Viewpoint: Patient Perspectives

Introduction: Defining the Value of Medicines

By: Diana E. Pankevich, Ph.D. (Pfizer) and Carolina Huber (Precision Health Economics)

Health influences many aspects of our lives including the ability to perform daily functions and work productively. Activities that define healthy living are interconnected and the ability to maintain, or improve, one’s health can be linked to the ability to meet individual needs for function and productivity. The relationship between health and productivity suggests that health is related more broadly to the overall economy. Perhaps most importantly, we think of health in terms of its innate value to us, our family and friends, and to society as a whole. Healthcare providers are focused on medical treatment and patient satisfaction and access to medicines and care. Accurate, timely, and comprehensive information on the cost of potential healthcare choices can help ensure that spending reflects the value of medicines to maintain or improve function and productivity.

Studies have been undertaken to estimate “value” and develop formulas to determine coverage and availability of medicines based on the relative cost and outcomes of different approaches to care. Some studies have taken a narrow perspective on defining ‘outcome’ and have not included patient views, while others have taken a short-term perspective and not considered how value can change over time. For example, if we measured all healthcare investments by years of life saved, a medicine that cured blindness would be worthless. If we evaluated all vaccines by outcomes that occurred in the next three to five years, we would underestimate a significant share of the true benefits accrued over a lifetime.

In the following viewpoint, the importance of incorporating patients’ perspectives into the “value of medicine” definition is explored in more detail. In most effective markets, consumers are the ultimate arbiters of value; though the ability to measure patient preferences is in its childhood, if not infancy. The evidence that informs policymaker and physicians’ decisions is not currently defined by how a patient values different outcomes or health states, such as whether he/she values a longer life even with lower quality or a shorter life with higher quality, or even an oral over an infused product.

Estimates of the value of medicines must take a wider view than those currently used. Measuring all costs and just some benefits inevitably distorts our picture of value, as does measuring all benefits and only some costs. It is essential to use all available information about how value changes over time; how the true value of a medicine to a patient can only be decided by the patient.

The Importance of Incorporating Patients’ Perspectives When Valuing Medicines

By: Roslyn Schneider, M.D. (Pfizer) and Carolina Huber and Suepattra Mayslater (Precision Health Economics)

When it comes to determining the value, or costs and benefits, of technologies designed to alleviate the pain and suffering associated with a health condition, those seemingly best-suited to make these determinations are the affected patients. There have been notable advances to include patients’ perspectives such as the establishment of the Patient-Centered Outcomes Research Institute,1 FDA’s Patient Network,2 and NIH’s Patient-Reported Outcomes Measurement Information System.3 But, it has been primarily physicians, researchers, and policymakers, not patients, who have undertaken the process of valuing healthcare technologies, such as new medicines.4 Even when the aim is to cure, the balance between the likelihood of success and the risks, benefits, and uncertainty associated with a treatment is an important decision involving the physician, the patient, care partners, and other members of the patient’s support community.

Even if society can agree that the best way towards understanding value in healthcare is via patients’ perspectives, there are limitations in the healthcare market; there is the assumption that physicians and policymakers know more about the effectiveness of potential therapies than do many patients. Health policy experts tend to place more weight on the opinions of professionals—because professionals have training and experience to assess the validity of data demonstrating that therapies achieve the health outcomes they set out to examine.

The value placed in this professional knowledge relies on a simple model that assumes there is one best treatment (outcome) for every case of disease and there only are two health states to maneuver between: sick and well. And, while expert knowledge of diseases and therapies is not shared by everyone, this model does not recognize that reality is more complex than the model in which a doctor acts as the only agent for the patient on treatment decisions. Many health outcomes are multifaceted and patients have previous experiences with and opinions about health states, subsequently patients have considerable knowledge and experience about how they value such states. Individual patients may place a greater value than what a population-based model might show beyond the simple state of sick or well; for example, patients might focus more on: disability intervention, improved functioning, return to activities of daily living or to work, or being discharged sooner from the hospital.

There are two challenges we would like to highlight around greater involvement of patients in the valuation of new health technologies; the first is the incorporation of patient-centric outcomes in randomized controlled trials and observational studies used to evaluate technologies. The second is the inclusion and participation of patients in decision-making around approval and reimbursement.
Treatments of chronic conditions or infectious diseases that are managed, rather than cured, draw the vast majority of health care resources today. Managing disease is very different from curing disease, and may not involve the removal of all pain and suffering, but rather the alleviation or tolerability of as much and as many of the symptoms of the disease as possible to help attain a state of wellness. These symptoms are often multiple, complex, and can be contradictory in nature. For example, a patient may desire to minimize his/her pain while also remaining alert and active. Alternatively, it may be important to dampen the strength of a patient’s immune system while protecting against the risk of infection.

Understanding the views of patients and their entire support system, including healthcare providers, is particularly important for patients whose condition may require treatment with regimens that can have great impact on day-to-day life. Valuing and selecting a therapy solely based on certain attributes or clinical endpoints may mischaracterize the benefits to patients and result in suboptimal valuation of treatments. For example, patients may value longer, contiguous periods of treatment-free or pain-free intervals—even when overall survival or prognosis does not change. This may offer peace of mind and periods of stability in health that allows the patient to focus more completely on other long-term life activities or achievement of personal goals.

Thus, the evidence that informs a physician’s treatment decision should not be limited to the average effectiveness of a medicine in alleviating a range of symptoms. Instead, it should include both the value a patient places on various health states and the treatment options that most likely maximize the patient’s welfare. Essentially, this is a shared decision-making process that requires a combination of empirical evidence on therapeutic options mixed with the individual patient’s needs, goals, and preferences. As a result, welfare maximization cannot be achieved with just one source of data; it needs multiple sources.

To highlight the importance of the point above, we can consider two patients with similar prognoses but different potential treatment pathways: one patient may place higher value on a longer life even if that results in a lower health-related quality-of-life, while the other patient would instead prefer to live a shorter life of what they may consider or is measured as higher quality.

The incorporation of patient values has been especially underutilized in studies looking at neurological conditions such as Parkinson’s disease. Research has demonstrated a clear discrepancy between the disease effects weighted highest by empirical outcome measures constructed by researchers, such as indices of health-related quality of life, and the ones patients have highlighted as most important to them (e.g., side effects, family burden, or likelihood of reaching a milestone family event). This includes, but is not limited to, the impact of fluctuating response to their medications, sleep problems, and mood changes. Further, the disease symptoms that are most important to patients change with the severity of the disease and prognosis, which suggests that setting a value on a health state for all patients with Parkinson’s provides limited utility. Severity of symptoms or health states has also been challenging to measure in clinical trials and largely excludes caregiver perspectives, a group that would also benefit from an effective Parkinson’s treatment. As a result, cost-effectiveness studies of Parkinson’s treatments often yield results that suggest these therapies are less cost-effective than they would be if evaluated in a more representative population including more holistic measures of benefit in those affected by Parkinson’s.

When evaluating health states in clinical trials and cost-effectiveness studies there is a tendency to define them in static terms, such as a single score measured on one specific day of a clinical trial using an endpoint based on a fixed selection of generally relevant symptoms. In reality, the concerns of patients are often dynamic in nature, changing from day-to-day and also over time. For example, according to a recent study involving patients with advanced Parkinson’s, the most commonly-stated problem symptom was “fluctuating response to their medication”—something that doesn’t make the list of most troublesome symptoms for patients with the condition for less than six years. Conversely, the three most important symptoms for patients with Parkinson’s treated for less than six years were slowness, tremors, and stiffness, none of these was a top three symptom complaint for the group of patients who had been receiving treatment for over six years. This dichotomy of most important symptoms illustrates the dynamic nature of the health states that Parkinson’s patients experience as their disease progresses.

Vast methodological advances have been made in how to evaluate and value new medicines, but these advances largely prioritize and utilize outcomes that are empirically measurable, rather than ones that are context dependent. Understanding how patients and their support communities deliberate their choices about treatments and how they assign value to treatments has significance, not only for physician-patient engagement and achieving clinical outcomes of highest value to patients, but also for the endpoints that research sponsors choose to include in trials. Despite a clear shift towards patient centricity, there remains a shortage of literature about the ability of these outcome measures to be effectively translated into measures of true patient welfare that influence decision making. If this second stage of determining value is ignored, this wealth of non-contextualized outcome measures becomes largely worthless; patients’ perspective of his/her welfare should be part of any estimate of value.

Endnotes
2. See: www.fda.gov/forpatients/about/default.htm.

Issued by Global Policy and International Public Affairs
For more information, visit Pfizer.com/ValueOfMedicines
Please follow @Pfizer on Twitter, hashtag #ValueofMeds
October 2017