Scale (KPS), Spitzer QOL-Index, Expanded Disability Status Scale (EDSS), and the Hamilton Rating Scale for Depression (HAM-D) were evaluated in terms of: conceptual framework; patient, literature and clinician contribution to development; process for deriving items; content validity; item reduction; linguistic and cultural adaptation; scoring procedure; internal validity, and ability to differ change. RESULTS: FDA discourages the use of CROs for symptoms that can only be known to the patient. However, clinical signs are usually observed and interpreted by the clinician. Despite the widespread use of the KPS as a classification of functional status in patients with cancer, there is little data supporting the development, content validity, and statistical measurement properties of the scale. Among other critiques, the EDSS has questionable inter-rater reliability. Although the Spitzer QOL-index was developed with patient input and has documented measurement properties, the use of proxy for QOL/HRQOL evaluations is widely discouraged. The HAM-D is considered the ‘gold standard’ of depression rating scales with good psychometric properties, but with questionable external validity.

CONCLUSIONS: The suitability of the selected CROs as endpoints for regulatory approvals varied widely. CROs are essential and complementary primary and secondary endpoints for drug evaluations for the purpose of FDA regulatory submissions, it is likely that sponsors will face increasing FDA scrutiny of CRO endpoints for the purpose of label claims.

CONCEPTUAL PAPERS & RESEARCH ON METHODS – Cost Methods

PMC2 REVIEW OF PHARMAECONOMIC RECOMMENDATIONS FOR THE DEFINITION OF A SOCIETAL PERSPECTIVE

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OBJECTIVES: To compare the ways pharmaeconomic (PE) guidelines in European and North American countries define the societal perspective for economic evaluations. METHODS: Full-text country-specific PE guidelines were obtained via the ISPOR website and where possible cross-referenced with Health Technology Assessment (HTA) guideline recommendations. The following countries were selected for comparison: Belgium, Canada, Finland, France, Italy, Norway, the Netherlands, Portugal and Sweden. The cost categories considered for the societal perspective were assessed and compared between country guidelines. RESULTS: PE guidelines from eight of the selected countries identified specific cost categories for the societal perspective. Guidelines agreed that the direct costs associated with this perspective included costs to the health service, costs to other publicly funded services and costs to patients/family. Costs of time lost by family/unpaid carers was considered by 6/8 guidelines, while indirect costs were considered by 7/8 guidelines. There were differences in the number of indirect costs considered; costs of time lost by patients was only considered by 2/8 guidelines. Intangible costs were considered by 4/8 guidelines, although it was generally accepted that these should be accounted for in the outcome measurement rather than through costs. Productivity loss was considered as an indirect cost by nine guidelines; however the preferred method of derivation differed between guidelines: 2/9 guidelines preferred the Friction Cost Method (FCM) and 3/9 guidelines preferred the Human Capital Method (HCM). Moreover, there were differing views regarding the types of productivity loss to include: Portugal recommended considering employee-related loss only, whereas Canada also included loss to the employer associated with hiring new staff. CONCLUSIONS: There are subtle differences between the ways the societal perspective is defined by PE guidelines in Europe and North America. This could be benchmarked when devising evidence generation activities to support economic modelling, and may ultimately impact the outcome of HTA decisions.

PMC3 REVIEW OF COST EFFECTIVENESS ANALYSES THAT INCORPORATE NUMBER NEEDED TO TREAT/HARM VALUES

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INTRODUCTION: Number needed to treat/harm (NNT/NNH) values are universally understood and applied by clinical decision makers. Therefore providing these values alongside cost effectiveness analysis (CEA) results may increase the relevance and clarify clinical implications of CEAs to decision makers. OBJECTIVES: To review the literature of CEA studies that incorporate NNT/NNH values. METHODS: We searched PubMed using the Mesh term "cost-benefit analysis" and "number needed to treat + "number needed to harm," "NNT," or "NNH." We included CEA studies and studies discussing relationships between NNT/NNH and quality adjusted life years (QALYs) published in English. RESULTS: There were 102 publications identified. Of these 47 provided both CEA and NNT/NNH results. There were 45 (91.5%) studies published in clinician-focused practice journals, 2 (4.3%) in policy journals, and 2 (4.3%) in economic journals. The CEA incorporated NNT/NNH directly as part of the CEA ratio in 23 (48.9%) studies and CEA was separately stated from NNT/NNH in 14 (29.8%) studies. The CEA was expressed as cost per QALY distinctly from NNT/NNH in 10 (21.3%). The focuses of the articles were disease treatments in 28 (59.5%) studies, disease prevention in 15 (31.9%), and patient education or disease management in 2 (4.3%) each. Also, there were 4 studies regarding relationships between NNT/NNH and QALYs. CONCLUSIONS: We found in a majority of articles incorporating NNT/NNH into CEA were published in clinical practice journals and most involved comparisons of specific disease treatments. Incorporating NNT/NNH into CEA results may improve relevance to clinical decision-making, but further research is needed regarding how they are being integrated. One alternative may include weighting NNT/NNH values for different outcomes in terms of QALYs.

PMC4 THE DERIVATION OF TRICARE SPECIFIC CONSUMER PRICE INDICES FOR PRESCRIPTION DRUGS

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OBJECTIVES: One of the chronic issues in pharmaceutical utilization management is the dearth of accurate price benchmarks available to establish pharmacy pricing performance. This paucity of appropriate benchmarks is further complicated for organizations, such as TRICARE, who have reason to believe that their demographics and medical prescription drug market baskets differ. Thus the need to obtain an accurate assessment of organizational performance, organization-specific benchmarks need to be developed. METHODS: A unique approach to developing organization-specific benchmarks is underway in a joint venture between the TRICARE Pharmacoeconomic Operations Center and the TRICARE Management Activity, Falls Church, VA. CONCLUSIONS: We will compare actual TRICARE drug prices to organized sets of killer benchmarks (BLS), the primary method is to match retail pricing information provided by BLS and portfolio information provided by TRICARE to construct organization specific benchmarks that can be tracked over time. Variables from the Consumer Price Index for Prescription Drugs (CPI-Rx) are transferred to the POD as specified in an inter-governmental memorandum of understanding. To form an overall TRICARE-CPI-Rx index for a specific month, all TRICARE prescription data are aggregated by specific drug and the number of prescriptions is computed for each drug for specific month using a file extracted from the Pharmacy Data Transaction Service (PDTS). CPI-Rx prices then aggregated by same list of unique drugs used in the TRICARE sample then averaged in a separate file. The files are then merged using NDC codes as the key. The index is computed using the average CPI-Rx price for each specific drug multiplied by the TRICARE N for the corresponding drugs then averaged. CONCLUSIONS: TRICARE spends roughly $7.5 Billion annually for prescription drugs. Thus the development of these indices, we can better gauge our cost containment priorities and efforts. This will help us determine if increases in costs are due to general drug price inflation specific to our unique market basket of drugs.

PMC5 VALIDATING A WEB-BASED INCREMENTAL COST-EFFECTIVENESS SOFTWARE PROGRAM THAT USES A MARKOV TRANSITION MATRIX ANALYSIS MODEL

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OBJECTIVES: Commercial software can be expensive when conducting pharmaeco-economic analyses. We developed a free web-based software program, which incorporates Markov transition probabilities to compare the cost-effectiveness of any two treatments. The web-based software program was based on the model described in a decision modeling for health economic evaluation textbook, edited by A. Briggs. This Markov web-based software program calculates the incremental cost-effectiveness based on Markov matrices using multi-state transition probabilities, along with corresponding Markov state costs and utilities and graphically displays the results, using JavaScript algorithms and is available free at www.healthstrategy.com. The variable inputs for two treatment options include state transition probabilities, number of cycles, cost per state, and utility per state. The software creates a plot of incremental costs versus incremental utilities in cost-effectiveness quadrants; and with death as an absorbing state, also graphs life expectancy curves for two treatment comparisons. The objective of this study was to validate this free web-based software. METHODS: The Excel spreadsheet structure and data downloaded from the web for the specific example described in the modeling textbook were used as the reference case. RESULTS: For the example used, considering four transition states for each therapy option, and 20 cycles with no discounting, the MS Excel spreadsheet model versus the web-based JavaScript software compared as follows: average incremental US dollar costs: ($67701 vs. $67853), average incremental utility: (5.89 vs. 5.90) and average incremental cost-effectiveness ratio: ($11500 vs. $11494). CONCLUSIONS: This free web-based Markov matrix JavaScript program gives similar results as the MS Excel spreadsheet model. With this free software, the user can input their own parameters, and generate incremental costs, incremental utilities, life expectancy curves, and incremental cost effectiveness ratios. This free web-based software has potential benefit as an educational tool for students and health professionals interested in exploring these analytical approaches.

PMC6 USE OF CLINICAL SIMULATION CENTERS TO CONDUCT PATIENT-CENTERED TIME-AND-MOTION SIMULATIONS AS A BASIS FOR ECONOMIC ANALYSIS

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BACKGROUND: Patient-centered professional practice and technology assessment research performed in health professional schools’ clinical simulation centers is a novel concept. Opportunities can be created for multidisciplinary collaboration relative to evaluation of medication regimen and device complexity. Micro-level costs can then be assigned and economic analysis conducted in a more precise fashion than in traditional clinical trials or database outcomes analysis. METHODS: One university’s pharmacy and nursing schools’ clinical simulation centers were used in two separate projects to conduct time-and-motion and activity-based costing analyses specific to