

SPOTLIGHTING INTERNATIONAL INNOVATION

Using Comparative Effectiveness Research to Inform Policymaking

Comparative effectiveness research (CER) assesses alternative treatments or diagnostic options for the same condition. Such research can prove useful for clinicians and patients as a tool to inform decisions about treatment and care. It also has potential to inform policymaking, such as decisions over which treatments to cover and at what price. In the United States, the 2009 Recovery Act for the first time [provided significant funding for CER](#), and the 2010 Affordable Care Act went further, establishing an independent institute to commission such research—the [Patient-Centered Outcomes Research Institute](#)—with dedicated long-term funding.

Several industrialized countries have operated organizations conducting and commissioning CER for many years. In some countries these bodies are government agencies, while in others they are freestanding organizations with more independence. Policymakers often use the research these organizations generate to determine the content of publicly provided health benefits—for example, to decide whether a new drug should be covered under regional or national formularies. Other uses include negotiating pricing arrangements with drug companies or designing “value-based” cost-sharing arrangements, wherein patients pay more out-of-pocket for drugs deemed less effective than their alternative. As the Patient-Centered Outcomes Research Institute develops and CER becomes more widely available, U.S. decision-makers [can learn from international experiences](#) using CER to drive health care toward improved quality and value.

England’s National Institute for Health and Clinical Excellence

The [National Institute for Health and Clinical Excellence \(NICE\)](#) was launched in 1999 as part of major reforms to improve access and quality and reduce variation in the British National Health Service. These reforms were accompanied in 2002 by deliberate, significant increases in national health care spending, with a stated goal for NICE being to ensure that these investments were not wasted on low-value treatments and medical technologies.

NICE only carries out a small amount of CER internally. In the majority of cases, NICE commissions CER—usually in the form of evidence synthesis and economic modeling—from other research organizations or considers such evidence presented by industry sponsors. Most new drugs and technologies are reviewed, with NICE then making recommendations on whether they should be covered under the National Health Service. Positive recommendations are mandatory for all providers across the National Health Service: drugs or treatments receiving a positive recommendation must be offered. Those receiving a negative recommendation can still be offered if considered clinically appropriate, but a special case has to be made to the local purchasing agency for their reimbursement. NICE operates independently of the Ministry of Health and, while the ministry can override its recommendations, this has not yet occurred. In addition to drugs and medical devices, NICE also conducts CER on diagnostic techniques, surgical procedures, and health promotion

activities. [NICE explicitly considers cost-effectiveness in making its recommendations](#). In addition to commissioning CER, NICE commissions and oversees the development of clinical guidelines by professional organizations, develops audit and educational tools, and in other ways supports activities to reduce variation and improve quality throughout the National Health Service. NICE recommendations inform the pay-for-performance scheme for primary care physicians (Quality and Outcomes Framework), the similar scheme for the hospital sector, and, most recently, has become responsible for developing metrics for the Commissioning Outcomes Framework – a pay-for-performance scheme for payer organizations across the NHS.

The future role of NICE will be shaped by [recent U.K. health reforms](#). One likely change will be to shift toward [value-based pricing](#), in which NICE's evaluations of comparative effectiveness may be used by the Ministry of Health in negotiating prices for new drugs and technologies. NICE is also experimenting with industry on risk-sharing or conditional reimbursement schemes for drugs or interventions for which the evidence base is too weak to make a final recommendation.

Further reading:

- S. Bryan, I. Williams, and S. McIver, [Seeing the NICE Side of Cost-Effectiveness Analysis: A Quantitative Investigation of the Use of CEA in NICE Technology Appraisals](#), *Health Economics*, Feb. 2007 16(2):179–93.
- K. Chalkidou, [Comparative Effectiveness Review Within the U.K.'s National Institute for Health and Clinical Excellence](#) (New York: The Commonwealth Fund, July 2009).
- K. Chalkidou, S. Tunis, R. Lopert et al., [Comparative Effectiveness Research and Evidence-Based Health Policy: Experience from Four Countries](#), *Milbank Quarterly*, June 2009 87(2):339–67.
- R. R. Faden, K. Chalkidou, J. Appleby et al., [Expensive Cancer Drugs: A Comparison Between the United States and the United Kingdom](#), *Milbank Quarterly*; 88(4):789–819.
- C. Sorenson, [Use of Comparative Effectiveness Research in Drug Coverage and Pricing Decisions: A Six-Country Comparison](#) (New York: The Commonwealth Fund, July 2010).

France's National Authority for Health (HAS)

In France, the [National Authority for Health](#) (Haute Autorité de Santé, or HAS) was established in 2005 to optimize the public health benefit package, as well as undertake a number of quality improvement activities including defining best practice, publishing guidelines, certifying doctors, and accrediting health care organizations. It is an independent, scientific institute, funded in large part through earmarked tax dollars. Among its activities, HAS performs or commissions CER on all new drugs and devices, and issues an opinion on whether they provide superior results or higher value than the current standard. The health ministry makes the final decision whether to add the drug or device to the public benefit package, generally following HAS's opinion. HAS opinions are also considered by the committee that determines price and copayment levels. HAS also offers opinions on procedures and tests; final decisions on whether to add these to the benefit package are made by the unions of “sickness funds” (public insurers), rather than the health ministry.

Further reading:

- K. Chalkidou, S. Tunis, R. Lopert et al., [Comparative Effectiveness Research and Evidence-Based Health Policy: Experience from Four Countries](#), *Milbank Quarterly*, June 2009 87(2):339–67.

- L. Rochaix and B. Xerri, [National Authority for Health: France](#) (New York: The Commonwealth Fund, July 2009).
- C. Sorenson, [Use of Comparative Effectiveness Research in Drug Coverage and Pricing Decisions: A Six-Country Comparison](#) (New York: The Commonwealth Fund, July 2010).

Germany's Institute for Quality and Efficiency in Health Care

The [Institute for Quality and Efficiency in Health Care \(IQWiG\)](#), established in 2004, tenders, conducts, and, occasionally, commissions evaluations of health services and then provides recommendations to the authority (the “Federal Joint Committee”) that determines coverage and pricing in the statutory health insurance benefit package. A positive recommendation is not required for a new drug or health service to be accepted in the benefit package—all are covered by default, with IQWiG then conducting evaluations on request by the Federal Joint Committee. IQWiG evaluations consider whether new drugs offer additional benefit compared with other treatments. IQWiG is financed through a small fee on ambulatory visits and hospital stays within the statutory health insurance system and operates as an independent foundation. In addition to CER evaluations, IQWiG produces reviews of clinical guidelines and methodological studies.

Further reading:

- K. Chalkidou, S. Tunis, R. Lopert et al., [Comparative Effectiveness Research and Evidence-Based Health Policy: Experience from Four Countries](#), *Milbank Quarterly*, June 2009 87(2):339–67.
- A. Gerber, S. Stock, and D. Charalabos-Markos, [Reflections on the Changing Face of German Pharmaceutical Policy: How Far is Germany from Value-Based Pricing?](#) *Pharmacoeconomics* 2011; 29 (7):549–53.
- M. Nasser and P. T. Sawicki, [Institute for Quality and Efficiency in Health Care: Germany](#) (New York: The Commonwealth Fund, July 2009).
- C. Sorenson, [Use of Comparative Effectiveness Research in Drug Coverage and Pricing Decisions: A Six-Country Comparison](#) (New York: The Commonwealth Fund, July 2010).

Australia's Pharmaceutical Benefits Advisory Committee

Since 1953, Australia's [Pharmaceutical Benefits Advisory Committee \(PBAC\)](#) has [reviewed all drugs](#) applying to be listed on the formulary of publicly covered drugs and made recommendations to the government. In 1988, the PBAC began to incorporate comparisons of clinical effectiveness and cost effectiveness versus existing therapies in its decision-making. Since the early 1990s, such comparative cost-effectiveness assessment has been mandatory. Following review, the PBAC makes a coverage recommendation to the minister of health, who makes the final decision but cannot list a drug in the absence of a positive recommendation. The PBAC is an independent government committee, and contracts with academic institutions for evaluation support. The PBAC's remit encompasses decisions regarding the national drug formulary, as well as vaccines for inclusion in the national immunization program. The PBAC does not determine prices; a drug's price is put forward by the applicant seeking listing and accepted if the medicine is considered comparatively cost effective at the price proposed.

Further reading:

- K. Chalkidou, S. Tunis, R. Lopert et al., [Comparative Effectiveness Research and Evidence-Based Health Policy: Experience from Four Countries](#), *Milbank Quarterly*, June 2009 87(2):339–67.

- R. Lopert, [*Evidence-Based Decision-Making Within Australia's Pharmaceutical Benefits Scheme*](#) (New York: The Commonwealth Fund, July 2009).
- S. G. Morgan, M. McMahon, C. Mitton et al., [*Centralized Drug Review Processes in Australia, Canada, New Zealand, and the United States*](#), *Health Affairs*, March/April 2006 25(2):337–47.
- C. Sorenson, [*Use of Comparative Effectiveness Research in Drug Coverage and Pricing Decisions: A Six-Country Comparison*](#) (New York: The Commonwealth Fund, July 2010).