Payers and other decision makers are keen to receive proactive information about the pipeline products prior to launch to help with financial planning or to anticipate the nature of the clinical and economic assessment they may have to perform.

Stakeholder preference should be built in the value assessment as early in the process as possible and data sources should be considered because they may impact decision making.

Economic models “lack transparency, are difficult to understand and evaluate, and have too many hidden assumptions.”

RWE is far from being considered as a gold standard similar to RCT, in particular due to lack of randomization, transparency regarding data-dredging and reproducibility of results.

The transferability of data to other contexts or settings has been a major challenge for drug pricing, particularly for the estimates of prices or unit costs.

Payers and providers require more information from manufacturers of the unapproved uses of approved products.

When estimating survival beyond the period of the trial, it is essential to address the heterogeneity in patient population directly.

Cost effective analysis is rarely used for formulary decision making.

Payers decisions are highly variable and hence, an urgent need to adopt more standardized methods and practices for technology assessment.

WG engaged clients and captured attention of ISPOR alike; with ISPOR mentioning WG as top influencers and also re-tweeting our live on site updates, WG sure had a successful conference coverage...
WG differentiated in content as well as engagement. Our Expert health economist was invited to a jury panel for a critical session in Oncology and Kidney Disorders. WG posters invited fair share of attention with attendees vying for expert opinion. Also, keeping in spirit of continued learning, our health economist gained some expert skills by attending the short courses.
SNAPSHOTS OF OUR TEAM’S COVERAGE OF DAY 1

Session: Dealing with the challenges of providing information to payers prior to product launch 11:00 am - 12:00 pm
- This panel discussed the result of a survey which explored the 175 U.S. payers’ need for information about new products of drug prior to market launch and the associated challenges in communicating clinical and pharmaco-economic information to payers and other decision makers prior to the launch of a new product
- As emphasized by Jessica Daw from UPMC, “payers and other decision makers are keen to receive proactive information about the pipeline products prior to launch, in order to help with financial planning or to anticipate the nature of the clinical and economic assessment they may have to perform”
- However, manufacturers face challenges in providing this information, either because it may breach regulations concerning the communication of ‘off-label’ information, or because the information available is preliminary or otherwise subject to uncertainty

Session: Value assessment debate: pluralistic approach or does one size fit all? 11:00 am - 12:00 pm
- The panel evaluated the contrasts in payers and patients value inputs when considering value assessment. Panel members suggested that value assessment must be tailored for reasons such as different preference in various stages of breast cancer, differences in how stakeholders prioritize factors contributing to value across stakeholder groups and sub-populations, etc.
- Another panel member spoke from the payer’s perspective about the challenges of current value frameworks, such as non-actionability of every framework but some, limited drugs considerations, that frameworks are not patient centric, and lack of data to fill in the framework
- The panel members expressed varied opinions on whether stakeholder’s preference matters and suggested that the “stakeholder preference should be built in the value assessment as early in the process as possible and data sources should be considered because they may impact decision making”

Session: ISPOR’s special task force report on US value assessment frameworks: what does it say and is it helpful? 2:15 pm - 3:15 pm
Session: & ISPOR’s special task force report on US value assessment frameworks: a deeper dive into its health economic underpinnings (invited workshop) 3:45 pm - 4:45 pm
- The panel discussed the appropriate approaches and relevant perspectives to support the use of high-quality health care value framework that aims to facilitate more efficient health sector, considering that in last few years, a number of value assessment frameworks have been developed
- The ISPOR Special TaskForce (STF) has produced a scientific white paper that reviews relevant perspectives and appropriate approaches and methods to support the construction and use of high-quality health care value frameworks with the objective of enabling more efficient health sector decision making in the United States
- It is interesting to note that the panel concluded that the payer decisions are highly variable, with US payers being hesitant to consider cost-effective analysis in their decision-making process
- The panel concluded that there is a strong reason to adopt more standard method for technology assessment considering the much-diversified payer decisions

Session: Controversies in transparency and sensitivity recommendations from the second panel on cost-effectiveness in health and medicine 5:00 pm - 6:00 pm
- This panel discussed the tradeoffs of closed or open-sourced cost-effectiveness analysis models in publications, issues including the reproducibility of science, assessment of model validity and findings, application of results, and credibility
- There was a wide spread need for transparency in economic models, with some suggestions being to make models accessible via request, journal supplementary material, public repository, and model registry or database
- Models “lack transparency, are difficult to understand and evaluate, and have too many hidden assumptions.” Hlatky, 2016
Session: Is there consistency in the evidence that payers report reviewing when determining drug coverage? Should it be consistent?
3:45 pm - 4:45 pm

- This panel discusses the consistency in use of evidence of payer coverage of drugs in cases of various coverage decision and the role of real-world evidence in payer decision making.
- The challenges payers face includes three parts: evidence concerns, organizational factors and market forces.
- Moreover, for the industry the current situation does not turn out to be optimal but undergo a sub-optimal process for institutional input on evidence requirements.

Discussion of Payer Challenges in assessing RWE, including:

<table>
<thead>
<tr>
<th>Evidence Concerns</th>
<th>Organization Factors</th>
<th>Market Forces</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Timing of evidence</td>
<td>• Resource constraints</td>
<td>• Regulatory requirements &amp; timelines</td>
</tr>
<tr>
<td>• Patient population differences</td>
<td>• Education &amp; training of staff</td>
<td>• Actuarial analysis &amp; budget forecasting</td>
</tr>
<tr>
<td>• Clinical importance of outcomes measured</td>
<td>• Clinical experience &amp; background of policy makers</td>
<td>• Medical versus pharmacy benefit</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Rebate considerations</td>
</tr>
</tbody>
</table>

SNAPSHOTS OF OUR TEAM’S COVERAGE OF DAY 2

Session: Improving reproducibility and robustness of evidence from large health care databases with specific reporting guidance.
11:00 am - 12:00 pm

- This workshop discussed the efforts to enhance the reproducibility and robustness of evidence from large health care databases, in which current practice lacks the details in guideline to achieve better transparency in research.
- Marc Berger compared RWE with RCT, and commented: “RWE as lack of randomization, trust and transparency regarding data-dredging and reproducibility of results.”
- His recommendations for confirmatory studies can be concluded that “good procedural practices are equal to good study hygiene,” which suggests RWE studies should be replicable and include key stakeholders in the research process.

<table>
<thead>
<tr>
<th>Reproducibility</th>
<th>Data Source</th>
<th>Methods</th>
</tr>
</thead>
<tbody>
<tr>
<td>Direct Replication</td>
<td>Same</td>
<td>Same</td>
</tr>
<tr>
<td>Reproduction of a specific study</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Conceptual Replication</td>
<td>Different</td>
<td>Same</td>
</tr>
<tr>
<td>Reproduction of a finding for the exposure (and comparator), outcome and estimated of interest</td>
<td></td>
<td>Different</td>
</tr>
</tbody>
</table>

- Shirley Wang mentioned that a fully transparent study is not “necessarily equal to a scientifically valid study but means replication and validity assessment are possible.”
- Fully transparent study does not indicate it is scientifically valid study.
- Fully transparent study indicates that the results can be replicated, valid and assessed.
Session: Medical Device and Diagnostics Studies 11:00 am – 12:00 pm
- Surgical Site Infections can result in significant negative clinical and economic outcomes
- Using Truven claims data, some researchers identified patient characteristics that increased the risk for SCS infection and defined the infection rate for SCS implants
- The approximate 3% device-related infection rate within 12-months of Spinal cord stimulation (SCS) implant in the US was determined

Session: Health care economic information: considerations for expanding proactive communications by biopharmaceutical manufacturers to population health decision makers 3:45 pm - 4:45 pm
- This session presented an overview of the current requirements for proactive communications HCEI and its challenges and perspectives of key stakeholders on the importance of accessibility to HCEI to better care for patients and on potential solutions to facilitate better communications
- Payers and provides require more information from manufacturers of the unapproved uses of approved products. However, FDA approval remains the gold standard for stakeholders
- Overall, FDA guidance documents has been a significant step forward. Now, manufacturers have more flexibility to communicate with payers and professionals about unapproved uses of drugs
- Pre-approval exchange of information is needed for new products and new indications, such as risk-sharing with providers, care pathways, indication based pricing, etc.
- From regulatory side, the background of Pharmaceutical Information Exchange (PIE) is compared between guidance from AMCP and FDA draft guidance, which the former includes new molecules and unapproved indications with an intent to file while the latter only new molecules. From legislative side, H.R. 2006 – PIE Act of 2017 is to improve patient access to emerging medication therapies

Session: Transferability of economic evaluation studies: is there a generally accepted alternative price benchmark to the WAC price? 3:45 pm - 4:45 pm
- This workshop presented the performance of commonly used drug pricing benchmarks, and discussed the need for a standardized drug pricing to improve the transferability of data across jurisdictions
- The transferability of data to other contexts or settings has been a major challenge, particularly for the estimates of prices or unit costs. Studies estimating drug costs in US economic evaluations have traditionally used Wholesale Acquisition Cost (WAC) or some percent discount of Average Wholesale Price (AWP) to compute to a WAC equivalent
- Three alternative price benchmarks have been identified: NADAC, VAFSS, and ASP
SNAPSHOTS OF OUR TEAM’S COVERAGE OF DAY 3

Session: Cost-effectiveness models for innovative oncology treatments: how different methodological approaches can be used to estimate the value of novel therapies 8:45 am - 9:45 am

- This panel discusses the values and challenges of novel oncology therapies and the new methodological approaches used to overcome the challenges
- A mixture cure models application to melanoma is used as an example. The Kaplan-Meier curves for I-O therapies have an initial decline in survival and a flattening of curve following, which has not been detected in chemotherapy patients. Such feature implies the presence of heterogeneity in the patient population
- The idea of the mixture cure model is to use regression models to estimate probability that a patient is cured and predict survival of patients who are not cured. This modeling may increase costs and QALY's for the cured patients and reduce costs and QALY's for the no-cured, as compared to standard modeling
- When estimating survival beyond the period of the trial, it is essential to address the heterogeneity in patient population directly
- A response based model can potentially capture the strange shape of I-O survival curves and make stronger assumptions such as treatment mediated through response and no residual treatment effect

Heterogeneity of patient populations: a two state mixture model

Pros and cons of response modeling

Pros
- Short-term response data available in key trials
- Facilitates use of other data that may be more mature (early-phase studies)
Cons
- Need to be sure that...
  - Response can distinguish patient prognosis
  - Response is a good surrogate for Tx
  - Tx effect only mediated through response
  - Bias is avoided (landmark analysis)

Follow WG Team to get live feed from ISPOR 2017. Meet our experts at booth 202 to gain key insights.

For more information, email at info@wg-group.com