multiple courses of TACE is difficult to ascertain since additional courses may be prescribed under a patient-specific treatment protocol or due to non-optimal tumor response. Nonetheless, mean survival after discontinuing TACE was relatively similar regardless of number of treatments received.

PODIUM SESSION I: CONCEPTUAL PAPERS

CP1 ADJUSTING FOR INFLATION IN ECONOMIC EVALUATIONS OF HEALTH TECHNOLOGIES: ARE WE DOING IT WRONG?

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OBJECTIVES: Economic evaluations of health technologies typically require consideration of future costs. Conventionally, all costs are represented in ‘real terms’ by adjusting for inflation. Future costs are then discounted to account for time preference. Although much has been written on the practice of discounting, health economists have paid surprisingly little attention to the issue of appropriately adjusting for inflation. This paper argues that the conventional approach to adjusting for inflation in economic evaluations of health technologies is inappropriate. METHODS & RESULTS: The conventional approach follows the recommendations of the Washington Panel. Costs must be converted to “constant dollars” using a single inflation rate representing the rate of “general price inflation”. However, “if the prices of the goods in question change at a rate different from general price levels, this variation should be reflected in the adjusted amount”. Some analyses therefore use the ‘Medical Component’ of the Consumer Price Index (CPI-M), or an equivalent measure. However, as the rate of inflation follows the general rate of inflation, it is not necessarily appropriate to use a specific rate to adjust future costs. Inflation is not a single rate, but is a function of the specific market where the costs are incurred. Instead, the use of appropriate criteria for the selection of representative languages is of key importance. CONCLUSIONS: The conventional approach to adjusting for inflation is inappropriate. A solution is to estimate a unique time profile for each component. Models routinely used by financial analysts may provide an example for how this projection can be done in practice.

CP2 THE NOTION OF REPRESENTATIVE LANGUAGES IN THE CONTEXT OF TRANSLATABILITY ASSESSMENT

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BACKGROUND: While current best-practices in PRO development include evaluation of the relative ease of translation for global trial use prior to instrument finalization, methodologies for this translatability assessment (TA) vary greatly. In the proposed approach, representative languages (RLs) are selected to assess the translation difficulty of PRO concepts without the time and cost of evaluating multiple languages with shared characteristics. METHODS: In the genealogical approach employed by linguists, languages sharing a common ancestor that became separated by geography or socio-political boundaries will evolve in distinct ways, resulting in sets of languages (families) with common linguistic features (e.g. word order, phrasal structure, morphology, lexicality, etc.); because of this relative similarity within language groups, efficiency can be gained by assessing translatability with sets of appropriately-selected RLs, which can in turn predict translation problems likely to affect others in their linguistic families. As such, use of a single RL should be considered as key importance in this context. Selection of RLs should be based on linguistic properties and other features salient to outcomes research. A family or group of languages may also be defined by shared characteristics that are not purely linguistic in nature. Features such as geographic and cultural (religious/dietary/social) aspects, number and distribution of speakers worldwide, and criteria related to health care utilization or study implementation should be considered in the definition of language families/groups and in the selection of RLs. CONCLUSIONS: Despite differences that undeniably exist between individual languages, limited information can be gained by the repetitive assessment of prospective translation difficulty within groups sharing similar characteristics. Instead, the use of a representative language to assess translation difficulty for a related group of languages provides greater resource efficiency and more effective application of TA in providing important feedback prior to finalization of newly developed measures.

CP3 PIECEWISE MODELING OF TIME-TO-EVENT DATA WITH FLEXIBLE PARAMETERIZATION OF COVARIATES AND EFFECTS

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Projection of time-to-event distributions is necessary to obtain accurate estimation of life expectancy, or prediction of event times for economic models. Parametric survival analysis techniques are typically used, and can represent a broad range of shapes. In some cases, however, the best distributional fit fails to capture the variation in hazards over the entire time span, or it provides acceptable fit to the data but yields clinically implausible projections (e.g., constant hazard of death). More flexible techniques, like piecewise exponential models, can overcome these issues but remain generally underused. In piecewise models, the time axis is divided into contiguous segments with a common parametric distribution assumed within each segment, but values of the parameters are allowed to vary. In addition to greater flexibility, this framing allows inclusion of time-dependent parameters in time-dependent effects. Two important considerations are the number and placement of divisions on the time axis, and the choice of the common distribution. Examination of the cumulative and log-cumulative hazard plots are useful in assessing these issues. For instance, the number/placement of divisions for a piecewise-exponential model could be determined visually such that the points within each division of the cumulative hazard plot follow a linear pattern. The same can be done with log-cumulative hazard function for a piecewise-Weibull model. Although piecewise-exponential models can be made progressively more flexible by increasing the number of segments to capture even very complex patterns, the hazard for the last segment can be limiting for projection. Thus, models based on Weibull distributions may be more appropriate, and possibly achieve similar fit with fewer segments. The application of these approaches is illustrated using king optimising strategies (e.g., grid search for placement of divisions) and use of fit statistics to select distributions.

CP4 MEASURING HEALTH OUTCOMES IN THE ABSENCE OF RIGOUR: WILLFUL IGNORANCE OR DELIBERATE MALPRACTICE?

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OBJECTIVES: Conventional approaches to measuring health outcomes require the availability of relevant health measures that are applicable to the target population. However, the availability of such measures may be limited. There are different approaches to measuring health outcomes in the absence of rigour. Using these approaches inappropriately may lead to incorrect conclusions. This paper identifies and discusses the main issues involved in these approaches and provides examples for each of them.

METHODS AND RESULTS: A selection of 10 examples is provided to illustrate the consequences of not using proper outcome measures. Examples include the use of a single indicator to assess the impact of a health technology, the use of a subjective preference measure instead of a more objective measure, and the use of a non-representative sample to assess the impact of a health technology. The examples are used to highlight the potential for biased conclusions.

CONCLUSIONS: The selection of appropriate outcome measures is critical to the accurate evaluation of health technologies. The use of alternative and inappropriate outcome measures may lead to incorrect conclusions. The selection of appropriate outcome measures should be based on the availability of relevant measures, the potential for biased conclusions, and the potential for improved decision-making.

PODIUM SESSION II: HEALTH CARE STUDIES – EXPENDITURE OR REIMBURSEMENT STUDIES

HCL ORPHAN DISEASE DRUG COSTS IN THE UNITED STATES: ASSESSMENT OF FUTURE IMPLICATIONS ON HEALTH SYSTEM ACCESS

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OBJECTIVES: With an increasing number of orphan disease (OD) drugs in development, the objective of the current study is to assess launch pricing trends of orphan drugs in the U.S. From this pricing assessment, implications and effects of increasing orphan drug prices on US managed care payer access is discussed. METHODS: Non-cancer OD approvals between 2003 and 2012 were extracted from the FDA Orphan Products database. Oncology and acute indications were excluded due to the confounders of acute and chronic treatments. Wholesale acquisition cost drug prices were collected from Medispan-PriceRx for product launch year. Annualized drug costs were calculated using the product label and consistent assumptions on weight-based dosing. Total costs were adjusted to 2012 dollars using the CPI. RESULTS: From 2003-2012, 33 ODs gaining U.S. market approval were included in the present analysis, with 36% of the drugs approved in 2011 and 2012. Launch pricing trends indicate that average launch price of ODs has increased 107% to $276,471/year during the examined time period. In 2012, 4 of 6 new ODs were priced between $294,000 and $295,000. CONCLUSIONS: The OD approvals and prices have grown substantially since 2003, however, those costs identified that the most appropriate patients through collaborative work with thought leaders and manufacturers. Payer investment should be made in patient management programs to ensure clinical benefit is delivered. The OD regulatory mechanism encourages manufacturers to invest modestly in clinical development and assign ultra-premium prices. Manufacturers may be