



Contents lists available at ScienceDirect

Health Policy and Technology

journal homepage: www.elsevier.com/locate/hlpt

Editorial

How can we make better decisions about the development and implementation of health technologies?



How can users and payers decide whether or not to use or reimburse the many health technologies that reach the market? This is a very difficult question to answer so let us start with the easy part. When it comes to health technologies like drugs and surgical procedures aimed at preventing or treating diseases, one should examine the evidence regarding their effectiveness and safety. This issue of *Health Policy and Technology* contains an article by Darba et al. that describes what they found in Spanish reports of the efficacy and safety of treatments for rare lysosomal storage diseases (LSDs) like mucopolysaccharidosis (MPS), Gaucher disease, Fabry disease and Pompe disease. [1] Based on their findings, it seems that the treatments for LSDs work to differing degrees in different subgroups and that the evidence of their effectiveness varies substantially. The authors note that new methods to assess effectiveness are needed since the current methods will not be very feasible in cases where just one subgroup of patients with a rare disease represents the entire target population. So while it may be easy to say that we need evidence of effectiveness when making decisions about whether or not to use a health technology, it may be difficult to generate that evidence.

Another important but controversial factor in reimbursement decision-making is the cost-effectiveness of the health technology, which involves comparing the costs and effects of two different ways to achieve the same goal (like treating a patient). An example of a cost-effectiveness study is the one by Nemeth et al., who compared cariprazine with a second-generation antipsychotic (risperidone) to treat certain types of patients with schizophrenia (i.e., ones with negative symptoms). [2] They found that cariprazine improved health (1.45 vs. 1.40 quality-adjusted or “healthy” life-years (QALYs)) but also increased costs (3,340 vs. 1,896 euros) per patient over a two-year time horizon. The incremental cost-effectiveness ratio (ICER), calculated by dividing the cost increase by the health gain, was 28,897 euros per QALY gained (i.e., 1,444 euros / 0.05 QALYs). The authors conclude that cariprazine is cost-effective since it is lower than the current Hungarian threshold for cost per QALY gain of 34,764 euros, which is based on three times the country’s GDP.

In fact, we can assess the cost-effectiveness of any kind of “health technology” and not just a drug. We can examine the cost-effectiveness of a disease screening programme, a medical test or even a smartphone app for use by the public to encourage a healthier lifestyle. Anything is fair game. Hinde et al. examined the cost-effectiveness of a prognostic test (EndoPredict) for UK breast cancer patients with an initial intermediate prognosis (or “risk score”) based on standard risk tools. [3] This kind of test could help to decide which of these women would benefit from chemotherapy

and which of them would not. The authors found that EndoPredict had an ICER of £26,836/QALY when compared with not using EndoPredict, which meant that EndoPredict was cost-effective based on the lower bound of the NICE threshold of £20,000/QALY but not cost-effective at the upper bound of £30,000/QALY. The authors therefore concluded that EndoPredict falls “within the grey area created by NICE’s soft threshold where consideration of broader factors including decision uncertainty is vital.” Moreover, the limited population size and follow-up of the clinical trial used in their analyses only increased the uncertainty about recommending EndoPredict for use in clinical practice. In this situation, policymakers therefore have to consider more than just cost-effectiveness when deciding whether or not to recommend the use of EndoPredict. The conclusions would have been clear-cut with a very low or very high ICER: if the ICER had been very low, the authors would have said that it was worth implementing, while if it had been very high, they would have said that it was not worth implementing.

In reality, there is no one-to-one relationship between the estimated cost-effectiveness of a health technology (i.e., the ICER) and whether or not the technology will be widely implemented. Even solid evidence of good effectiveness is no guarantee of wide implementation. Other factors play a role when making these decisions. But what other factors are there? Mentzakis et al. performed a so-called multicriteria decision analysis (MCDA) using a discrete choice experiment to determine how Spanish policymakers use different characteristics about a health technology when making decisions about its overall value. [4] They found that the policymakers considered not only cost-effectiveness, but also disease severity, the size of individual benefits, and the number of beneficiaries when making a decision about the value of a health technology. As we should have expected, different factors play a role in decision-making.

There is a critical difference between decisions based on different factors and *good decisions* based on different factors. Therefore, if we really want to ensure that good policy decisions are made, we need consensus about which information is needed to make good decisions. And if transparency is truly valued, we should also make this information widely accessible. However, these two steps (of deciding which information is needed and telling that to others) are insufficient since we need to explain how that information actually gets used in decision-making. This transformation from information into decisions requires some kind of value system, which may be based on philosophy, religion, politics, or very likely a mix of the above. One way to understand that value system is to apply the method used by Mentzakis et al., where the factors associated with a series of decisions are identified and measured. [4] And

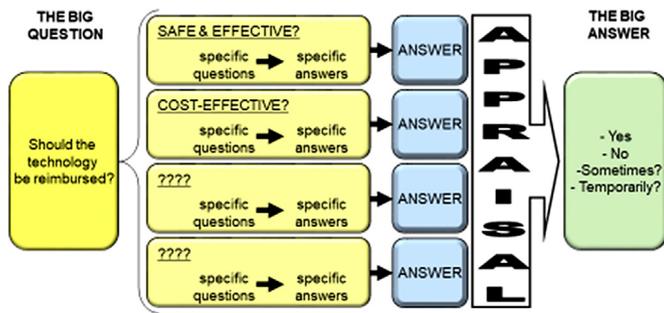


Figure 1. A conceptual framework regarding the reimbursement decisions regarding a health technology.

let us not forget the critical distinction between what is found in a study (or guidelines) and what is actually done in practice. For example, Kelley et al. examined a series of reports in which MCDA was presumed to be used, but found that while the effectiveness of a health technology was almost always considered, factors like quality of the evidence of effectiveness, ethics and cost-effectiveness were not. [5]

Figure 1 provides a generic overview of how we make any kind of decision. For example, when faced with the “big” question, in this case the question of whether or not to reimburse a health technology, we can collect information (or evidence) about different factors. For example, we might collect information about the safety and effectiveness of the health technology, and perhaps also its cost-effectiveness; see above for examples of studies that provide this kind of information. However, we may also believe that information on other factors is needed. For example, as mentioned above, Mentzakis et al. included other information such as disease severity and the number of beneficiaries in their study. [4] If we want to be systematic, we can look at specific subcriteria, or formulate and answer specific subquestions in each category. For example, how good is the evidence of effectiveness or how long was the follow-up in the pivotal clinical trials? Once we have this information, we can answer questions about each factor (e.g., is it very cost-effective?). Once that is done, we then need to step back and examine the overall findings. Based on these findings, should the health technology be reimbursed? Various answers are possible, including yes, no, sometimes (with certain patients), and maybe (e.g., if the manufacturer agrees to reduce the price). The final “appraisal” phase remains a very non-transparent, subjective phase that needs to be addressed head on if we truly want to make better decisions for the good of society. Ultimately, the arguments brought to the table in this phase should actually be examined more systematically to determine which of them should be based on information and which must be based on value systems.

Are the ideas expressed above related to how we should make decisions about whether or not to use or reimburse digital health technologies (DHTs)? Can the various types of DHTs used to prevent and treat disease be assessed in the same way as the health technologies (like drugs and medical tests) described above? The answer is yes, more or less. A recent NICE publication provides some guidance about the required evidence of effectiveness of a DHT, and it also states that the required evidence should be based on the potential risks to users. [6] For example, DHTs in ‘evidence tier 3b’, which focus on treatment (e.g., apps to support treatment decisions by clinicians) or active monitoring (e.g., remote monitoring of heart failure patients) should have more evidence of effectiveness than DHTs in ‘evidence tier 1’, which are DHTs with no direct health benefits by the user (e.g., ward management system). Interestingly, these evidence standards also recommend other evidence besides effectiveness, including proof of credibility with UK health and social care professionals, acceptability with users, and equalities considerations.

In a brief article in *The Lancet* describing the NICE guidance about DHTs and evidence of effectiveness, Greaves et al. state that if money is to be spent on DHTs instead of on other interventions like drugs or surgical procedures, “they should be held to the same level of scrutiny”. [7] I thoroughly agree with this statement. However, the actual approach to assessing DHTs must be different since, as Greaves et al. admit, DHTs “tend to iterate, update, and improve, rather than provide a stable common intervention”. It will be interesting to see how much these standards will affect the studies that are performed in the coming period.

I think we would all agree that decisions about the use, reimbursement and implementation of health technologies, including DHTs, should be based on good evidence. Therefore, we need to agree on what kind of evidence we want to see. The NICE DHT evidence framework provides some very constructive ideas about how to proceed and I look forward to reading about its use in actual studies. In fact, we at *Health Policy and Technology* would welcome any articles that describe these studies.

One last thought in closing. If we’re not very consistent about how we assess health technologies, how can we possibly expect industry to develop technologies that are worth using? Or phrased in a more positive way, I believe that once we become more consistent (and communicative) about how we decide which health technologies are worth using, industry will make better decisions about which health technologies to develop, how to develop them, and how to demonstrate their value. Ultimately, better decisions about the implementation of today’s health technologies should lead to better decisions about the development of tomorrow’s technologies.

Funding

None.

Competing interests

None declared.

Ethical approval

Not required.

W. Ken Redekop

Editor-in-Chief, *Health Policy and Technology*
Erasmus School of Health Policy and Management, Erasmus
University Rotterdam, The Netherlands

References

- [1] Darba J, Ascanio M. Enzymatic replacement therapy for lysosomal storage disorders: drug evaluations review in Spain. *Health Policy Techn* 2019;8(1):14–23.
- [2] Nemeth B, Bendes R, Nagy B, et al. Cost-utility analysis of cariprazine compared to risperidone among patients with negative symptoms of schizophrenia. *Health Policy Techn* 2019;8(1):84–91.
- [3] Hinde S, Theriou C, May S, et al. The Cost-Effectiveness of EndoPredict to Inform Adjuvant Chemotherapy Decisions in Early Breast Cancer. *Health Policy Techn* 2019;8(1):75–83.
- [4] Mentzakis E, García-Goñi M, Paolucci F, Sequeira AR. Equity and efficiency priorities within the Spanish health system: A discrete choice experiment eliciting stakeholders preferences. *Health Policy Techn* 2019;8(1):30–41.
- [5] Kelley LT, Egan R, Stockley D, Johnson AP. Evaluating multi-criteria decision-making in health technology assessment. *Health Policy Techn* 2018;7(3):310–17.
- [6] National Institute for Health and Care Excellence (UK). Evidence Standards Framework for Digital Health Technologies: National Institute for Health and Care Excellence (UK); 2018 [Available from: <https://www.nice.org.uk/Media/Default/About/what-we-do/our-programmes/evidence-standards-framework/digital-evidence-standards-framework.pdf>].
- [7] Greaves F, Joshi I, Campbell M, Roberts S, Patel N, Powell J. What is an appropriate level of evidence for a digital health intervention? *Lancet* 2018;392(10165):2665–7.