OP38 Managed (Early) Access In England: The 'Ins And Outs'

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Introduction. Managed access approaches have been used by The National Institute for Health and Care Excellence (NICE) in partnership with NHS England since 2015. The Cancer Drugs Fund is an exemplar of this approach which enables earlier patient access to promising new treatments while further real-world data is collected to address evidential uncertainties. Increasingly, this approach is being applied to rare diseases and other conditions to address unmet clinical need with extensive involvement from patient organizations and clinicians.
Methods. All Managed Access Agreements (MAAs) in development or published in England were reviewed to present data on the number of technologies (i) entering, (ii) in active monitoring, and (iii) exiting managed access, for both cancer and other disease areas.
Results. After six years since the first MAA (at December 2021), over 73,000 patients have benefited from earlier access to promising new treatments for cancer, genetic and rare diseases, including cystic fibrosis, spinal muscular atrophy and sickle cell disease via managed access. Fifty-eight technologies were commissioned via managed access: thirty technologies in active data collection, eleven technologies being re-evaluated, and seventeen technologies have exited managed access. Patient and clinical engagement have been essential to the successful real-world data collections delivered and underway.
Conclusions. Managed access is an approach for providing earlier patient access to promising new technologies that would not otherwise be recommended for use in England. The approach to managed access in England is maturing at the same time the volume of topics entering and exiting managed access in England is expected to grow throughout 2022 with the introduction of the Innovative Medicines Fund.

OP39 The Real-Option Rate Of Return Approach To Inform The Pricing Of Medicines

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Introduction. Prices of medicines have increasingly come under payer and societal scrutiny in many countries around the world. As the price-setting process is quite untransparent, the concept of cost-based pricing has been brought forward as an alternative method to inform reimbursement decision-making. A Real-Option rate of Return (ROroR) approach, was recently proposed as a method for a multi-stakeholder driven collaborative investment model. This study showed that there are public-private medicine development opportunities that could lead to lower research and development (R&D) costs for products with a challenging business model. The aim of the current study is to assess the practical use of the ROroR approach and highlight its sensitivity regarding input parameters.
Methods. The ROroR approach incorporates medicine-specific parameters: R&D costs, the number of patients treated per year, the time horizon for recouping the investments set by the stakeholders, the production costs and a predefined profit margin. Three hypothetical case-studies were selected for the ROroR analysis comprising of an orphan, oncology, and a more regular medicine. Parameter input data was derived from the available literature. Cost-based prices were calculated based on applying the ROroR equation under a constant profit margin. Ultimately, the corresponding prices of the case-studies were analyzed for their sensitivity using ten changes of the original value.
Results. The ROroR approach was most sensitive to the length of the time horizon and the number of patients treated per year. The largest sensitivity was found for the oncological drug, with an asymmetric price change ranging from -25 percent to +271 percent if varying the time horizon or number of patients. The profit margin, and total R&D costs have the least effect on the price: +/-4 percent and +/-45 percent, respectively.
Conclusions. This study shows that cost-based pricing is highly beneficial in uncovering pricing-underlying business or economic mechanisms and suggesting a transparent price. Further research is needed on implementing public-private development models and cost-based price determination using the core parameters.

OP41 Facilitating Dialogue Of Real-World Evidence Use In Health Technology Assessment: Taxonomy Of Question/Data Source Pairings To Support A Registry Of Studies

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Introduction. This paper reports the results of the collaboration within the European initiative of new Reimbursement and aCCess Approaches (EUreccA) which is concerned with the use of real-world evidence (RWE) in health technology assessment (HTA) decision-making. The work grew from the observation of a large, very experienced group of HTA practitioners which found that the use of RWE varied depending on the type of question asked and the particulars of the data source(s) used. We set out to examine how RWE is used in HTA decision-making and to make proposals on its facilitation.

https://doi.org/10.1017/S0266462322000952 Published online by Cambridge University Press