

# Grand challenges in pharmacoeconomics and health outcomes

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Pharmacoeconomics and health outcomes research are playing an increasingly important role in informing clinical development and market access decisions of new innovative medicines. Both disciplines are dealing with the evaluation of the costs and outcomes of healthcare interventions and can be considered as two branches of the same “value for money” tree.

Pharmacoeconomics is the part of health economics that focuses on the economic evaluation of pharmaceuticals. Health outcomes research, and patient-reported outcomes (PRO) in particular, aim at understanding patient value in terms of impact of disease and its treatment on physical functioning and psychosocial wellbeing, known also as “health-related quality of life” (HRQL). PRO’s are usually measured by self-reported questionnaires, thereby reflecting the patient’s own viewpoint on the value of a new medicinal product. In many clinical development studies, HRQL is nowadays routinely measured to help establish the product’s value for purposes of pricing and reimbursement.

Despite this growing interest, both disciplines face numerous challenges going forward. The key question remains to what extent pharmacoeconomic evaluations and outcomes research are delivering better value-based decisions.

Today, economic evaluation of new medicines is mandatory in many countries, so the question is no longer whether or not pharmacoeconomics is here to stay. The train has left the station. But challenges remain, mainly related to methodological issues.

In contrast, I believe the jury is still out on HRQL and PRO research. A recent survey suggests that although clinicians recognize the importance of PRO's, limited experience and information is a barrier to the use of quality of life assessment in their own clinical practice. Interestingly, a large majority acknowledged that they would use more expensive medicines if these could improve HRQL ( [Bossola et al., 2010](#) ).

## **Shared Challenges and Concerns**

As is the case for pharmacoeconomics, there is a huge need for training and education on what HRQL is all about, how to measure it, and – above all – how to analyze and interpret results. The biggest challenge is with the interpretation and effective communication of the clinical meaningfulness of HRQL data. Indeed, the question that many clinicians and payers are asking about PRO's is “ What do these numbers and scores really mean?”

Another major challenging area of interest for both pharmacoeconomics and outcomes research is the role of Quality Adjusted Life Years (QALYs) in economic evaluation. The QALY concept used in cost-utility analyses is increasingly subject of methodological debate. The main concern is that different methods for valuing health state preferences may yield different results ( [Drummond et al., 2009](#) ).

A related issue is the respective role of preference-based utilities versus other PRO's to facilitate market access. The use of cost-utility analysis is the preferred form of economic evaluation in several countries to inform market access decisions. Other countries are in favor of disease-specific outcomes.

The challenge is to convince the decision makers that there is more to HRQL

than health utilities alone. Therefore, HRQL PRO's should be given a more prominent role in support of value-based pricing and reimbursement.

The predominant role of generic measures to estimate utilities is also reason for concern. Advances in methods for mapping disease-specific quality of life measures onto generic preference-based measures open new prospects for better capturing clinically meaningful changes in response to treatment.

## **Pharmacoeconomic Challenges**

This is a challenging time for the pharmaceutical industry, with blockbuster drugs going off-patent, generics competition and a pressing need for innovative medicines, associated with rising drug development costs. The role for pharmacoeconomics could not be more timely.

Early cost-effectiveness evaluations are likely to become a key component in defining no go areas for clinical development. Likewise, pharmacoeconomic evaluations will be able to assist in the development of performance based pricing and reimbursement agreements. Personalized drug treatments are also a fast growing area of interest. Genetic tests that allow to predict responders to treatment offer substantial opportunities for efficient use of expensive new therapies.

A major methodological challenge is the limited generalizability of the results of randomized controlled trials (RCT). On the one hand, the RCT is the gold standard design for establishing safety and efficacy, with the highest degree of internal validity. However, findings from RCT's may have poor external validity for the wider patient population in daily clinical practice. Typical

extrapolation issues of RCT designs include inadequate sample size, restrictive patient selection (patient characteristics, co-morbidities, disease severity), inappropriate comparator, short time horizon, protocol-driven resource use, artificially enhanced compliance, and inappropriate consequence measures ( [Simoens, 2009](#) ).

Healthcare resource utilization data can be collected alongside RCT's ( [Drummond and Davies, 1991](#) ), but this is not always feasible. Economic evaluation models can be used when the relevant clinical trials are not possible for ethical or logistical reasons. Modeling allows data from different available sources to be combined ( [Nuijten, 1998](#) ). Further improvements in evaluating health- and economic outcomes in daily practice are expected from pragmatic trials with a minimum of exclusion criteria, purpose build databases, registries and qualitative research. There will always be a trade-off between the evidence obtained in controlled conditions and data coming from other sources of information, but scientific and clinical judgment on the merits of all the available evidence should greatly assist the quality of decisionmaking ( [Rawlins, 2008](#) ).

## **Patient-Reported Outcomes Research Challenges**

Some PRO's, such as pain and other symptoms severity, are used routinely as primary endpoints in clinical trials to demonstrate the patient benefit of new medicines, and are well accepted. However, HRQL PRO's are still perceived by some as a subjective measure of secondary importance, despite an increasing body of evidence of a significant growth in PRO research and applications. Further acceptance of PRO's is substantiated by

the final FDA PRO guidance and the European Medicines Agency reflection paper on the regulatory guidance for the use of HRQL measures in the evaluation of medicinal products ( [European Medicines Agency/Committee for Medicinal Products for Human Use/Efficacy Working Party, 2006](#) ; [Food and Drug Administration/Center for Drug Evaluation and Research, 2009](#) ).

Another challenge concerns the use of quality of life assessments during the whole lifecycle of a new treatment, and not only during pre-registration clinical development studies. As a unique measure of the patient perspective, PRO assessment may provide a useful tool for informing daily medical practice.

A third major challenge is associated with the difficulty in understanding and communicating the clinical meaningfulness of HRQL data. Cumulative distribution curves rather than minimal important difference criteria have recently been recommended by the FDA to demonstrate effect of treatment on PRO endpoints ( [Patrick et al., 2007](#) ). Increased cumulative distribution curve reporting will improve the interpretation of PRO data as they show the full pattern of response over time and therefore enable the entire distribution of responses to be compared between treatment groups ( [Dubois et al., 2010](#) ).

The above challenges are exemplary but by no means limitative. There are many more issues of interest to be considered. For example, what is the experience with newer psychometric methods, such as item response theory and computerized adaptive testing? Do they enable HRQL assessment in

daily practice? What is the incremental value of probabilistic versus deterministic sensitivity analyses alone? Does it lead to different decisions?

Sharing these current and emerging issues and opportunities will be a key success factor for developing high quality solutions for the grand challenges ahead. To that end, *Frontiers in Pharmacoeconomics and Health Outcomes* welcomes a broad range of contributions that may help the field going forward.

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