Providing Evidence for Evidence-Based Health Policies: Comparative Effectiveness Research in Four Countries

In all developed countries, the share of healthcare in GDP is steadily growing (Anderson et al. 2007). This development is driven by both the supply and demand side: in the medical sector, new technologies and procedures usually do not save resources (Newhouse 1993) but "buy" additional years of life. These extra life-years are also typically spent in better health but nevertheless feed back into increased healthcare consumption and cost. Unlike growth in other sectors of the economy, and despite healthcare's undisputed role in increasing welfare, the expansion of the health sector is frequently considered problematic. A part of these concerns arises from the inevitable re-distributional effects of universal healthcare provision. Another part is driven by the suspicion that healthcare is often provided inefficiently. Stakeholders, it is assumed, tend to exploit the information asymmetries and complicated buyer-seller relations that are typical in the healthcare sector to maximize individual profit. This issue is very evident, but not limited to the pharmaceuticals sector. Manufacturers issue new drugs and, citing successful trials, seek coverage under private and public financing schemes. However, since these trials are usually carried out or funded by the manufacturers themselves their scientific quality has been questioned (Relman and Angell 2002). In addition, in many healthcare systems financial coverage for new drugs does not require evidence that the new (more costly) drug represents an actual medical advancement over comparable existing products. As a result, pharmaceutical spending is unnecessarily increased by costly pseudo inventions (Relman and Angell 2002).

Against this background, more and more countries seek to impose mechanisms that increase healthcare efficiency. One means is the establishment of independent institutions that assess new treatment options and broader health management strategies in terms of their relative clinical effectiveness, safety, and in certain cases, their relative costs. In a recent article, Chalkidou and colleagues (2009) review key features of such Comparative Effectiveness Research (CER) institutions in four countries, namely for Britain's National Institute for Health and Clinical Excellence (NICE), France's Haute Autorité de Santé (HAS), Germany's Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (IQWiG) and Australia's Pharmaceutical Benefit Scheme (PBS). CER institutions also exist in countries like Canada and New Zealand, and in the United States the recent economic stimulus bill assigns USD 1.1 billion to the development of CER (U.S. Congress 2009). By choosing Britain, France, Germany and Australia the authors cover CER entities across very different healthcare systems: Britain has a tax-financed, nationalized single payer system, whereas Germany and France use insurance systems with public and private options. Australia has a public tax-financed system that is supplemented by private insurance options.

The Table summarizes main characteristics of the four CER entities under review. Australia was the first to establish a CER mechanism that is limited, however, to the assessment of pharmaceutical products. In contrast, NICE, HAS and IQWiG cover a much broader scope of responsibilities. Their field of work not only includes the assessment of medical technologies and procedures but also the evaluation of public health programs, the provision of patient information and, in the case of HAS, the accreditation of new hospitals.

The explicit weighing of economic cost against health benefits, for instance to inform pricing decisions for new drugs often dominates the CER institutions' public perception. However, with the exception of NICE which has considered cost in its assessments since its inception in 1999, the original purpose of the reviewed CER entities was limited to clinical effectiveness comparisons, while cost-effectiveness and budgetary impact research were introduced later to complement the clinical dimension.

The methods of assessment are similar across the four countries. For their recommendations, all predominantly rely on the synthesis of existing primary studies rather than conducting primary research themselves. There are different reasons for this. On the one hand, primary studies are time-consuming whereas secondary synthesis studies permit a timely market launch of new medical technologies. On the other hand, the budgetary constraints faced by CER institutions – none receives more than 0.1 percent of national healthcare expenditure – render own trials financially unfeasible. The reliance on secondary research alone however bears certain problems. Firstly,

Table

Key features of CER institutions in Great Britain (NICE), France (HAS), Germany (IQWiG) and Australia (PBS)

	NICE	HAS	IQWiG	PBS
Budget and source of funding	Government funded (Ministry of Health), £35 million per year.	€70 million in 2006. 34% from taxation of pharmaceutical firms' spending on advertising, 15% from hospital accreditation fees, 7% from fees for medical devices and drug manufacturers, 32% from insurers, 10% from government, 2% investment income.	€20 million per year. Levy based on a percentage of each reimbursed case in the Statutory Health Insurance fund to grant independence from stakeholders.	Government-funded: uncapped as all activities are demand-driven.
Scope of assessment	Coverage recommendations for medical technologies like drugs, devices, and diagnostic tests; clinical guidelines for disease management; public health guidelines for disease prevention; health promotion programs; information for patients.	Medical technologies like drugs, devices, procedures and diagnostic tests for pricing and reimbursement; hospital accreditation; clinical guidelines for disease management; public health guidelines for disease prevention; health care system organization; labelling of patient information websites.	Medical technologies like drugs, devices, surgical procedures and diagnostic tests; quality control; guidelines for disease management programs; evaluation of clinical guidelines; development of information for patients and the general public.	Assessment of prescription drugs for subsidy.
Topic selection	Own decision based on explicit criteria, but final approval for assessment of new technologies is ministerial responsibility.	Companies seeking listing on formulary, Ministry of Health, suggestions from other stakeholders such as insurers, medical societies, patients' associations.	Own initiatives or by commission from FJC or Ministry of Health.	Pharmaceutical companies seeking listing on formulary.
Research used and conditional coverage decisions	Synthesis of existing studies, economic modelling, small number of prospective trials funded by public source. Conditional coverage: "only in research option" of conditional reimbursement where effectiveness data are collected in the real world and NHS receives a rebate if the new technologies do not work as promised – increase in price is also possible if the measure works better than initially assumed.	Synthesis of existing studies, increasing use of economic modelling and public health analyses. Analysis of post-marketing or postlisting studies that can be required by HAS. Conditional coverage: temporary access for some new and innovative health products or procedures possibly limited to certain health centres when their effects are uncertain.	Synthesis of existing evidence, economic modelling. Based on IQWiG's recommendation, FJC can decide to undertake "coverage with evidence development".	Pharmaceutical companies present synthesized evidence on proposed drug, ideally in comparison with similar drugs. Economic modelling is required.
Consideration of costs in assessment	Comparative cost-effectiveness analysis since inception in 1999. Budget impact analysis to inform implementation but not as decision input.	Consideration of economic dimension in assessment since January 2008. Cost-effectiveness however not part of initial listing decision of medical technologies in which HAS offers advice on price and co-pay levels set by government and insurers – here only clinical effectiveness plays a role. When reassessing classes of drugs, devices or organizational aspects of health delivery, cost-effectiveness now supplement the clinical effectiveness data.	Cost-benefit analysis since 2007; budget impact analysis to provide a decision basis for ceiling prices of drugs under development.	Comparative cost-effectiveness analysis since 1988, mandatory since 1993; budget impact analysis is mandatory and considered part of recommendation.
Status of guidance	Guidance on medical technologies binding (must be covered by NHS); public health and clinical guidelines have advisory status.	Guidance on drugs and devices effectively binding since 1999. As a basis for Ministry of Health and National Insurance Fund pricing and reimbursement decision. Guidelines for procedures and other public health and clinical guidelines have advisory status.	Advisory to the Federal Joint Committee, which after approval from the Ministry of Health issues a binding directive.	Positive advice on listing, pricing, and reimbursement is subject to ministerial/parliamentary approval; negative advice is mandatory.

Source: Chalkidou et al. (2009).

as discussed above, the large majority of clinical trials are carried out by the new therapies' manufacturers themselves and full transparency of these trials is often not warranted. Secondly, comparisons between similar technologies are complicated when the number of available studies is small and the study methodologies differ, as is often the case. Against this background, CER institutions are increasingly experimenting with conditional coverage arrangements. Here, new therapies enter the market and receive preliminary coverage under the respective healthcare system but manufacturers are required to produce additional evidence for the value-added of their product after its launch.

None of the CER-institutions is completely free to choose the subjects of review. Instead prioritized topics are determined mutually with the healthcare systems' stakeholders – policy makers, insurers, health care providers and patients.

The status of CER guidelines varies between the different subjects of assessment and by country. NICE's judgements in terms of drug coverage are for example binding, while IQWiG's ceiling price recommendations only serve to inform a final decision taken by the Federal Joint Committee (FJC) which includes representatives from payers (insurance funds) and providers (hospitals and professional associations). Guidelines for best clinical practices are usually non-binding but efforts are increasingly made to ensure a broad implementation. NICE here has the largest variety of instruments at its disposal ranging from monetary incentives for clinicians (prices reflecting cost of best practices) to linking adherence to NICE standards to the accreditation of providers in the National Health System (NHS) and guaranteeing every patient a right to NICE recommended treatments.

In sum, while the scope of assessment and the regulatory powers vary across CER institutions, they share, in addition to their common purpose, key structural and technical characteristics. With a growing need to appropriate limited healthcare resources effectively and fairly, evidence based health policy and CER as its basis will continue to grow in importance.

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