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Health technology assessment at age 25—Squaring the circle of strong methodology and context-dependency?

Health Technology Assessment (HTA) is the systematic evaluation of the properties and effects of a health technology, addressing the direct and intended effects of this technology, as well as its indirect and unintended consequences, and aimed mainly at informing decision making regarding health technologies. Already in 1994, i.e. 25 years ago, Health Policy published a special 424-page long issue on "Health care technology and its assessment in eight countries", namely Australia, Canada, France, Germany, Netherlands, Sweden, United Kingdom and United States [1,2]. Since then, around 300 articles have addressed HTA and related issues in Health Policy, studying HTA processes and policies within countries (most recently Japan [3]), developing HTA to better address the needs of health system needs [4], trying to classify HTA actors and systems across countries [5] or, more recently, taking a comparative perspective across countries regarding certain technologies (especially pharmaceuticals and their assessment [6]).

As a policy-informing tool, Health Technology Assessment (HTA) faces a dilemma: On the one hand, it stresses the availability and usage of methodologically sound studies. Thus far, a fair amount of energy has been invested in evaluating and classifying the quality of data sources as well as developing and optimizing the methodology to evaluate the evidence and report the results. On the other hand, it predicates the applicability of evidence and evaluation results to the national, regional or local context.

The first pillar – i.e. the consensus that standards of study methodology and evaluation in HTA apply independent of context – has developed substantially over the last 20 years: the European Collaboration for Assessment of Health interventions and Technology's "Best practice in undertaking and reporting HTA" was published in 2002 [7] and European collaboration on methodological development in HTA was institutionalized as the European Network for Health Technology Assessment in 2005 [8]. Developments in this direction have mainly led to relative homogeneity in how HTA institutions evaluate the evidence coming from RCTs, which are usually undertaken as part of drug licencing requirements. In principle, there is agreement also on the need for

(i) high-quality studies providing data beyond study settings, thus allowing a calculation of "community effectiveness" and "comparative effectiveness" (or "additional benefit") as compared to current practice (instead of only having data on efficacy, which is required for marketing approval),
(ii) expanding the methodology to technologies other than pharmaceuticals (especially medical devices), and
(iii) context-dependent evidence on ethical, organizational/ professional, social, legal, and economic implications of the introduction of new technologies.

While many decision-makers, and HTA institutions themselves, rightly stress the context-dependency of HTA-based decisions, the actual availability of high-quality "real world" studies (which allow an assessment of community effectiveness, rather than efficacy, and additional benefit) is hindered by the fact that specific HTA requirements (e.g. regarding patient subgroups or comparators) are defined in every decision-making context differently.

Building on 25 years of funding collaborative research on HTA (the first European project EUR-ASSESS started in 1994, at the same time as the above mentioned Health Policy issue), the European Commission issued a proposal for regulation mandating joint assessments of clinical elements (effectiveness and safety) for certain new technologies (drugs and some medical devices), while leaving the consideration of other domains such as the economic and organizational impact to national authorities on 31 January 2018. The proposal further institutionalizes joint scientific consultations (which will allow developers to seek advice from a newly instituted coordinating body of HTA agencies on the data and evidence likely to be required for the joint clinical assessment) and horizon scanning for emerging technologies expected to have a major impact on patients, public health or health care systems at the European level. The proposal was met with criticism from several sides and in early October 2018, the European Parliament adopted a report with a number of proposed amendments.

Setting the specifics of the proposal aside, proponents of a new phase of international collaboration in the sense of making joint clinical assessments mandatory argue that undertaking and using joint cross-country assessments would

(1) be based on joint methodologies and requirements, and that thus the availability of high-quality "real world" data will increase (much like harmonized drug licencing requirements have improved the quality of clinical trials), also for medical devices (with the new EU Medical Devices Regulation acting in the same direction) and possibly other groups of technologies (such as algorithms based on "artificial intelligence") and

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0168-8510/

(2) increase the availability of HTA reports especially in smaller countries, which cannot support their own HTA infrastructure.

Opponents, however, fear that context-specificity (and thus the usefulness of HTA for the national, regional or local setting) will get lost if assessments are done on a higher level.

Thus, there is a clear need to balance the possible benefits from joint assessments (and ensure that they materialize) and keeping context-specificity. Overall, this is a very fruitful point in time to invest further in identifying policy levers as well as procedural and methodological insights that can enable positive change in the area of HTA. This is the rationale behind this issue of Health Policy – 25 years after the first one.

While the variability in assessment processes and outcomes across countries identified by Visintin et al. (despite the very similar evidence base) points to a need for further understanding to foster readiness for joint assessments, it also substantiates the argument presented above regarding the difficulty of conducting widely applicable real world studies [9]. Cross-country differences in HTA methodology are accompanied by variability in reimbursement decisions. But are they sufficient to explain why patient access to medicines differs across countries, at least as far as reimbursement is concerned? In their contribution to this issue, Maynou and Cairns pursue a double goal: answering the question for cancer drugs and developing a foundation for robust, transferrable econometric modelling to aid policy makers and others in exploring drivers of HTA-based decisions in general [10]. Perhaps unsurprisingly, they find that economic parameters (such as the product's cost-effectiveness and the country's socioeconomic position) play an important role. However, the characteristics of the HTA system (labelled "system-level variables"), including differences in evidentiary requirements, do not seem to have a significant impact on the final decision.

Among the most challenging subjects for HTA are orphan medicinal products. Given their small target populations and the often limited understanding of the pathophysiological mechanisms behind rare diseases, orphan medicines are commercially unattractive and priced accordingly. The regulatory incentives instituted to address commercial unattractiveness, consisting mainly of favourable conditions for market entry and exclusivity, complicate the work of HTA both in terms of available evidence and value for money. Based on the logic delineated above, orphan drugs are prime candidates to accrue the benefits of joint assessments, particularly regarding the facilitation of primary research (which in turn has the potential to lower R&D costs and improve value). Nicod et al. evaluate existing initiatives towards enabling HTA approaches tailored to orphan medicines, including the European project MoCA, a type of continued dialogue between manufactures, payers and other stakeholders that aims to optimize evidence generation on and access to orphan medicines [11]. The growing consensus around the insufficiency of "traditional" HTA methods for determining the value of orphan medicines was the motivating force behind the research carried out by Lopez-Bastida et al., who used a discrete choice design to determine important attributes for coverage decision-making in the realm of rare diseases and conclude that such designs can be used to ensure "fair play" in the reimbursement of orphan medicines [12]. Their contribution is both methodological and confirmatory regarding the elements that are important for this specific group of technologies (e.g. severity of disease, onset of the condition early on in life but also side effects and improvement in health, which further support the argument for strengthening primary research).

Quality of life plays a particularly important role in HTA as a crucial dimension of understanding improvement in health and quantifying relative effectiveness. Particularly for HTA approaches using quality-adjusted life years (QALYs) for economic evaluation, carefully considering the methodology of measuring health-related quality of life (HRQoL) is of critical importance. Two contributions in this issue investigate the implications from an HTA perspective of what (Efthymiadou et al. [13]) and who (Ogorevc et al. [14]) is asked when the EQ-5D-5L instrument is used to measure HRQoL. This widely used operationalization was first published in this journal almost 30 years ago [15] and has been broadly discussed and adapted since.

As highlighted earlier, health technologies beyond pharmaceuticals, such as medical devices, continue to be "underserved" by HTA, despite considerable progress in recent years and repeated calls for change in scientific and policy-relevant publications. Given the rapid pace of innovation in the area of medical devices – further compounded in recent years by the proliferation of digital health applications – collaborative assessments combined with reasoned prioritization of technologies to evaluate seem like the most feasible way forward towards achieving HTA-based decisions. Building upon work also published in Health Policy [16], Fuchs et al. test a theoretical taxonomic model for prioritizing medical devices for assessment by systematically analyzing the practices of European HTA institutions and verify areas where particular methodological consideration is needed [17]. García-Mochón et al., looking at HTA structures and processes in emerging settings in Europe, stress that most if not all activities are focused on pharmaceuticals – substantiating one of the other favourable arguments regarding increased collaboration in HTA: providing the tools to inform coverage decisions in countries without established structures for the evaluation of new technologies [18].

Finally, this issue takes a look at Canada, a country with a long and varied tradition in HTA. Wranik et al. identify internal and external threats to the robustness of HTA, arising from undue influence on appraisal committee members and evidence generation, respectively [19], while McNeil et al. investigate barriers and facilitators for innovation in health technologies, starting from their development, through HTA and to monitoring and sustainability practices [20].

Health Policy will continue to follow, critically assess and support the further development of HTA to contribute to squaring the circle of maintaining high methodological standards while care of context-dependency and specificities of various types of technologies.

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