The following Editors’ Choice articles appear in the May and June 2018 issues of Value in Health.
For more information, visit: www.ispor.org/valuehealth_index.asp.

May 2018

THEMED SECTION: Rare Diseases
The May 2018 issue features a themed section on rare diseases, edited by Kati Copley-Merriman. This themed section included 9 papers, plus an editorial, and discusses a number of issues on rare diseases relating to patient access.

Editorial
Rare Diseases: Addressing the Challenges in Diagnosis, Drug Approval, and Patient Access
Kati Copley-Merriman

Articles
Challenges in Research and Health Technology Assessment of Rare Disease Technologies: Report of the ISPOR Rare Disease Special Interest Group
Sandra Nestler-Parr, Daria Korchagina, Mondher Touni, Chris L. Pashos, Christopher Blanchette, Elizabeth Molsen-David, Thomas Morel, Steven Simoens, Zoltan Kaló, Ruediger Gatermann, Ken Redekop

The Problem of Rarity: Estimation of Prevalence in Rare Disease
Stéphane Auvin, John Irwin, Paul Abi-Aad, Alysia Batterby

Clinical Outcome Assessments: Use of Normative Data in a Pediatric Rare Disease
Dawn Phillips, Beth Leiro

Economic Modelling Considerations for Rare Diseases
Christopher Knight, Isobel Pearson, Ben Rothwell, Andrew Olaye

Budgetary Impact and Cost Drivers of Drugs for Rare and Ultra-Rare Diseases
Michael Schlander, Charalabos-Markos Dintsios, Afschin Gandjour

Can Severity Outweigh Smaller Numbers? A Deliberative Perspective from Canada
Monica Magalhaes

Societal Preferences for Funding Orphan Drugs in the United Kingdom: An Application of Person Trade-Off and Discrete Choice Experiment Methods
Dyfrig Hughes, Siobhan Bourke, Catrin Plumpton

Evaluating and Valuing Drugs for Rare Conditions: No Easy Answers
Dan Ollendorf, Richard Chapman, Steven D. Pearson

Patient Access to Medicines for Rare Diseases in European Countries
Mitja Kos, Andreja Detics, Igor Locatelli

June 2018

COMPARATIVE EFFECTIVENESS RESEARCH/HEALTH TECHNOLOGY ASSESSMENT
Selection of and Evidentiary Considerations for Wearable Devices and Their Measurements for Use in Regulatory Decision Making: Recommendations from the ePRO Consortium
Bill Byrom, Chris Watson, Helen Doll, Stephen Joel Coons, Sonya Eremenco, Rachel Ballinger, Marie Mc Carthy, Mabel Crescioni, Paul O’Donohoe and Cindy Howry on behalf of the ePRO Consortium.

Wearable devices offer huge potential to collect rich sources of data to provide insights into the effects of treatment interventions. However, limited regulatory guidance on the use of wearables in clinical trial programs has been published. The objective of this report is to present recommendations regarding the selection and evaluation of wearable devices and their measurements for use in regulatory trials and to support labeling claims.

ECONOMIC EVALUATION
A Transparent and Consistent Approach to Assess US Outpatient Drug Costs for Use in Cost-Effectiveness Analyses
Joseph F. Levy, Marjorie A. Rosenberg, David J. Vanness
The authors of this paper review available cost measures and propose a novel strategy that is transparent, consistent and applicable to all CEAs taking a US healthcare sector or societal payer’s perspective.

METHODOLOGY
Experiences of Structured Elicitation for Model-Based Cost-Effectiveness Analyses
Marta A Soares, Linda Sharples, Alec Morton, Karl Claxton, Laura Bojke
The authors of this paper review applications of SEE in cost-effectiveness modelling with the aim of summarizing the basis for methodological choices made in each application and record the difficulties and challenges reported in the design, conduct and analyses.
Call for Papers

Back to the Future:
A 20th anniversary issue of *Value in Health*

To mark *Value in Health*’s 20th anniversary, the Editors are commissioning articles for a “Back to the Future” theme that features topics that have been widely discussed in the journal over the past 20 years, but for which there is an exciting future agenda.

Potential topics might include, but not restricted to:

- How has the definition of value in healthcare changed over the past two decades?
- How have regulatory agencies’ views evolved regarding their role in determining value in healthcare?
- If QALYs have inadequacies, what would an alternative measure of benefit look like?
- The exponential growth in cost-effectiveness analyses suggests that their importance and impact has matured, but is there empirical evidence for that?
- If Markov models are the norm, how would we decide that we need alternative modeling approaches?
- Has the increased complexity of health economic models advanced the field by improving scientific validity or further confused decision makers?

Authors should submit manuscripts through our web-based tracking system at [https://mc.manuscriptcentral.com/valueinhealth](https://mc.manuscriptcentral.com/valueinhealth) and indicate in the cover letter that it is part of the “Back to the Future” themed section.

For more information about *Value in Health* visit [www.ispor.org](http://www.ispor.org).

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