The increasing role of economic evaluation in private payer submissions: Trends and advice from the field.

Richard Lavoie is a senior economist and founding partner at Synergyx Consulting. He serves clients in the pharmaceutical and insurance industries by conducting projects in pharmacoeconomics, forecasting and market access. He also advises insurers and benefit consultants on data analytics and drug plan management optimization.

By Richard Lavoie

Canadian drug plan administrators realize that the rising number of specialty drugs requires a consequent increase in their ability to manage them. Over the past fifteen years, provincial drug programs have centralized their review process through CADTH (and INESSS) and have successfully leveraged their collective purchasing power. Cost-effectiveness considerations have a role to play in listing decisions, for establishing reimbursement criteria and for negotiating confidential pricing agreements (PLAs) with manufacturers. Pharmacoeconomics (PE) is now an important and well-established notion in drug reviews.

While a bit late to the party, private insurers recognize that plan sponsors expect them to take a more active role in managing the drug plan and preserving its sustainability. In this competitive environment, insurers are following the government's lead and using PE in their drug review processes. Manufacturers have an opportunity to improve the private payer's perception of the value of their products and facilitate reviews by paying attention to the quality and relevance of the economic evidence they present.

From my perspective as an economist involved in reviewing submissions for insurers and preparing PE analyses and BIAs for manufacturers, this article discusses some of the issues common to private payer economic evaluations and provides advice on how to design and submit PE in the private payer market.

The type of economic evaluation.

A common misconception in private submissions is to assume that the PE analyses you present to public health technology assessment (HTA) organizations are well-suited for private payers. While this may be correct in certain cases, the needs of organizations like CADTH and INESSS are not necessarily the same as those of the private sector.

An example is cost-utility analysis (CUA). In their guidelines, CADTH states that a CUA is the preferred type of evaluation. As a result, manufacturers end up submitting a whole lot of CUAs even in situations where this approach might be of questionable value to an insurer.

For example, when a drug is a subsequent entrant in a class of products, its outcomes may not be materially different from existing drugs, either due to having a similar mechanism of action or because no evidence exists to the contrary. In these cases, a simple cost-minimization analysis is often a more appropriate framework for private plans than a complex CUA built on many assumptions and carrying significantly more uncertainty. (This is the pragmatic approach advocated by INESSS in their own guidelines.) In other words, thesimplest framework that provides the assessment is preferable. This is particularly true for private payers who generally have less resources to dedicate to drug reviews.

Model complexity and quality.

Private payer analysts can become frustrated with complex cost-effectiveness analyses submitted by manufacturers that are often riddled with quality issues and biases. Models are typically designed at the global (international) level to cater to the needs of multiple countries. Too often, the result is large, bloated, academically-oriented, overly-complicated models. They sometimes evade or omit evidence that may be somewhat less favorable, beat around the bush or use circumvoluted methods. It is not rare to have in excess of 50 Excel sheets. Long running times are also a problem. Is it normal that a CUA takes literally hours to run? A sceptic might think it is more likely a symptom of poor design or attempt at obfuscation.

Au contraire, a complex framework is not beneficial to a manufacturer's dossier. By making it challenging for private payers to assess models, the outcome is more likely to be a lower overall confidence in their validity (and an irritated analyst as a bonus).

PE analysis perspective.

Whereas provincial drug programs will seek to evaluate the impact of a new drug on healthcare system costs, private payers do not pay for publicly-funded healthcare and will be less concerned with these considerations. A private payer provides benefits on behalf of plan sponsors, i.e. employers, unions and trade associations. These stakeholders are more concerned with keeping productive employees at work, that is, productivity, disability and absenteeism.

Ideally, the reference case in PE analyses for private payers should discard publicly-funded costs (hospital costs, doctor visits, lab tests and medical procedures, chemotherapy administered in public facilities) and incorporate productivity considerations. A societal perspective can be presented as ancillary information. Productivity estimates should be transparent and incorporate Canadian labour market characteristics like employment rate, average wage and worked hours.

Relevance of disease progression and clinical practice.

Valid economic analysis should be based on a realistic representation of disease progression. Whenever possible, validate the disease progression model against external sources like the literature or clinical expert opinion. The same applies to selecting the appropriate set of comparator treatments – they should be relevant to Canadian clinical practice. While most studies abide by these principles, a certain proportion still present unrealistic disease progression/survival prospects or use comparators that are not used or infrequently used in Canadian practice.

Time horizon.

A regular bone of contention in cost-effectiveness analyses is the length of the time horizon. It should be long enough to realistically capture potential differences in costs and outcomes between interventions. However, this does not necessarily mean that long or lifetime horizons should be the norm. Balance is required; long-term extrapolations based on clinical trials with short durations are uncertain. For example, if both study arms have converged after 10 years, or if nearly all patients have died, extending the time horizon beyond that timeframe has limited impact on results and merely increases uncertainty.

Recognize too that private payers primarily cover a working age population and their dependents. The time horizon should adequately represent plan member demographics. Most plan members are under 65 years of age and retiree benefits typically constitute less than 10% of the book of business. For diseases affecting older populations, long horizons might go far beyond the age range considered relevant by private payers. This issue is common with age-related chronic disease, where incidence tends to rise with age, and cancer patients in advanced/metastatic stages. This does not mean that the time horizon should stop at age 65 indiscriminately – it still needs to reasonably reflect long term outcomes. But exert caution when projecting beyond that age as the analysis becomes less relevant to the context of a private payer.

Utility estimates.

Utility estimates tend to be under the spotlight in CUAs; they can materially impact results but they are based on a certain degree of subjectivity. Manufacturers, and sometimes even HTA organizations, tend to take a little too much liberty with these concepts or disregard important theoretical considerations.

In economics, a rational agent seeking to maximize its welfare will prefer a certain health outcome over others based on the perceived welfare payoff among the different choices. In CUA, the payoff is defined as marginal utility. This notion has important implications for PE evaluations. First, utility values should normally reflect society's preferences for certain outcomes. Of course, asking every member of society how they value each health state is not practical. A good proxy for society is to use a representative sample of the general public.

In CUAs submitted by manufacturers, however, utility estimates are often based on other approaches. It is common to administer quality of life assessment questionnaires to patients enrolled in clinical trials, and subsequently convert data into utility estimates. Other studies rely on clinical expert opinion. While clinicians are certainly helpful for validating treatment patterns, using them to validate notions of welfare economics is a stretch to say the least.

High utilities can overestimate the incremental QALYs provided by a drug that extends survival versus existing treatments; low utilities would result in the opposite effect. In other words, inconsistent utility estimates lead to inconsistency across analyses. I have seen a situation where a health state is assigned a utility value around 0.80 in one study, and a value closer to 0.65 in another study submitted by a different manufacturer. Inconsistent methods introduce uncertainty in the mind of the reviewer. The recommended approach is to consistently favour a utility value based on a representative sample of the general public with proven methods (e.g. standard gamble, time tradeoff, visual analog scale).

Negative utility.

In some studies, certain health states are considered "worse than death" and assigned negative utility values. This is an eloquent illustration of taking liberty with the concept of utility. Marginal utility theory would normally assume that an agent is rational and seeks to maximize their welfare (utility). When considering the choice between a state "worse than death" and death itself, the rational agent would opt for death (zero utility) because it provides greater total utility than staying alive. It would be irrational to opt to decrease one's utility, and assuming that agents are irrational pretty much defeats the purpose of conducting these analyses for decision-making.

As well, that a patient considers that a certain health state is worse than death does not mean that society at large shares the same judgment. The standards of a society get reflected in its laws and regulations, which evolve over time alongside mindsets. For instance, in countries where medical assistance in dying is illegal, one can only conclude that society considers that "nothing is worse than death" and that, consequently, utility should be bounded to zero.

In countries like Canada, recent legislative changes suggest that societal norms have evolved and now consider death as a valid choice to avoid unnecessary suffering. Under the assumption of rationality, death (zero marginal utility) would be the only choice once a patient's state becomes worse than death. This also effectively bounds marginal utility to zero given that states "worse than death" are implicitly eliminated from the set of possibilities.

The elusive WTP threshold.

A sizeable portion of drug R&D has been redirected toward very rare/orphan diseases and oncology. New drug discoveries are intended to treat very small cohorts of patients, going from a few thousand to less than a hundred Canadians in some cases. The corresponding price tag is inversely proportional and may in some cases reach stratospheric proportions.

Applying a traditional willingness-to-pay (WTP) threshold of $50,000 per QALY eliminates the possibility of ever reimbursing most of these treatments. Specialty drugs may call for higher WTP thresholds, not with the intent of justifying scandalously high price points, but simply to balance the need of patients affected by severe disease and the legitimate demand of plan members for proper drug plan economic governance.

There is no right WTP threshold per se, though it could be justified to consider, for example, that products treating diseases affecting less than 100,000 Canadians (0.3% of the population) could justify a higher WTP threshold, as would diseases affecting less than 1,000 Canadian (0.003% of the population). This approach has its limits and is eminently subjective – and debating over numerical thresholds is not the objective of this article.

These rare diseases pose unique challenges for private drug plans. Many would question whether private plans are even the right vehicle for protecting society against financial hardship when rare diseases are involved. Private drug plans are intended to offer employee drug benefits to the greater number and funding these extremely expensive therapies may go beyond this purpose. Although pooling is an option to spread the burden, it is not a sustainable solution given that expensive therapies are a phenomenon in constant expansion, and as they are generally chronic rather than episodic, costs are bound to increase exponentially. The issue will simply shift to managing pooling premium inflation.

Until different solutions for expensive therapies are available, private insurers and manufacturers have little choice but to develop their own analytical and PLA negotiation capabilities.

Projecting incidence/prevalence for BIAs.

Private payers regularly receive budget impact analyses (BIAs) where disease incidence/prevalence reflects the entire Canadian population, without regard to the population demographics covered by private payers. This can result in epidemiological projections that are quite erroneous. For example, a rare genetic disease affecting primarily children may have higher prevalence under private drug plans versus the general population. Conversely, a cancer occurring mostly in patients aged 65 years and over is not likely to affect many private drug plan beneficiaries. Manufacturers should adjust their projections to the demography of private payers.

Conclusion.

Gone are the days where the economic studies submitted to private payers can be a straight copy-paste of CADTH/INESSS submissions. As insurers get more sophisticated with drug formulary management, pharmaceutical manufacturers have everything to gain from taking their specific context into consideration. In my experience, adapting components to make them relevant to the insurer's plan sponsors does not require drastic changes or enormous resources, but it pays off in improving a payer's perception of the validity and relevance of the submitted evidence and can only improve your reimbursement prospects.