How does society use evidence to judge the risks and benefits of medicines?

History

During the 1980s two eminent academics sought to influence the British Government to base the choice between alternative health treatments (including medicines) on scientific evidence. The late Professor David Sackett, epidemiologist working in the US but later based in Oxford, advocated 'evidence-based medicine' in which individual clinical decisions depend on evidence on the ***relative clinical effectiveness*** of treatments under consideration. The late Professor Alan Williams of the Centre for Health Economics at the University of York advocated cost per quality-adjusted life-year (QALY) as the criterion for those decisions. Though that strictly assesses the ***relative cost-effectiveness*** of alternative treatments, Sackett and Williams soon recognised their common goal, and agreed that Sackett should focus on effectiveness and Williams on costs. This enabled Frank Dobson, Secretary of State in the New Labour Government that took office in 1997, to create the National Institute of Clinical Excellence (NICE – now the National Institute for Health & Care Excellence) as the executive public body responsible for improving the quality of care as judged by health outcomes.

Strengths of EBM as implemented by NICE

NICE has acquired a strong international reputation as a model for the rigorous evaluation of health technologies in the widest sense, the explicit definition of cost-benefit criteria for those technologies, and the thorough development of clinical guidelines to disseminate these evaluations. NICE also supports technology assessment in other healthcare systems through NICE International. NICE has ameliorated the 'postcode lottery' of healthcare decision-making, whereby choice of treatment depended upon the area in which the patient happens to live. In seeking rigorous evidence about health technologies, NICE has empowered the National Institute of Health Research to build arguably the strongest programme of applied health research in the world, and the Cochrane Collaboration to strengthen its international programme of systematic reviewing led from the UK.

Weaknesses of EBM as implemented by NICE

NICE's founding principles included giving equal value to QALYs regardless of who gained or lost them. When surveys showed that the general public wished to discriminate in favour of terminally ill patients, NICE increased the threshold for funding medicines for terminal cancer patients. More recently NICE has relaxed thresholds for drugs for rare conditions, which might never achieve a universal threshold.

There has been scientific criticism of the EQ5D, the European instrument used by NICE to measure and value health-related quality of life, arguing that the use of five dimensions each measured on a 3-level scale was not sensitive to the small changes in clinical effectiveness typical of improvements in medications. Fortunately evidence is emerging that the recent introduction of 5-level scales in the form of the EQ5D5L may soon ameliorate that weakness.

Most criticism of NICE and its methodology has come from the pharmaceutical industry arguing that it discriminates against individual drugs, and from specific patient groups arguing that it discriminates against their members. While NICE's concessions to medicines

for patients with terminal or rare conditions were understandable, they have unfortunately encouraged much ill-informed lobbying.

Synthesis

My past appointments listed below show I have a vested interest in evidence-based medicine and the conduct of clinical trials and economic evaluation to underpin it. Nevertheless I judge that NICE and its methodology has made an enormous contribution to health and welfare across the UK, indeed the world. Even so I support the intention of the Academy of Medical Sciences to review the evaluation of medicinal products in general, and NICE and its methodology in particular. I am confident that they will be able to suggest several marginal improvements. But my main conclusion is that NICE is in good shape and does not need radical change.

References

Wikipedia. National Institute for Health and Care Excellence. Accessed 28 September 2015.

Ian Russell
Senior Research Leader, Health & Care Research Wales (2013-6).
Professor of Clinical Trials, Swansea University (2008-15).
First Chair of Health Technology Assessment Commissioning Board, National Institute for Health Research (1993-5).
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