

defined as the health outcome achieved per dollar spent.” This infers that a lesser weighting should be given to those elements of value which are not directly related to patients receiving the technology such as wider societal benefits.

Tunis and Eddy (3) introduced the concept of clinical and health policy decisions consisting of two critical components, namely evidence and then judgment. The evidence gathering component evaluates through a technical and scientific lens the benefits, harms, and costs between treatment options, while the second component represents judgment on the value elements themselves and addresses aspects such as personal preferences. It must be recognized that, if the quality of the evidence relating to the patient is poor or uncertain, then the judgment phase addressing wider benefits will be highly problematic.

Some elements of value need to be considered carefully in the context of an equity framework. An example is the use of the value element of a productivity gain. This will only be potentially realizable to someone who is in the work force and may disadvantage others such as children and the aged. Another example of an element of value that requires careful consideration is adherence to dosage regimens. If improved compliance is achieved through a new technology then, presumably, if relevant, it is also reflected in improved health outcomes. To add further value in the judgment phase simply for improved compliance may be seen to be double counting. Furthermore, factors that may be captured by a utility measure (which may include direct and indirect benefits to patients) and therefore included in a quality of life gain and form part of the evidence base should not be then heavily weighted in the final decision making requiring judgment.

There is clearly a divergence of opinion between decision makers and sponsors of new technologies regarding whether, and to what degree, broader aspects of value are taken into account in decision making. Sponsors generally believe that decision makers are too focused on the results from clinical trials and ignore additional benefits that a new technology may provide. However, as mentioned earlier often these “values” are not discrete entities but part of a “value framework” where interactions occur between value elements and care must be taken to ensure in these circumstances that the overall assessment of value is appropriate (e.g., helping to ensure no double-counting). That a divergence of opinion occurs indicates that greater transparency and information around the decision context is needed. If there is not an explicit acknowledgement of the potential of a certain value and how that was managed as part of the decision process then there will be an assumption that any such value(s) was not considered.

What the debate highlights is the need for sponsors of new technologies to broaden their horizons regarding the nature of clinical trials and what endpoints are being measured. To undertake a trial without a quality of life measure or some other patient relevant outcome, and base it only on a surrogate outcome that may be accepted by regulatory agencies,

needs to be questioned. The methodologies used to identify and quantify the range of potential value elements needs to be advanced not only in clinical trials but in the postmarketing environment.

It must, however, be recognized that, even if all elements of value are considered and taken into account in decision making, this does not necessarily extrapolate into acceptance of “value for money.” There is a risk that, if there is recognition of a wider range of values being considered by decision makers, then there will be an expectation that higher prices will be the result. That hypothesis is yet to be tested.

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## HTA AND VALUE - AN INDUSTRY PERSPECTIVE

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The assessment of value within healthcare is undergoing a major transformation. Gone are the days when a new mechanism of action alone would be regarded as a high value innovation. Today, there is much more of an emphasis on what are the outcomes (i.e., mortality and morbidity benefits), how does this compare with the current standard of care and what is the impact to the usage of healthcare resources. This emphasis is quite understandable given the financial crisis we are experiencing with an ever growing and aging population that is placing considerable strain on the healthcare system.

There are few Eureka moments in science. Incremental innovation such as a new mechanism of action is vital for furthering the scientific understanding and fostering the development of future innovations, especially when it is linked to targeted patient populations and coupled with optimization of the disease management process. It should come as no

surprise that healthcare systems are asking “what is the value for money we are receiving?” It is very human of us to ask this simple question, “is it worth the money?” We do this every day when we purchase a consumer good and consider other expenses.

The facts are very simple: innovation comes at a cost. The attrition rate for compounds from initial exploration through to the market is more than 999 of 1,000, and these 999 abandoned compounds and their associated costs are just part of the cost of innovation (*Forbes 2011*). If innovation is not rewarded the advancements we have seen within the pharmaceutical space would not have taken place or would not have taken place so quickly. Assessors of value need to ensure that innovation is evaluated in a transparent manner that allows for constructive dialogue to take place for future advancements.

This is where health technology assessment (HTA) can play a significant role. Although the mandates of HTAs may vary from one to the other there is always one constant, HTAs are evaluating the current treatment paradigm and estimating how it will change with the introduction of new interventions. This is a fair question that the pharmaceutical industry also asks itself when it begins the development of a new treatment. Where differences in opinion can arise is the criteria used to define value. In some instances, value is assessed by HTAs with a limited scope. Aspects such as productivity, social benefits, benefits of efficient healthcare delivery, and improved patient relevant outcomes can often be overlooked by HTAs. But all of these factors in addition to the core elements of value (e.g., efficacy and safety) provide a more complete picture of what the introduction of a new pharmacological treatment represents as a healthcare solution.

Establishing the benefit of a new treatment primarily resides with the pharmaceutical industry and we can often fall into the trap that the benefit can be rather intuitive. Significant efforts have and are being made to establish this benefit with evidence. Given the constraints of the current operating model for delivering evidence (i.e., randomized controlled trials) which can limit the type of information the pharmaceutical industry can provide (i.e., efficacy vs. effectiveness), we must find more efficient ways of generating the evidence to convey the value of our treatments.

Evidence from a multitude of sources—whether it be concrete data or a formalized opinion from various stakeholders—should be given consideration when assessing value as it can project the credible promise of the treatment.

These issues lead to some recommendations by the pharmaceutical industry to ensure that HTAs can robustly assess and allow access to value in a timely manner. Such considerations include:

- **Ensuring the HTA decision-making process is transparent** – HTAs should be clear and explicit on the methodology they are using and the criteria and data they need to reach their decisions
- **Encouraging dialogue with all stakeholders throughout the process** – HTAs should be available for active engagement with various stakeholders (patients, physicians, industry) from clinical development to real world usage. The development and usage of treatments is a dynamic process but is currently subjected to a static assessment. We need to allow for continuous dialogue throughout the process of value assessment so that multiple perspectives can be shared across the life cycle of an asset
- **Reflecting all aspects of value** – Improved productivity, convenience, reduced burden of care on caregiver/family support are just some examples of value that are considered as wider elements of value that are not routinely evaluated by HTAs. There can be an insistence that hard evidence be provided to substantiate these elements but many issues can arise which prevent such data collection. Such data must be collected in an ethically correct manner and current constraints that exist within the system need to be acknowledged (e.g., convenience cannot be readily assessed in a double blind randomized controlled trial as the patient does not experience the benefit as they do not know what they are receiving).

Significant strides have been made by industry to demonstrate value and by HTAs to assess value. A cooperative and collaborative spirit has emerged between both parties to begin and continue the dialogue on assessing value in a direction where the potential ground for consensus can be reached. Given the challenges posed by demographic change and budgetary constraints, we (all healthcare stakeholders) can only succeed by working together to ensure optimization of healthcare to the wider society.

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