Valuing Trial Designs from a Pharmaceutical Perspective using Value Based Pricing (VBP)

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Method Applied: Systemic Lupus (SLE)
We developed a simple CE model for SLE in which costs and QALYs were estimated analytically conditional on average lifetime disease activity, average lifetime organ damage and mortality.

Bayesian Clinical Trial Simulation (BCTS) was developed to sample disease activity and organ damage outcomes for individuals recruited into a RCT. The longest and largest trial was simulated a subset of the data were analysed for the shorter and smaller trials to reduce variation in the outcomes between trial designs. This was repeated 10,000 times for each trial design. We updated the CE model with trial data using the Brennan & Karroubi Bayesian Approximation method 3.

Expected total profit forecast for a given sampled dataset, is dependent on
1. the expected profit per patient per year given trial evidence \( n(\theta, X_{\text{new}}) \)
2. the annual incidence of patients needing treatment \( k \)
3. the current time horizon of the treatment, \( h \)
4. the market share of the new treatment, \( s \)

\[ PF_{X_{\text{new}}} = \pi(\theta | X_{\text{new}}) \times khs \]

Expected profit per patient depends on the Value Based Price, \( P^*(\theta | X_{\text{new}}) \), duration of treatment, \( t \), and production costs associated with manufacture, marketing and selling the new treatment per patient per year, \( c \).

We assume the pharmaceutical company has a minimum price at which they would submit for reimbursement approval, \( P_{\text{min}} \).

Hence, profit per patient is
\[
\pi(\theta | X_{\text{new}}) = \begin{cases} 
0 & \text{if } P^* < P_{\text{min}} \\
(P^* - P_{\text{min}})^+ & \text{if } P^* > P_{\text{min}} 
\end{cases}
\]

The value-based price is which gives an ICER at exactly the reimbursement authority's willingness to pay \( \lambda \). That is, \( P^* \) is:

\[ P^* = \frac{\lambda \times (Q_1(\theta_1) - Q_2(\theta_2) + C_1(\theta) - C_2(\theta))}{t} \]

where \( Q_1, Q_2, C_1, C_2 \) are the Quality Adjusted Life Years for treatments 1 and 2 and \( C_1, C_2 \) are healthcare cost consequences (excluding price of the new drug), and \( t \) is the duration of treatment with the new drug.

Conclusions
We have illustrated how ENBS can be adapted to value clinical trials in the pharmaceutical industry using expected VBP to integrate price uncertainty into the decision criteria.

Case study analyses indicated larger sample sizes are more efficient than longer trials in SLE. This simple example took 5 days for 10,000 sets of trial results. Analyses can be more time-consuming to run for complex models.

References