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

Charlotte Bee (charlotte.bee@nice.org.uk), Claire Hawksworth, Leanne Wakefield and Brad Groves

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

Simon van der Schans (simonvanderschans@health-ecore.com), Marcel Schöttler, Maarten Postma and Cornelis Boersma

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

Ron L Akehurst, Linda A Murphy (linda.murphy@lumanity.com), Jorge Mestre-Ferrandiz, Oriol Solà-Morales, Gérard de Pouvourville, David Cunningham, Sorcha Corry, Matthew Franklin and Ann-Marie Chapman

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/S0266462322000976 Published online by Cambridge University Press

Methods. Literature reviews covering earlier reviews of RWE use, academic papers, and HTA agency websites were combined with case studies involving interviews with decision-makers in four countries (England, France, Italy, Sweden) to identify the circumstances of breakdown of RWE use and to build a categorization of the uses of RWE and associated difficulties. This evidence supported the creation of a taxonomy of pairings of data sources and the questions they were used to address. The face validity of the approach was tested at an advisory board of senior HTA practitioners.
Results. In total, 27 questions were identified and 10 types of data source, giving 270 pairings. These pairings were linked to relevant methods guidance and to examples of their use, itemizing HTA issues and decisions made. Reports are being prepared for publication, covering the detail of the methods of the literature searches; methods of the country case studies; a description of the taxonomy; and guidance on governance.
Conclusions. When using RWE in HTA decision-making, the detail of the particular data sources and question addressed matter. Recently, both the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) and the Real-World Transparency Initiative have argued for a registry of the uses of RWE. The work described here offers a starting classification of the material that should be held in such a registry, and which in itself could be developed by the stakeholders, both agencies and companies, that use it, furthering trust and confidence.

OP42 Increasing Access To Real-World Data To Move From Health Technology Assessment To Health Technology Management

Shaun Rowark (shaun.rowark@nice.org.uk), Pall Jonsson and Seamus Kent

Introduction. When assessing existing or emerging technologies using a one-off health technology assessment (HTA) we do not take into consideration the effects on people who will receive the technology once approved. Developments in real-world data (RWD) can help to address this by moving to ongoing health technology management (HTM).
Methods. To move to HTM, we first need to develop HTM data requirements. We undertook user interviews with National Institute for Health and Care Excellence (NICE) HTA developers to develop a list of requirements. We surveyed the types of data that NICE currently has access to and performed a gap analysis to understand where further data is needed. We then worked with external systems partners to identify and review available data sources that could support HTM.
Results. From our user interviews we established eight HTM data requirements. Data needed to be linked, cover full care pathways, contain data from new collections, be shareable, have direct access, be of high quality, have comprehensive coverage, and be responsive to technological developments (such as artificial intelligence). The review of data sources revealed a fragmented landscape of health data in the United Kingdom (UK). We identified National Health Service Digital's (NHSD) Trusted Research Environment as the main data source that could address HTM requirements. This addresses challenges with fragmented data by providing approved researchers with timely and secure access to a range of linked health and care data. We also identified that a large national data collection would not capture all technologies, such as orphan technologies for rare conditions. We therefore established a process for accessing data from smaller data collections such as disease specific registries. To address how we can use this data, we developed the NICE Real-World Evidence (RWE) Framework that provides clear guidance on the expectations for the planning, conduct, reporting, and appraisal of RWE studies.
Conclusions. We have established requirements for the type of data that will help to deliver HTM as well as developed a process for accessing several suitable data sources that meet these requirements.

OP43 Conceptual And Methodological Factors Driving The Integration Of Real-World Evidence In Drugs And Technologies Reimbursement Appraisals

Geneviève Plamondon (genevieve.plamondon@inesss.qc.ca), Yannick Auclair, Marie-Ève Tremblay, Sara Beha and Isabelle Ganache

Introduction. Real-world evidence (RWE) can be of value to support comparative effectiveness of drugs and technologies by providing additional information about their use for a variety of patients in real contexts of care. However, the integration of RWE in appraisals can be challenging, and INESSS felt the need to reinforce and explicit the underlying methodological and theoretical foundations.
Methods. A comprehensive literature review was carried out, followed by collaborative development work by members of the methodological and assessment teams.
Results. The literature review led to a common understanding of RWE underlying principles and fed the subsequent phases of the project. Three factors were identified as driving the integration of RWE in reimbursement appraisals at INESSS. Specifically, (i) the design and conduct of the real-world studies are done in accordance with best practices, (ii) the results are presented transparently and include all relevant information to assess the quality of the study and the data, and (iii) the RWE submitted is appropriate and relevant for decision-making. This third component is further ascertained by considering the decisional context (what are the circumstances motivating the submission of RWE and how does it correlate or not with existing evidence?), the data (is the dataset fit for decision needs?) and the study methods (are study design and analytical methods robust enough?). Globally, INESSS considers the integration of RWE in appraisals and its weighting, in relation with the (more traditional) available evidence, to be a case-by-case exercise.

https://doi.org/10.1017/S0266462322000976 Published online by Cambridge University Press