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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