

## Value in Health Volume 18, Issue 8

### The following articles will be included in the December 2015 issue of Value in Health (Volume 18, Issue 8):

The current issue of Value in Health contains two articles related to benefit-risk assessment methodologies, with a particular focus on conducting these assessments earlier on in the product life cycle. Early benefit-risk assessment allows decision makers to make informed coverage and management decisions when new products enter the market.

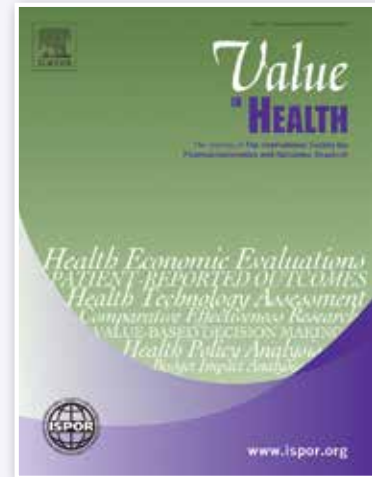
This issue also contains two articles that describe proposed methodological advancements for measuring quality of life in Multiple Sclerosis. New or improved tools for measuring patient preferences and quality of life provide more scientifically valid and patient-centered approaches for assuring that comparative effectiveness research and economic evaluations reflect relevant and impactful measures.

In addition to these methodological articles, there are a wide array of empirical and policy pieces, as well as two brief reports.

We hope you enjoy this issue of Value in Health!

Kind regards,

Michael F. Drummond, MCom, DPhil and C. Daniel Mullins, PhD, Co-Editors-in-Chief, Value in Health



### FEATURED ARTICLES:

#### Prospective Benefit-Risk Monitoring of New Drugs for Rapid Assessment of Net Favorability in Electronic Health Care Data

In this article, Joshua Gagne et al. examine Benefit-risk assessment (BRA) metrics for prospective monitoring of new drugs in electronic health care data.

### ISSUE HIGHLIGHTS:

#### Decision-Maker Commentary

##### HIQA's CEA of Breast Screening: Pragmatic Policy Recommendations are Welcome, but ACERs Reported as ICERs are Not

In this article James O'Mahoney et al. comment on the Health Information and Quality Authority's (HIQA's) reporting of average cost-effectiveness ratios (ACERs) as incremental cost-effectiveness ratios (ICERs) in breast cancer screening.

#### Economic Evaluation

##### The Suitability of Endpoint Designs for Health Technology Assessment in Chronic Pain Studies

William Dunlop et al. identify the pain instruments and study endpoints most commonly used in clinical trial settings and provide insight into the extent that outcome measures in clinical studies are meeting payer needs.

#### Patient-Reported Outcomes

##### Reliability and Validity of the Work Instability Scale for Rheumatoid Arthritis

In this article, Dennis Revicki et al. evaluate the psychometric properties of the Rheumatoid Arthritis-Work Instability Scale in a clinical trial setting. (See page 42 for summary)

#### Preference-Based Assessments

##### A Quality-Adjusted Life-Year Measure for Multiple Sclerosis: Developing a Patient-Reported Health State Classification System for an MS-Specific Preference-Based Measure

In this article, Elizabeth Goodwin and Colin Green report the first stage in the development of a Multiple Sclerosis (MS)-specific preference-based measure to quantify the impact of MS and its treatment. (See page 42 for summary)

#### Comparative Effectiveness Research / HTA

##### Comparison of Benefit-Risk Assessment Methods for Prospective Monitoring of Newly Marketed Drugs: A Simulation Study

In this article, Joshua Gagne et al. compared benefit-risk assessment (BRA) methods for determining whether and when sufficient evidence exists to indicate that one drug is favorable over another in prospective monitoring.

#### Health Policy Analysis

##### Appropriateness of Prescriptions of Recommended Treatments in Organisation for Economic Co-Operation and Development (OECD) Health Systems: Findings Based on the Long-Term Registry of the European Society of Cardiology (ESC) on Heart Failure

In this study, Kine Pedersen et al. identify clinical variables and health systems characteristics associated with incomplete guideline application in drug treatment of patients with chronic health failure across 15 countries.

#### Methodology

##### Using Generalized Linear Mixed Models to Evaluate Inconsistency within a Network Meta-analysis

Yu-Kang Tu proposes an arm-based approach to evaluating the design-by-treatment inconsistency in linear mixed models, which is flexible in modeling different types of outcome variables.

#### Systematic Reviews

##### Health Utility Values Associated with Surgical Site Infection (SSI): A Systematic Review

In this review, Adrian Gheorghe et al. summarize the evidence base on surgical site infection (SSI) health utility values reported in patient-level studies and decision models.

#### Brief Reports

##### Health-Related Quality of Life and Health Utilities in Metastatic Castration Resistant Prostate Cancer: A Survey Capturing Experiences from a Diverse Sample of UK Patients

This report by Cicely Kerr et al. aimed to collect health related quality of life (HRQL) data in men with metastatic castration-resistant prostate cancer (CRPC) stratified by disease states. (See page 42 for summary)

For all articles in this issue, and to see what services Value in Health provides for its authors see: [http://www.ispor.org/valuehealth\\_index.asp](http://www.ispor.org/valuehealth_index.asp).

## Spotlight on Value in Health

This is the first in a series of brief overviews of select articles published in Value in Health. We hope that by highlighting key aspects of highlighted articles, readers will be able to identify research that is most relevant to their fields. We also recognize that many ISPOR members may wish to be kept abreast of the most salient points of articles that are outside their areas when they are not able to read each issue of Value in Health in its entirety.

Value & Outcomes Spotlight would like to thank the authors for contributing their overviews to the ISPOR membership.

From Volume 18, Issue 8 (December 2015):

### Patient-Reported Outcomes

#### Reliability and Validity of the Work Instability Scale for Rheumatoid Arthritis (pp. 1008-1015)

Patients with rheumatoid arthritis (RA) are at high risk for losing their employment due to disease-related symptoms and disability. Measuring risk for work disability provides information on the risk of losing employment. The current study evaluated the psychometric properties of the Rheumatoid Arthritis-Work Instability Scale (RA-WIS). Analyses were conducted using data from a 56-week clinical trial of early RA patients. Patient-reported outcomes included the RA-WIS, Health Assessment Questionnaire (HAQ), Rheumatoid Arthritis Quality of Life Questionnaire (RAQoL), and Global Assessment of Disease Activity and Pain, collected over the 56 week study. RA-WIS data were analyzed for reliability, validity, and responsiveness. All analyses assessed RA-WIS total scores. Among 148 patients who were analyzed, 56% were female with mean age of 46.8 years. RA-WIS demonstrated excellent internal consistency ( $\alpha=0.89$ ) and test-retest reliability (ICC=0.91). Moderate to strong correlations were seen between RA-WIS total scores and the HAQ, Global Assessment of Disease Activity, Pain and RAQoL scores ( $r = 0.47$  to  $0.81$ ,  $p<0.0001$ ). Mean RA-WIS total scores varied by clinical severity based on the DAS28, HAQ-DI and Physician Global Assessment of Disease Activity (all  $p<0.05$ ). Mean Baseline to Week 24 RA-WIS total change scores were significantly different among American College of Rheumatology responder and non-responder groups ( $p\leq 0.0001$ ) and between DAS28 remission and non-remission status groups ( $p<0.001$ ). These findings support reliability, validity and responsiveness of the RA-WIS for evaluating work disability in RA patients. The psychometric evidence suggests that the RA-WIS may be an acceptable endpoint for clinical trials comparing treatments for early RA patients.

### Preference-Based Assessments

#### A Quality-Adjusted Life-Year Measure for Multiple Sclerosis: Developing a Patient-Reported Health State Classification System for an MS-Specific Preference-Based Measure (pp. 1016-1024)

This paper presents a new condition-specific measure for use in eliciting preferences for health states: the eight-dimensional Multiple Sclerosis Impact Scale (MSIS-8D). Preference-based measures (PBMs) provide a standardised "classification system" for describing health states, based on a relatively small number of dimensions and levels, and provide a preference weight for each of the health states described. Increasingly, generic PBMs are employed to derive quality-adjusted life-years (QALYs) for use as the primary economic endpoint in cost effectiveness analyses. These generic measures, however, may not adequately capture the aspects of health-related quality of life (HRQoL) that are important

to people with specific conditions, such as multiple sclerosis (MS), and this may adversely affect the results of economic evaluations and the adoption of health technologies. This research aimed to develop a condition-specific PBM for MS. The article describes how the MS Impact Scale (MSIS-29), a commonly used measure of HRQoL in MS, was selected for use and how it was adapted from a 29-item questionnaire to an 8-dimensional classification system amenable for health state valuation. A high quality longitudinal data set was analysed using standard psychometric methods and Rasch analysis to confirm the dimensional structure of the MSIS-29 and to select items for inclusion in the MSIS-8D, providing a robust foundation for this new measure. The estimation of preference weights for the MSIS-8D is presented in an accompanying paper. This new preference-based measure provides a new source of data on health outcomes and extends the methods available to inform economic evaluations in an MS setting.

### Brief Report

#### Health-Related Quality of Life and Health Utilities in Metastatic Castrate-Resistant Prostate Cancer: A Survey Capturing Experiences from a Diverse Sample of UK Patients (pp. 1052-1057)

This brief reports health-related quality of life (HRQL) data, including health utilities, elicited from men with metastatic castration-resistant prostate cancer (CRPC). While there is some published information on the HRQL impact and utility of CRPC, it is lacking for metastatic CRPC patients who have progressive disease in the period before chemotherapy becomes clinically indicated, particularly UK patients. In this study, HRQL data were collected via an online survey from four metastatic CRPC sub-groups reflecting disease states along the treatment pathway: asymptomatic/mildly symptomatic before chemotherapy; symptomatic before chemotherapy; receiving chemotherapy; post chemotherapy. UK participants were recruited via relevant charities, support groups and a specialist recruitment agency. HRQL data were collected using validated prostate cancer-specific measures (EORTC QLQ-C30 and PR-25). Utilities were estimated using two methods, providing the opportunity to compare the performance of a generic preference-based measure (EQ-5D-5L) with a condition specific measure (EORTC-8D, scored from QLQ-C30 data). While some systematic differences were noted between the EQ-5D and EORTC-8D derived utilities, the pattern of utility and HRQL scores across the sub-groups was consistent, showing relatively better HRQL and higher utility in the mCRPC subgroup that were asymptomatic/mildly symptomatic before chemotherapy. The report is intended as a useful resource for groups undertaking analyses of different treatment interventions in this disease setting as well as clinicians and payers interested in understanding changing HRQL burden along the metastatic CRPC treatment pathway.

## Value in Health Regional Issues Volume 8

### The following articles will be included in *Value in Health Regional Issues Volume 8* (December 2015)



It is a pleasure to present the new Latin America (LA) region issue of *Value in Health Regional Issues* (December 2015; Volume 8). In this issue, we have an overview of the significant progress our field is making in our region. There are 18 studies from the northern to the southern parts of LA, with several papers from Brazil, Chile, Colombia, Mexico, Guatemala, Uruguay, and others with a regional focus.

This issue deals with an interesting scope of topics. Examples include avoidable mortality, new drugs inclusion to a national benefit package, the implementation of patient-reported outcomes, consequences of chemotherapy wastage, equity in access to oncologic drugs, and unexplained variation in cesarean deliveries.

We hope you enjoy this issue; we will continue to strive to contribute to the use of sound principles for decision making in Latin America.

Cheers,

**Federico Augustovski, MD, MSc, PhD**, Co-Editor-in-Chief, *Value in Health Regional Issues*

### HIGHLIGHTED ARTICLE:

#### **Impacto de Una Política De Equivalencia Terapéutica en el Precio de Medicamentos en Chile**

In this study, Carlos Balmaceda and Manuel A. Espinoza show the effect of bioequivalence enforcement on drug prices in Chile.

### ISSUE HIGHLIGHTS:

#### **ECONOMIC EVALUATION**

##### **Medical Costs Related to Enzyme Replacement Therapy for Mucopolysaccharidosis Types I, II and VI in Brazil: A Multicenter Study**

In this article, Fernanda Hendges de Bitencourt et al. report results of 27 patients receiving enzyme replacement therapy (ERT) and show that though patients on ERT have less disease-related costs, these are relatively small compared to the very high cost of ERT.

#### **PATIENT-REPORTED OUTCOMES**

##### **Patient-Reported Outcomes in Latin America: Implementation in Research and Role in Emerging HTA Systems**

This interesting paper by Randall Winnette et al. reviews the use of patient-reported outcomes (PROs) in Latin America, and shows that PROs are increasingly important in the region.

#### **COMPARATIVE EFFECTIVENESS RESEARCH / HTA**

##### **Equidad en el Acceso a los Medicamentos Oncológicos de Alto Costo en Uruguay**

This study, authored by González-Vacarezza et al. evaluates an essential dimension of the health system—equity—by evaluating the access to high cost oncology medicines reimbursed by the Uruguayan National Health System.

#### **METHODOLOGICAL ARTICLES**

##### **Costing Dengue Fever Cases and Outbreaks: Recommendations from a Costing Dengue Working Group in the Americas**

This study by Dagna Constenla et al. tackles an important neglected disease very relevant to Latin America (Dengue fever), and reports the output of an expert panel convened to develop standards for costing this disease in order to be better prepared for future strategies.

#### **SYSTEMATIC REVIEWS**

##### **Epidemiology of Malaria in Latin America and the Caribbean from 1990 to 2009: Systematic Review and Meta-Analysis**

In this article, Ariel Estaban Bardach et al. report the first systematic review of malaria, in which they evaluate the burden of disease in 21 Latin America and the Caribbean countries.

For all articles in this issue, or to submit an article, see: <http://www.ispor.org/publications/VIHRI/index.asp>.