ISPOR US 2017 Report

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20–24 May 2017

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ISPOR 22nd Annual International Meeting, Boston MA, USA

Costello Medical Consulting joined over 4,100 other delegates in attending the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) 22nd Annual International Meeting in Boston MA, USA, from 20th–24th May 2017. This year's meeting was entitled "Evidence and Value in a Time of Social and Policy Change" and included a full programme around this subject.

A number of key themes emerged at this year's conference, especially value frameworks in the US given the upcoming policy changes in US healthcare and future directions for cost-effectiveness. This report summarises the meeting's plenary sessions, along with the most discussed topics.

Plenary Sessions Summary

Where is US Health Policy Going?

The first plenary session[1] brought four bipartisan leaders of US health economics to discuss topical health policy issues facing the US and the world. The experts discussed what concerned them most with regards to current US health policy:

- Reducing the number of uninsured individuals
- Limiting the scope of coverage
- Decreasing consumer choice
- Destabilisation of the US insurance market

Social Network Interventions and Population Health

The second plenary session[2] presented the ongoing research aimed at addressing how and why social networks form, and how such networks can be manipulated in order to improve health outcomes. Professor Nicholas Christakis described how obesity is an epidemic both in terms of prevalence and it being a social contagion, spreading by up to three degrees of separation. It is now possible to simulate experimental social networks; by examining the structure of networks, it may be possible to affect health, for example, by improving how physicians share knowledge, limiting diseases spreading through a community, or launching an effective anti-smoking campaign by targeting the most influential demographic. With the rise of online social networks, there is an exciting opportunity to use digital media to influence health outcomes.

When do we Really Need Randomised Controlled Trials?

The third and final plenary[3] addressed how 'big data' is changing the strength of evidence from observational studies and how this is influencing the types of studies where randomisation is necessary. Three leading researchers presented their perspective on the usefulness of real-world evidence for healthcare decision-making and the remaining challenges.

Some approved products have been subsequently withdrawn based on post-regulatory observational data; with access to 'big data', it may now be possible to identify such concerns earlier on in the product lifecycle, or help fast-track the approval of technologies with more limited randomised evidence. Given the many choices faced when designing an observational study, and the high risk of bias in such designs, the speaker recommended that the robustness of any study using 'big data' needs to be explored by the authors, who may wish to conduct several parallel studies using different methods and 'big data' sources to determine if these provide similar answers. The 21st Century Cures Act now requires the US Food and Drug Administration (FDA) to develop a framework and guidance for evaluating real-world evidence in the context of drug regulation, so understanding the robustness of such analyses is of great importance.

Value Frameworks in the US

In recent years, several value assessment frameworks have been developed in the US as the healthcare system attempts to move towards a value-driven approach that focuses on evaluating therapeutic options based on health outcomes, value to the patient and cost-effectiveness compared with other potential treatment options. Costello Medical presented a poster at the conference,[4] which analysed the online reaction to these value frameworks, and found that the Institute for Clinical and Economic Review (ICER) had been heavily criticised, with the majority of the responses being focussed on the methodology underlying the frameworks.

The ISPOR Special Task Force has produced a scientific policy white paper to discuss the different perspectives of the widely diverse value frameworks and produce some recommendations to enable more efficient decision-making in the US healthcare sector.

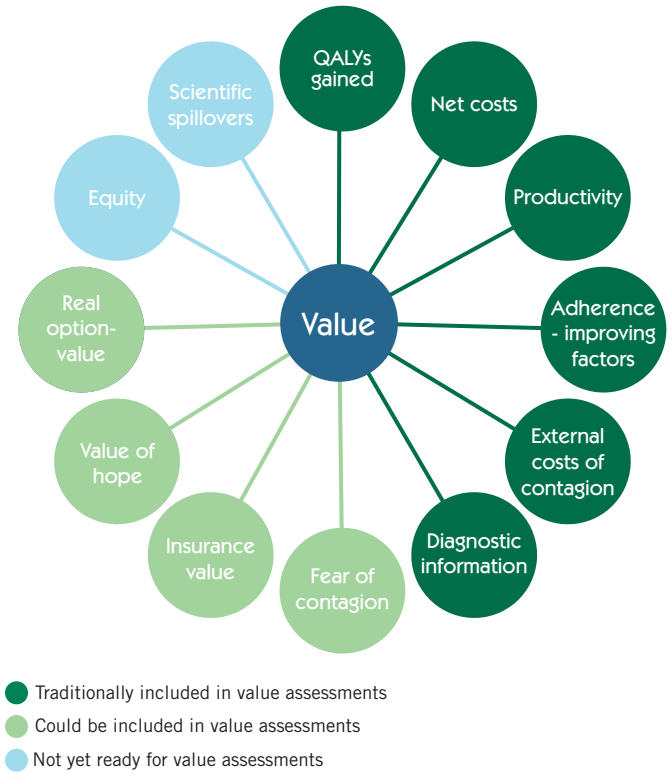
Key recommendations presented at the conference included:[5]

- Apply conventional cost-effectiveness analysis in public and private coverage and reimbursement decision-making
- Embrace potential quality-adjusted life year (QALY) refinements, including insurance value and scientific spillovers
- Adopt decision rules based on cost-per-QALY thresholds
- Improve the specificity of value assessment frameworks

It was noted that moving from listing the elements that matter, to measuring them, to weighting them, are three very different steps. Experience in other countries, such as the UK, shows that it can be done. The preferences of the public and patients need to be understood; societal weighting and a structured deliberative decision-making process are needed.[6]

There is a diverse range of elements of value, but it is a challenge to map each element into an underlying economic framework for value assessment. **Figure 1** was presented[7] to highlight how many elements of value are still not included in value frameworks, but there could be potential in the future to include these.

Figure 1: Elements of Value



QALYs: Quality-adjusted life years.

Medical Devices

Medical devices made another large appearance at ISPOR this year, with 23 issue panels, workshops, forums and podium presentations considering the field of medical devices, and 66 posters including the term 'device'. A range of sessions were delivered at ISPOR; an entire podium session was devoted to the original research for medical devices, alongside various panels discussing health technology assessment (HTA) and market access of new technologies.

Portfolio Optimisation

A workshop discussed how to use optimisation techniques alongside conventional modelling to better allocate resources when managing a medical device portfolio.[8] 'Early HTA' analyses such as these can be used to determine which technologies should be taken forward with a limited budget; multi-criteria decision analysis (MCDA) can be used to score the value of each pipeline technology to determine value for money of each stream, which should consider possible synergies between complimentary devices.

Pre-Approval Exchange

A survey of the role of the Academy of Managed Care Pharmacy (AMCP) dossier,[9] designed to aid communication between manufacturers and healthcare decision makers, was completed by payers; a majority stated that the format facilitated information exchange (92%), made it easier to understand what information is available from manufacturers (77%), and that the manufacturer response rate is 40% better when participating in the AMCP format. The panel concluded that the pre-approval exchange of information is currently happening, and that the AMCP format is facilitating such communication.

Performance Based Risk Sharing

Medical devices lacking sufficient evidence to fully support a formal HTA process may benefit from performance-based risk-sharing schemes,[10] leading to the device being reimbursed temporarily to help fund additional evidence collection. The expert panel warned that these schemes are not suitable for all cases; simple discounting is likely a better approach if the goal is to increase market share, and such risk-sharing deals are very slow and challenging to agree upon in practice.

Medical Device HTA

HTA of medical devices remains a substantial challenge.[11] The efficacy and costs of medical devices are influenced by distinct and unique properties:

- Complex classification of devices
- Reutilisation of devices and maintenance/repair
- User learning curve to maximise efficacy (which may be affected by the skill and experience of the operator, their caseload, and any training that the manufacturer may provide)
- Robustness of clinical evidence (particularly driven by the difficulty of blinding, small sample sizes and ethical concerns)
- Technological and price dynamism

Medical devices therefore cannot be modelled in the same way as drugs. The panel discussed the techniques that are available to better model the intricacies of medical devices, and concluded that government intervention is likely required to aid HTA of medical devices.

Future Directions for Cost-Effectiveness

Several presentations looked at the development of cost-effectiveness analysis, and potential future developments in this area.[12,13] The way that treatment benefits are defined, and the willingness to pay for such benefits, are two key elements that were highlighted that are likely to change, resulting in potential changes to the way cost-effectiveness analyses are conducted.

One presentation stated that productivity costs are likely to feature more prominently in cost-effectiveness analyses in the future;[12] instruments may need to be developed to prospectively capture household productivity in trials, and developing measures of productivity losses across a range of prevalent health states will be valuable. Furthermore, whilst the impact on caregiver time has traditionally been accounted for in cost-effectiveness analyses, assessing the impact on caregiver quality of life independently of the impact of a condition on patient quality of life will provide a separate challenge. Elicitation techniques will need to be refined and applied to specific disease areas. In addition, prediction models for technology diffusion will become more important, to develop more reliable estimates of value creation.

More dynamic methods for cost-effectiveness analysis were suggested for the future, with changes in prices, the evolution of clinical evidence, and the incorporation of real-world evidence all being seen as important elements of these methods. One session[13] highlighted that the issue of affordability, as well as cost-effectiveness, will need to be considered in greater detail in the future; new treatments for hepatitis C and gene therapies have brought this matter into sharp focus.

Oncology Modelling

There was a focus on tackling the challenges associated with building economic models for immuno-oncology therapies at the conference, with several sessions discussing this.[14–16]

Immuno-oncology therapies, such as nivolumab and pembrolizumab, have been shown in clinical trials to significantly improve survival and patient outcomes. However, demonstrating the full value of immuno-oncology therapies has proven to be a challenge, with conventional techniques and methods having several limitations.

One workshop at the conference[14] detailed the challenges being faced by those in the industry attempting to demonstrate the value of immuno-oncology therapies:

Immature OS	• Curve flattening for IO arm • High uncertainty in extrapolation of OS
Heterogeneity in treatment outcome	• OS outcomes differ by response status • OS and response differ by PD-L1 status • Multiple PD-L1 tests and test cut-offs
Response criteria	• RECIST 1.1 may not capture main patterns of response • RECIST-based PFS become disconnected with patient experience and OS outcome
Quality of life	• Limited QoL data for responders and long-term survivors, especially after disease progression • Increasing difference between trial-based QoL and literature
Treatment duration	• Lack of data informing treatment stopping rule
Subsequent IO treatment	• RCT might be confounded to compare IO vs. no IO • First-line IO studies often had a high level of subsequent use of IO for control arm

IO: Immuno-Oncology; OS: Overall Survival; PD-L1: Programmed Death-Ligand 1; PFS: Progression Free Survival; QoL: Quality of Life; RCT: Randomised Controlled Trial; RECIST: Response Evaluation Criteria in Solid Tumours.

Survival partition models, which are commonly used in HTA submissions, have the following underlying assumptions:

- Progression free survival accurately describes disease progression and the changes in disease progression attributable to the treatment
- Death and progression are the key drivers of changes in patient costs and utilities
- The survival curves follow the same pattern as prior to the end of trial follow-up, and can be extrapolated using a single distribution

However, these assumptions do not tend to hold for immuno-oncology therapies, particularly given the complexity of the tumour kinetics involved. Several new modelling methods were suggested in the sessions in order to account for these assumptions not holding:

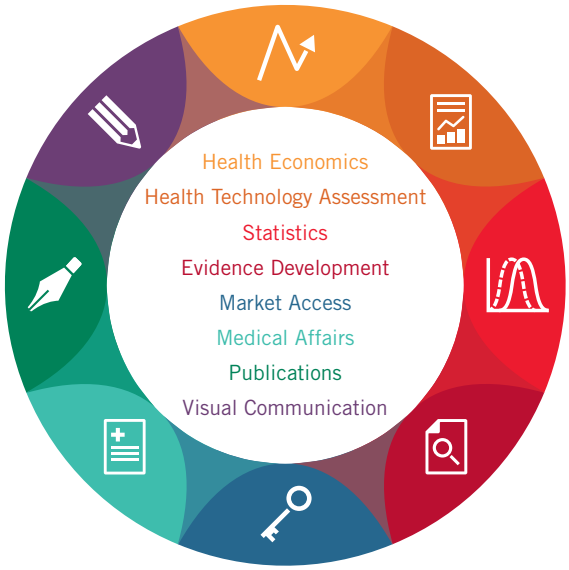
- Dynamic modelling
- Cure modelling
- Landmark analysis and response-based modelling
- Segmented parametric analysis
- Different model structures for different treatment options

Overall, a slow move away from standard partition models is being observed, in order to fully capture the benefits of immuno-oncology therapies.

Costello Medical Consulting

Costello Medical provides scientific support to the healthcare industry in the analysis, interpretation and communication of clinical and health economic data. Due to growing demand across an increasing range of service offerings and geographies, Costello Medical has grown organically since foundation in 2008 to a team of over 100 based in Cambridge, London and Singapore.

Alongside our widening technical and creative capabilities, we remain committed to our core values of high quality scientific work coupled with exceptional customer service at competitive and transparent prices. Our talented team has experience with a variety of leading pharmaceutical companies and medical device manufacturers and a track record of success in a broad range of disease areas. For more information on our services in HTA, Health Economics, Statistics, Evidence Development, Market Access, Medical Affairs, Publications or Visual Communication please visit our website at www.costellomedical.com.



Costello Medical will also be attending the ISPOR 20th Annual European Meeting 4th–8th November 2017, Glasgow, UK

Further Assistance

If you would like any further information on the themes or research presented above, please do not hesitate to contact William Marsh at: william.marsh@costellomedical.com.

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8. W15: Optimal Allocation of Resources in Managing Medical Device Portfolios, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.
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12. IP1: Future Directions For Cost-Effectiveness Analysis, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.
13. IP9: Perspectives On The Relationship Between Cost-Effectiveness And Affordability, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.
14. W3: Developing Cost-Effectiveness Models To Assess Value of Immuno-Oncology Therapies: Challenges And Approaches, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.
15. F9: Health Economic Modelling In Oncology, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.
16. IP17: Cost-Effectiveness Models For Innovative Oncology Treatments: How Different Methodological Approaches Can Be Used To Estimate The Value Of Novel Therapies, ISPOR 22nd Annual International Meeting, Boston MA, USA, 2017.