Cost-effectiveness of precision oncologic decision: challenges in methodology

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Introduction

The detection and understanding of tumor molecular profiles provided a basis for the introduction of new, targeted cancer treatment strategies. An essential part of these strategies is to perform the appropriate molecular diagnostics, and based on these, individualized therapies can be identified (precision oncology). In Hungary, more and more targeted therapies have become available in the last few years, and, in addition to monoclonic and companion diagnostics, molecular diagnostics that examine large gene panels and form the basis of treatment recommendations have also become available. With these changes, it’s become more challenging for the financier to make the right decisions. The aim of our study was to suggest a value assessment framework for evaluation in precision oncology in Hungary, which can help the financier in the economic evaluation of the transformation of oncology care and its cost-effective operation.

Methods

Precision oncology is a complex process. It covers the full patient pathway, which includes identifying the appropriate patients, performing molecular diagnostics and, based on the appropriate professional decision, choosing the appropriate targeted therapy. Reviewing the last 5 years of pharmacoeconomic publications, the experience is that complex decision supporting models for the whole of precision oncology aren’t available, instead, each step of the process is evaluated independently using traditional economic modelling methodologies in the case of drugs; QALY and UV-based Markov models, in the case of diagnostics: decision tree models with different outcomes. However, these solutions only provide reliable decision support for the financier for the evaluation of population-level care, the individual level can only be evaluated with some (ROI) RR measured costs and, therefore, the financial cost-effectiveness can be determined at every decision point by the appropriate selection of the cost and effectiveness pairs. Based on this, the financier can evaluate and make more cost-effective each individual point of care and the whole precision oncology care as well (Figure 1).

Figure 1. Handling uncertainty of therapeutically effects in precision oncology

The accuracy of the model is constantly increasing with the progression of time, because the uncertainty of included parameters is constantly decreasing (e.g. sample size increases, technology advances, more professional experience, etc.), and the functionality of the model can be increased as well, and more financing and professional decisions can be supported. The interim aim is the development of a return on investment (ROI) type model, in which many factors that influence the return on investor investment are also taken into account, and which results in multi-perspective cost-consequence lists. From these lists, composite outcome indicators can be derived using proper weights, which will help assess and improve cost-effectiveness of individual decision points, based on the principles of multi-criteria decision making (Figure 2).

The realization of the above requires professional supervision on a national level, and also the coordination of professional and finance protocols. Since currently the modelling of the patient pathway is complicated by uncertainties and by leaving out several influencing factors, the implementation of precision oncology care necessitates the development of disease specific rules of accessibility and performance evaluation, and adequate financial risk sharing agreements, with which the financing risk arising from the uncertainty of the system can be reduced. The ultimate aim is a cost-effective precision oncology care, based on evidence-based financing. Individual observations should be continuously gathered in disease-specific registers and reimbursements should be determined with the use of these data. With this complex system, the aim of precision oncology can be achieved: the right patient gets the right treatment at the right time. Furthermore, the financier can maintain a cost-effective health care and the pharma companies can get a feedback about real world efficacy of treatments and the treatment unmet needs (Figure 3).

Figure 2. Workflow of the methodological framework processes

The appropriate methodological framework for Real World Data processing can support to resolve the uncertainty of decision-making at an unknown or incomplete observed medical situation, which is one of the most important challenges in the expansion of precision oncology and innovative therapies. The evolution of health-economical models use individual patient path simulation based on Real World evidences greatly contributes for the success: for the drug is available for the right patient at the best time. Furthermore, a complex ROI modelling combined different resource uses and outcomes can support the implementation of a multiple-criteria decision making system in health-care development with the potential early access of some cost-effective targeted therapies in oncology. However, this type of assessment needs well-structured, individual level level clinical data and cost data, but the derivates reports used for the evaluation of any conditional reimbursement or risk-sharing contracts in a country can help in the economical maintenance of the required disease-specific register.

Figure 3. Usefulness of Real World Data in evidence-based financing

Conclusions

We suggest that the whole precision patient pathway can be modelled with an uncertainty-based, dynamic, stochastic model, which includes all possible pathways and decision-making factors. For this, stratification of patients and interim-, replacement-, and process indicators are necessary for costs and efficiency. With these indicators, the effects of individual oncology providers/influencing factors on final efficiency can be measured. Then, cost-effectiveness can be determined at every decision point by the appropriate selection of the cost and effectiveness pairs. Based on this, the financier can evaluate and make more cost-effective each individual point of care and the whole precision oncology care as well (Figure 1).

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