Vignette Presentations 93

dataset, whilst also having high prices, due to small patient populations, limiting commercial returns, may necessitate increased utilisation of alternative reimbursement mechanisms.

VP75 Improving Access To Ultra-Orphan Medicines In NHS Scotland

Noreen Downes (noreen.downes@nhs.net), Jan Jones, Anne Lee, Ailsa Brown, Pauline McGuire and Helen Wright

Introduction. Medicines for very rare conditions present challenges for healthcare globally due to uncertain evidence and often extremely high costs. In 2014, SMC introduced an ultraorphan framework placing less emphasis on the cost per quality adjusted life year (QALY). Despite this, many medicines continued to be not recommended. A new pathway aimed at improved patient access based on further evidence collection is now being implemented.

Methods. The development of the new pathway has involved collaboration with key stakeholders including patient groups, the pharmaceutical industry, and clinicians. Medicines that meet a new definition (based on four criteria including the prevalence of the condition treated) will be appraised by the SMC committee and a data collection plan will then be agreed with the pharmaceutical company.

Results. From April 2019, medicines validated as ultra-orphans will initially be appraised using the broader decision-making framework and the SMC committee will outline key uncertainties in the clinical effectiveness. The medicine will then be available for a period of at least three years while further data are gathered, potentially comprising ongoing clinical trials, registry data, and patient reported outcome measures. SMC will then re-assess the clinical and economic evidence to inform a final decision on routine use of the medicine in NHS Scotland.

Conclusions. The new pathway for ultra-orphan medicines will allow further evidence on their longer-term clinical benefits to be collected before a final decision on routine use. This approach reflects the current direction of travel in medicines regulation, by making medicines that address an unmet need available to patients at an earlier stage of development.

VP77 Extrapolating ICERs At Different Discount Rates

Conor Teljeur (cteljeur@higa.ie) and Máirín Ryan

Introduction. Applicability of incremental cost-effectiveness ratios from another jurisdiction is often affected by a different local discount rate, creating uncertainty about the ICER using the local discount rate. The ICER is sometimes reported at additional discount rates in the sensitivity analysis. We aimed to investigate the extent to which an ICER can be predicted at a given non-differential discount rate if estimates are available for at least two discount rates.

Methods. We used six previously published economic models representing analyses with a range of time horizons and ICERs calculated at discount rates from 1% to 8%. A simulation exercise was applied whereby the ICER at a discount rate selected from the range 2% to 5% was calculated based on ICERs provided at two or three randomly selected discount rates. With two discount rates a linear model was used to predict the ICER at the selected rate. For three discount rates an exponential model was used. Error between the predicted and actual ICER was calculated as the absolute difference divided by the actual ICER.

Results. For four of the models, ICERs could be well predicted by a linear model (i.e., with two points), with average errors of less than 5%. For the final two models the error was substantial with a linear model but substantially improved to under 15% with an exponential model (i.e., with three data points). The two models with a poor fit to a linear model assessed childhood vaccination programmes over a lifetime horizon.

Conclusions. For studies with a relatively short time-horizon, or where the majority of costs and benefits accrue in the short-term, a simple linear extrapolation can facilitate calculation of the ICER at a discount rate other than those reported. With longer time horizons, a third data point facilitates more reliably extrapolation of ICERs at desired discount rates.

VP82 Impact of Evidence Synthesis Methods on Outcome of Economic Evaluation

Claire Gorry (cgorry@stjames.ie), Joy Leahy, Felicity Lamrock, Cathal Walsh, Arthur White, Michael Barry and Laura McCullagh

Introduction. Evidence synthesis (ES) is often required for economic evaluation (EE) of pharmaceuticals. Commonly used methods are based on the assumption of proportional hazards in trial data, using the hazard ratio (HR). Alternative methods for ES are increasingly used in EE, in situations where the pattern of hazards in the trial data indicates that the proportional hazards assumption may be violated. The impact of these methodological choices on model outcomes is explored.

Methods. A network of trials of BRAF-targeted treatments for advanced melanoma, derived using a systematic review of the literature, is chosen for the study. Guyot's method is used to create individual-patient Kaplan-Meier (K-M) data from published survival curves. Log-cumulative hazard plots and Schoenfeld residuals are derived to examine patterns in hazards within the trial data. All analyses are conducted in R version 3.5.0©. Three alternative methods for ES are tested: 1) Network meta-analysis (NMA) based on published HRs and the assumption of proportional hazards. 2) NMA using fractional polynomials (FP) based on digitised K-M data, allowing the relaxation of the proportional hazards assumption. 3) NMA using an accelerated failure time (AFT) model based on digitised K-M data, allowing the relaxation of the proportional hazards assumption. The derived estimates of relative efficacy from each method are applied in a partitioned survival cost-effectiveness model programmed in Microsoft ExcelTM.

94 Vignette Presentations

Results. The model outcomes predicted by each method (HR, FP and AFT) are presented and compared. Both deterministic and probabilistic results are presented, alongside a discussion around how the uncertainty in these structural assumptions may be captured in EE.

Conclusions. Structural assumptions in ES may lead to differences in model outcomes. The impact of these differences may be important in situations where decision uncertainty is high. Methods should be chosen and justified based on patterns of hazard present in the trial data.

VP83 Health Economics Distance Learning For Healthcare Workers In Brazil

Ângela Bagattini (angelabagattini@gmail.com), Adélia Marçal dos Santos, Juliana Juk, Renata Soares, Sergio Piola and Cristiana Toscano

Introduction. Despite increased healthcare systems costs, limited opportunities for health economics training are available to healthcare professionals. From 2016-2018, with a grant from the Brazilian Ministry of Health, the Federal University of Goias with 7 other universities, implemented the distance learning Postgraduate Certificate in Health Economics for Health Care Professionals (PCHE) aimed at enhancing technical capacity of professionals working in the Brazilian Public Healthcare System (SUS).

Methods. This is a descriptive and qualitative assessment of the PCHE implemented in Brazil 88 healthcare professionals working in SUS and involved in decision making in all levels of managament were enrolled in a health economics training, through long-distance learning strategy. We present course metrics, describe its workload, content, modalities and structure of training.

Results. PCHE was structured with 3-day workshops introducing each of the modules, during which students were also evaluated regarding the previous module content. With a total workload of 360 hours, structured in four modules: Public Health and Epidemiology; Introduction to health economics and healthcare funding; Management of healthcare resources; and Healthcare economic evaluation. The module coordinator was resposible for supervision of course materials development, workshop, distance based tutoring activities, and evaluation. Course material included theorethical content and practical tools for economic evaluation and health technology assessment in the workplace, applying problem-based learning strategies. Certificates were granted to students with 75 percent presence and approved in all modules, and final papers approved by an examination board. Each module was completed in 8 weeks (90 hours/module). Within groups of 20 students, tutors performed communication witn chats twice weekly and discussion forums by topic.. A total of 88 students were enrolled. Drop-out rate was 35.2 percent (n = 31). Additional 10 students did not pass the exams. In total, 47 students completed the

Conclusions. Health economics training through distance learning is a more efficient use of resources with good results.

VP89 A Preliminary Equity Checklist To Support The HTA Process

Maria Benkhalti (maria.benkhalti.ciussse-chus@ssss.gouv.qc.ca) and Pierre Dagenais

Introduction. There is increased recognition of the need to include equity considerations in HTA. Despite this, a recent World Health Organization report has found that this is seldom the case. We developed a preliminary version of an equity checklist in the hopes that tangible guidance will increase such analyses in the future and contribute to smart capability building.

Methods. The checklist is based on the Equity Framework for HTA developed by Culyer & Bombard (2012). The elements presented in the framework were revised to follow the stepwise HTA process. A comprehensive literature search was used to update and complete the elements. The checklist was then piloted in an HTA in 2018 and subsequently further refined through a workshop during a national HTA conference in Canada.

Results. These steps resulted in a 27-item checklist leading to consider different aspects of the three major phases in the HTA process. The scoping phase brings questions relative to defining and contextualizing equity, such as highlighting potential minority groups and including vulnerability factors in the logic model. The development phase leads methodological approaches facilitating the analysis of inequities as well as considering contextual realities leading to inequities. The last phase, drafting of recommendations, aims to be aware of the evidence synthesis approaches as well as the various aspects to ensure recommendations consider existing inequities and avoid contributing to their development.

Conclusions. Given the essence of HTA to protect health by ensuring optimal technologies and interventions are adopted to the benefit of all system users, the consideration of inequities should constitute an integral part of its process. The use of a pragmatic and simple checklist to aid the planning of an HTA could contribute to greater consideration of inequities in the future. A movement in this direction could also lead to greater methodological developments for health equity analysis in HTA.

VP90 Which Matching Adjusted Indirect Comparison Method Is Best?

Jonathan Alsop (jonathan.alsop@numerus.com), Lawrence Pont and Martin Scott

Introduction. Matching adjusted indirect comparison (MAIC) methods are extremely useful when conducting ITCs, as they reduce baseline imbalances between studies, particularly upon patient characteristics that are confounded with treatment. The standard approach when conducting MAIC is that proposed by Signorovitch et al. (2010). However, there are newer, and potentially better, methods available.

Methods. Three different MAIC methods (Signorovitch, Entropy Balancing, Polynomial Weighting) were compared using multiple phase 3 RCTs conducted in Diabetic Retinal Edema. The