

## Value in Health Volume 20, Issues 1 & 2



### From the Editors

In the January 2017 issue of *Value in Health*, we publish the reports of two of ISPOR's Good Research Practices Task Forces. The first paper contains part two of the Task Force report on clinician-reported outcome (ClinRO) assessments. The first part, which was published in the July/August 2015 issue, provided an overview of clinical outcome assessments (COAs), including definitions important for an understanding of COA measurement practices. This paper defines three types of ClinRO assessments (i.e., readings, ratings, and clinician global assessments) and describes emerging good measurement practices in their development and evaluation.

The second Task Force report discusses good research practices in mapping to estimate health state utility from non-preference-based outcome measures, such as descriptive quality-of-life measures. Ideally, clinical studies designed to assess the effectiveness of health technologies would include outcome measures that are directly linked to health utility in order to calculate quality-adjusted life years (QALYs). Often this does not happen, and even where it does, clinical studies may be insufficient for a cost-utility assessment. Mapping can solve this problem.

In addition, we include an additional paper from the Economics of Making Choices on the Journey to Universal Health Care Coverage themed section from our December 2016 issue. In this paper entitled, *Should Countries Set an Explicit Health Benefits Package? The Case of the English National Health Service*, the authors examine the arguments for and against setting an explicit benefits package, and discuss the circumstances in which increased detail in specification are most appropriate.

In the February 2017 issue, we cover the topic of value assessment frameworks. A number of approaches for assessing the value of health care have been used in different jurisdictions for a number of years, most notably the quality-adjusted life year (QALY). However, the topic has recently gained prominence in the United States (US), with several independent groups proposing alternative value assessment frameworks.

This issue, edited by Jalpa Doshi, PhD and Richard Willke, PhD, explores a number of facets of assessing the value of health care, considering both the recent attempts in the US and experience from elsewhere. The issues discussed include: 1) the strengths and weaknesses of the current frameworks proposed for the US; 2) the arguments for expanding the concept of value beyond the QALY; 3) the consideration of affordability alongside value; and 4) the approaches for combining varying stakeholder preferences in the assessment of value. Overall, this issue provides a timely and comprehensive analysis of the key issues in the development and use of value assessment frameworks in health care.

Finally, because of the increased amount of quality content available to *Value in Health*, we will be publishing 8 regular article-based issues in 2017, rather than the 6 we have published in the past. This change will enable us to publish papers in a timelier manner, and will help bridge the gaps in our regular publishing schedule due to the publication of the two abstract issues from ISPOR's international scientific meetings.

Kind regards,

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

**The following Editors' Choice articles (beginning on pp. 29) will be included in the January & February 2017 (Volume 20; Issues 1 & 2) issue of Value in Health.**

Summaries of selected Editors' Choice articles begin on page 31.

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).

## Value in Health Issue Highlights

### JANUARY 2017 – EDITORS' CHOICE:

#### THEMED SECTIONS

#### **VALUE TO DECISION MAKERS OF EVALUATIONS OF PERSONALIZED/PRECISION MEDICINE: APPLICATIONS TO OTHER EMERGING TECHNOLOGIES**

**Guest Editor:** Kathryn A. Phillips

#### **Estimating Preferences for Complex Health Technologies: Lessons Learned and Implications for Personalized Medicine**

Deborah A. Marshall, Juan Marcos Gonzalez, Karen V. MacDonald, F. Reed Johnson

#### **Decision-Making on Medical Innovations in a Changing Health Care Environment: Insights from Accountable Care Organizations and Payers on Personalized Medicine and Other Technologies**

Julia Trosman, Christine B. Weldon, Michael P. Douglas, Patricia A. Deverka, John Watkins, Kathryn A. Phillips

#### **"What Goes Around Comes Around:" Lessons Learned from Economic Evaluations of Personalized Medicine Applied to Digital Medicine**

Kathryn A. Phillips, Deborah A. Marshall, Michael P. Douglas

#### **Lessons Learned When Introducing Pharmacogenomic Panel Testing into Clinical Practice**

Marc B. Rosenman, Brian Decker, Kenneth D. Levy, Ann M. Holmes, Vicky Pratt, Michael Eadon

#### **ECONOMICS OF MAKING CHOICES ON THE JOURNEY TO UNIVERSAL HEALTH CARE COVERAGE**

**Guest Editors:** Kalipso Chalkidou and Anthony J. Culyer

#### **Should Countries Set an Explicit Health Benefits Package? The Case of the English National Health Service**

Peter C. Smith, Kalipso Chalkidou

The paper examines the arguments for and against setting an explicit benefits package, and discusses the circumstances in which increased detail in specification are most appropriate. The authors use the English National Health Service as a case study, based on institutional history, official documents, and research literature.

#### ISPOR KNOWLEDGE PRODUCTS

#### **Clinician-Reported Outcome Assessments of Treatment Benefit: Report of the ISPOR Clinical Outcome Assessment Emerging Good Practices Task Force**

John H. Powers, Donald L. Patrick, Marc K. Walton, Patrick Marquis, Stefan Cano, Jeremy Hobart, Maria Isaac, Spiros Vamvakas, Ashley Slagle, Elizabeth Molsen, Laurie B. Burke

This is the second of two reports by the ISPOR Clinical Outcomes Assessment—Emerging Good Practices for Outcomes Research Task Force. The first report provided an overview of clinical outcomes assessments (COAs), including definitions important for an understanding of COA measurement practices. This report focuses specifically on issues related to clinician-reported outcome assessments.

#### **Mapping to Estimate Health State Utility from Non-Preference-Based Outcome Measures: An ISPOR Good Practices for Outcomes Research Task Force Report**

Allan Wailoo, Monica Hernandez-Alava, Andrea Manca, Aurelio Mejia, Joshua Ray, Bruce Crawford, Marc Botteman, Jan Busschbach

Mapping bridges the evidence gap between available evidence on the effect of a health technology in one metric and the requirement for decision makers to express it in a different one (QALYs). This Good Practices for Outcomes Research Task Force report is unique because it takes an international perspective, is comprehensive in its coverage of the aspects of mapping practice, and reflects the current state of the art.

#### ECONOMIC EVALUATION

#### **The Burden of Obesity on Diabetes in the United States: Medical Expenditure Panel Survey, 2008-2012**

(See summary on page 31)

#### PREFERENCE-BASED ASSESSMENTS

#### **The Influence of Genotype Information on Psychiatrists' Treatment Recommendations: More Experienced Clinicians Know Better What to Ignore**

This study applies attribute non-attendance to medical decision making.

## **COMPARATIVE EFFECTIVENESS RESEARCH / HTA**

### **Variation in Health Technology Assessment and Reimbursement Processes in Europe**

Ronald Akehurst, Eric Abadie, Noël Renaudin, François Sarkozy

The aims of the research reported in this paper were to provide an up-to-date snapshot analysis of the status of HTA and reimbursement systems in select European countries, and to investigate the implications of these processes, especially with regard to delays in market and patient access.

## **HEALTH POLICY ANALYSIS**

### **Priority to End-of-Life Treatments? Views of the Public in the Netherlands**

(See summary on page 31)

## **SYSTEMATIC REVIEWS**

### **Systematic Review of Health Economics Simulation Models of Chronic Obstructive Pulmonary Disease**

The purpose of this study was to review chronic obstructive pulmonary disease (COPD) models for their adherence to the best practice modeling recommendations and their assumptions regarding important aspects of the natural history of COPD.

## **FEBRUARY 2017 - EDITORS' CHOICE**

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### **THEMED ISSUE:**

#### **IMPROVING THE METHODS AND PROCESSES FOR CONDUCTING VALUE ASSESSMENTS OF HEALTH CARE INTERVENTIONS**

**Guest Editors:** Jalpa Doshi and Richard J. Willke

#### **Editorial: Advancing High-Quality Value Assessments of Health Care Interventions**

Jalpa A. Doshi and Richard J. Willke

Assessing value in health care is not a new concept; however, discussion around using value assessment frameworks to support payers, physicians, and patients in health care decision making, particularly related to prescription drugs, has increased dramatically in the United States (US). Numerous value assessment frameworks have been proposed recently by several US organizations, such as the American College of Cardiology and the American Heart Association (ACC–AHA), the American Society of Clinical Oncology (ASCO), the Institute for Clinical and Economic Review (ICER), the Memorial Sloan-Kettering Cancer Center (MSKCC), and the National Comprehensive Cancer Network (NCCN). All of these frameworks differ in their objectives, scope, approach, and the type of factors accounted for in measuring the value of new therapies. Each framework has its strengths and weaknesses, many of which have been raised by a variety of stakeholders. At the same time, additional efforts are ongoing to develop new value assessment frameworks, particularly from the patient perspective.

#### **Evaluating Frameworks that Provide Value Measures for Health Care Interventions**

Charles E. Phelps, Jeanne S. Mandelblatt, Scott D. Ramsey, Tracy Lieu

(See summary on page 31)

#### **Toward a Broader Concept of Value: Identifying and Defining Elements for an Expanded Cost-Effectiveness Analysis**

Lou Garrison, Sachin Kamal-Bahl, Adrian Towse

(See summary on page 31)

#### **Advancing Value Assessment in the United States: A Multi-Stakeholder Perspective**

Corinna Sorenson, Gabriella Lavezzari, Gregory Daniel, Randy Burkholder, Marc Boutin, Edmund Pezalla, Gillian Sanders, Mark McClellan

(See summary on page 31)

#### **Value Assessment in the Regulatory Context**

Kathleen Miller and Janet Woodcock

To assist with benefit/risk value assessments, the Center for Drug Evaluation and Research (CDER) has two ongoing initiatives: the Patient-Focused Drug Development Initiative that aims to substantially increase the role of patient voice in the regulatory process, and a transparency initiative that focuses on creating a structured framework for assessing benefit/risk.

For other Editors' Choice articles and issues, see: [http://www.ispor.org/valuehealth\\_index.asp](http://www.ispor.org/valuehealth_index.asp).

## VALUE IN HEALTH EDITORS' CHOICE ARTICLES

January 2017

### ECONOMIC EVALUATION

#### The Burden of Obesity on Diabetes in the United States: Medical Expenditure Panel Survey, 2008-2012 (pp. 77-84)

Su-Hsin Chang, Man Yee (Mallory) Leung, Nils Carlsson, Graham A. Colditz

Diabetes is one of the most prevalent and costly chronic diseases in the United States (US). This study presents the risk of developing diabetes and the annual cost of diabetes for the US general population with different degree of obesity. Data from the Medical Expenditure Panel Survey, 2008-2012, were used. This study found a >6-fold increase in the risks of developing diabetes for populations with a body mass index (BMI)  $\geq 40$  or class III obesity, compared with populations with a BMI between 18.5 and 24.9 or normal weight BMI. In terms of costs, using individuals age 50 as an example, there is a >3-fold increase in annual health care expenditures for patients with diabetes versus those without diabetes. The annual health care expenditure differentials between patients aged 50 years with diabetes versus those without diabetes were the highest for populations with at least class II obesity (BMI  $\geq 35$ ). Finally, the study highlights the importance of obesity on diabetes burden and suggests that obesity (in particular, BMI  $\geq 35$ ) is associated with a substantial increase in the risk of developing diabetes and imposes a large economic burden.

### HEALTH POLICY ANALYSIS

#### Priority to End-of-Life Treatments? Views of the Public in the Netherlands (pp. 107-117)

Sofie Wouters, Job van Exel, Rachel Baker, Werner Brouwer

Recent debates in the Netherlands on health care priority setting have focused on the relative value of gains generated by life-extending medicines (mostly new cancer drugs) for people with a terminal illness. These treatments are generally expensive, provide relatively small health gains, and therefore usually do not meet common cost per quality-adjusted life year (QALY) thresholds. Nevertheless, these drugs may be provided under the assumption that there is public support for making a special case for treatments for people with a terminal illness. This study investigated the views of the public in the Netherlands on a range of equity and efficiency considerations relevant to priority setting and examines whether there is public support for making such a special case. This was done using Q-methodology, a method to identify shared public viewpoints on a specific topic. Three shared viewpoints were discovered. Data were collected through ranking exercises conducted by 46 members of the general public in the Netherlands, including 11 respondents with personal experience with cancer. Viewpoint 1 emphasized that people have equal rights to health care and opposed priority setting on any ground. Viewpoint 2 emphasized that the care for terminal patients should at all times respect the patients' quality of life, which sometimes means refraining from invasive treatments. Viewpoint 3 had a strong focus on effective and efficient care and had no moral objection against priority setting under certain circumstances. Overall, there was little public support for the assumption that health gains in terminally ill patients are more valuable than those in other patients. This implies that making a special case for people who have only a short period of life left, does not correspond with societal preferences in the Netherlands.

February 2017

### THEME ISSUE: IMPROVING THE METHODS AND PROCESSES FOR CONDUCTING VALUE ASSESSMENTS OF HEALTH CARE INTERVENTIONS

#### Evaluating Frameworks that Provide Value Measures for Health Care Interventions (pp. 185-192)

Charles E. Phelps, Jeanne S. Mandelblatt, Scott D. Ramsey, Tracy Lieu  
Recently, a number of groups—both medical specialty societies and organizations with interest in specific diseases—have proposed alternatives to

traditional methods to value health care. Some build upon cost-effectiveness or cost-utility analysis, while others offer wholly new approaches. Potential audiences vary and the key issues can differ for patients, payers, providers, or society as a whole. To help guide potential users of these models, our paper proposes some key questions that put these models into perspective, highlighting their potential strengths, limitations, and biases; building upon previous work including “best practice” recommendations for cost-effectiveness analysis, recent ISPOR studies of multi-criteria decision models, techniques for meta-analysis for health care interventions, conflict of interest disclosure rules from medical journals, and our own experiences as health services researchers and physicians. We find significant limitations with many of the proposed alternative value models—both conceptual and methodological—as well as conflicts that potentially question their impartiality. Further work can improve these models, e.g., through use of data from electronic records and adding education about value frameworks in medical training. We intend these questions to support unified, consistent, and rigorous approaches to define the value of health care decisions in the era of modern medicine.

#### Toward a Broader Concept of Value: Identifying and Defining Elements for an Expanded Cost-Effectiveness Analysis (pp. 215-218)

Lou Garrison, Sachin Kamal-Bahl, Adrian Towse

The core elements of value as reflected in conventional cost-utility analysis are health gain (measured in quality-adjusted life years) and cost-offsets. This paper argues that—while these elements are the key drivers for most health technologies—other elements related to the value of knowing and information should be considered as expansions to this core for some technologies. Five additional elements of value related to reducing uncertainty were identified in the health economics literature: 1) a reduction in uncertainty reflecting the benefit of a companion diagnostic increasing the certainty of a patient's response to a treatment; 2) insurance value related to greater peace of mind due to protection against catastrophic health loss as well as catastrophic financial loss; 3) the value of hope for a “cure,” leading individuals to become risk-seekers in some circumstances; 4) real option value due to life extension opening up possibilities for individuals to benefit from future innovation; and 5) spillovers or externalities arising from benefits of scientific advances that cannot be entirely appropriated by those making them. Further thought and research are needed on how best to measure these elements and use them in an expanded cost-utility analysis to support coverage and pricing decisions.

#### Advancing Value Assessment in the United States: A Multi-Stakeholder Perspective (pp. 302-310)

Corinna Sorenson, Gabriella Lavezzari, Gregory Daniel, Randy Burkholder, Marc Boutin, Edmund Pezalla, Gillian Sanders, Mark McClellan

In the last few years, a number of value assessment frameworks have been developed by various organizations (e.g., ASCO, NCCN) to evaluate the costs and benefits of new health care interventions and services compared to other available treatment options. These frameworks have received both praise and criticism. This article provides an assessment of the existing value assessment frameworks and outlines ten guiding principles—reflective of the diversity of stakeholder interests, needs, and values—to improve value assessment at present and in the future in the United States. Based on our analysis, it is clear that there is no one perfect model or framework for value assessment, or even one that will garner consensus across all stakeholders. Decision makers will likely benefit from multiple value frameworks to support their decisions and ensure the availability of relevant and timely information. Despite the need to accommodate a range of frameworks, a common set of principles or guidelines, focused on ways to ensure adequate transparency, robust methods, open stakeholder engagement, and meaningful implementation, may help to ensure high-quality value assessment across different approaches. Next steps for further evolving and evaluating the proposed principles are also discussed.