VP21 Factors Associated With Recommendations On Drugs For Rare Diseases

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INTRODUCTION:

In Canada, reimbursement recommendations on drugs for common and rare indications (for example, orphan drugs) are made through the pan-Canadian Oncology Drug Review (pCODR) and the Common Drug Review (CDR). However, some stakeholders have called for a separate mechanism for orphan drugs, arguing that existing processes place too much weight on their high price tags. The purpose of this study was to examine factors associated with positive recommendations on drugs for rare diseases.

METHODS:

Information was extracted from CDR and pCODR recommendations on drugs for diseases (prevalence of less than 1 in 2,000) up to April 2018. Univariate and multivariate logistic regression models were applied to explore the influence of the following variables on recommendations: year; prevalence; clinical safety and effectiveness (safety, quality of life, symptoms, surrogate outcomes, and survival); quality of evidence (availability of comparative data, external validity, and bias); unmet need; treatment cost; and incremental cost-effective ratio (ICER). Two-way interactions were also tested.

RESULTS:

Of 128 recommendations, fifty-four (77 percent) and forty (69 percent) were positive for cancer and non-cancer indications, respectively. For cancer indications, all submissions reporting meaningful improvements in surrogate, quality of life, and survival outcomes were significantly more likely to have a positive recommendation. Submissions showing a lack of external validity were significantly less likely to receive a positive recommendation. For non-cancer indications, more recent submissions and those presenting no safety issues were associated with positive recommendations. Prevalence, treatment cost, and ICER were not determinants of positive or negative recommendations.

CONCLUSIONS:

For both cancer and non-cancer orphan drugs, impact on clinical safety and effectiveness, rather than cost, appears to be a key factor in the formulation of recommendations.

VP22 Future Trends For Managed Access Agreements In The UK

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INTRODUCTION:

In recent years, the National Institute for Health and Care Excellence (NICE) has increasingly agreed to reimburse innovative products with high levels of uncertainty as part of managed access agreements (MAAs) while new data are collected; namely, this has occurred through the new Cancer Drugs Fund (CDF) and highly specialized technology (HST) appraisal pathway. This research aimed to provide a review of ongoing data collection arrangements as part of MAAs agreed with NICE.

METHODS:

We reviewed all current MAAs entered into between the National Health Service (NHS) England and manufacturers as of 24 November 2017 and extracted relevant information related to the data collection arrangements.

RESULTS:

Thirteen MAAs were identified (10 through the CDF; 3 through HST). All MAAs involved an observational data collection agreement. The source of observational data collection was existing NHS databases (11 MAAs: 85 percent), existing independent registries (1 MMA: 8 percent [ataluren]); bespoke MAA registry maintained by manufacturer (1 MAA: 8 percent [asfotase alfa]), and registries developed as a requirement for regulatory approval and maintained by the manufacturer (1 MAA: 8 percent [elosulfase alfa]). Only 4 MAAs (asfotase alfa, ataluren, elosulfase alfa, and venetoclax) had observational data collection as the sole basis of the data collection agreement. The other 9 MAAs (69

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percent; all from the CDF) also required on-going data collection from clinical trials as a key component of the data collection agreement.

CONCLUSIONS:

This research shows that current MAAs have predominantly utilized either ongoing data collection (e.g. from RCTs) or existing registries to date for which limited additional set-up administration and costs would be required. However, NICE plan to increase the use of MAAs, with ongoing NICE consultation for changes in the appraisal process to expand MAAs to include all indications. In future, manufacturers will have more opportunities to explore and leverage innovative and bespoke MAAs to help achieve access.

VP23 The New Cancer Drugs Fund: The Future Model Of Oncology Reimbursement

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INTRODUCTION:

The Cancer Drugs Fund (CDF) was set up in 2011 in England to enable patients to access oncology therapies that are not routinely publicly funded. In April 2016, the CDF became a temporary reimbursement fund under the remit of the National Institute for Health and Care Excellence (NICE) with the aim of collecting observational data to inform subsequent technology appraisals. This study aims to evaluate how the reformed CDF has been utilized in the 18 months since this reform.

METHODS:

NICE Final Appraisal Determinations for Single Technology Appraisals of oncology drugs from (29 July 2016 to 24 November 2017) were identified and key data extracted.

RESULTS:

Seventy-four oncology drug:indication appraisals were identified, 54 (73 percent) were recommended/ optimized, 10 (14 percent) were not recommended and 10 drug:indication pairings (14 percent: osimertinib, brentuximab vedotin, pembrolizumab, olaratumab, obinutuzumab, venetoclax, nivolumab [3 indications], and ibrutinib) were referred to the CDF. For most, the greatest uncertainty in their cost-effectiveness analyses related to their survival benefits, intended to primarily be resolved through subsequent clinical trial readouts. However, for venetoclax, ibrutinib and brentuximab, the main areas of uncertainty (relating to comparative survival benefit, pre-progression mortality, and rate of subsequent stem cell transplants, respectively) are expected to be resolved primarily through observational data collected under the CDF.

CONCLUSIONS:

The newly reformed CDF has been utilized in a minority of cases. Typically, the CDF acts as a temporary access mechanism for treatments that receive market authorization based on early/single-arm trial data until longer-term and/or Phase III data are available. However, venetoclax, brentuximab, and ibrutinib demonstrate how the CDF may address significant areas of uncertainty through the collection of uncontrolled observational data. For venetoclax, with only single-arm supportive clinical trial data, observational data of this intervention and appropriate comparator are to be collected, providing a potential case study of how to appropriately manage reimbursement in the face of significant clinical uncertainty.

VP24 HTA To Assess Esthetic Procedures In France: Haute Autorité de Santé (HAS) Seven Year Experience

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INTRODUCTION:

The Health Technology Assessment (HTA) of esthetic procedures was performed by the French National Authority for Health (HAS), at the request of the French Ministry of Health (MoH), and under a new regulatory framework enabling the government to ban esthetic procedures considered harmful or potentially harmful to patients and consumers by HAS. Objectives: Describe

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