.007$) or EMA ($p<0.001$). However, there was no association between pivotal trial endpoints (OS vs. non-OS) and orphan drug designation by the FDA ($p=0.094$) or EMA ($p>0.9$). There was also no association between rare cancers as defined by the RCE and pivotal trial endpoints ($p=0.2$ [US] and $p=0.3$ [EU]).

Conclusions: Approval of drugs for non-rare cancers should be better aligned with OS as pivotal trial endpoint. Since surrogate measures may not be adequately predictive of patient-centred outcomes, cancer drugs approved based on surrogate endpoints should be closely followed up also after approval to offer patients optimal value.

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1588MO_PR A comparative study on costs of cancer and access to medicines in Europe

N. Wilking[1], G. Brådvik[2], P. Lindgren[3], C. Svedman[4], B. Jönsson[5], T. Hofmarcher[6]

[1]Oncology and pathology, Karolinska Institutet, Stockholm, Sweden; [2]The Swedish Institute for Health Economics, The Swedish Institute for Health Economics, Lund, Sweden; [3]Karolinska Institutet, The Swedish Institute for Health Economics, Lund, Sweden; [4]The Swedish Institute for Health Economics, The Swedish Institute for Health Economics, Lund, Sweden; [5]Economics, Stockholm School of Economics, Stockholm, Sweden; [6]Lund University, The Swedish Institute for Health Economics, Lund, Sweden

Background: Costs and value of new cancer treatments are often causing headlines without being discussed in a larger context. This study estimates the cost of cancer and access to medicines in Europe in 2018 and extends a previous analysis for 1995—2014.

Methods: Cancer-specific health expenditure for 31 countries (EU-27 plus Iceland, Norway, Switzerland, and the UK) were derived from national estimates. Data on cancer drug sales were obtained from IQVIA. Productivity loss from premature mortality was estimated from data from Eurostat and the WHO. Productivity loss from morbidity and informal care costs were estimated based on previous studies.

Results: The total cost of cancer was €199 billion in 2018. Total costs ranged from €160 per capita in Romania to €578 in Switzerland (after adjustment for price differentials). Health expenditure on cancer care were €103 billion, of which €32 billion were spent on cancer drugs. Informal care costs were €26 billion. The total productivity loss was €70 billion, composed of €50 billion from premature mortality and €20 billion from morbidity. Patient access to cancer medicines was much greater in wealthier than poorer countries in 2018, in terms of value and volume. The top spenders were Austria, Germany, and Switzerland (€92 to €108 per capita), whereas Czechia, Latvia, and Poland spent the least (€13 to €16). The largest country differences were seen in immuno-oncology medicines. Between 1995 and 2018, cancer incidence increased by 50% in Europe, but cancer mortality increased only by 20%. Health spending on cancer doubled from €52 billion to €103 billion (in 2018 prices and exchange rates), but the share of cancer care on the total health expenditure remained stable at around 4—7%. A shift from treatment in inpatient care to ambulatory care has likely saved costs. Expenditure on cancer medicines tripled from €10 billion to €32 billion between 2005 and 2018 (excluding confidential rebates). Productivity loss from premature mortality decreased over time, linked to mortality reductions in working-age patients.

Conclusions: There are large and persistent country differences in spending on cancer care, access to new cancer medicines and outcomes in Europe. Inequalities are mainly related to countries' economic strength and not to the disease burden of cancer.

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1593P Prices and price developments of cancer drugs in the US and Europe

K.N. Vokinger[1], P. Daniore[1], T. Hwang[2], A. Kesselheim[3]

[1]Academic Chair for Regulatory Sciences, University of Zurich, Zurich, Switzerland; [2]Program on Regulation, Therapeutics, and Law, Brigham and Women's Hospital, Boston, MA, USA; [3]Program on Regulation, Therapeutics, and Law, Brigham and Women's Hospital / Harvard Medical School, Boston, MA, USA

Background: Cancer drug costs are rising in the US and Europe. While drug manufacturers set prices without restriction in the US, European countries have regulations that allow national authorities to directly negotiate drug prices at launch and over time. We analyzed and compared the launch prices and price developments of cancer drugs in the US, Germany, Switzerland and England.