approval, however, cover all types of healthcare research (including RCT and RW data studies) and it is essential that the HRA considers RW data studies specifically, as progress is made towards addressing this important recommendation. This offers an opportunity for the UK to be a more attractive environment for the conduct of RW data studies.

• **Skills and education**

The strong links that the UK pharmaceutical industry has with the academic community are crucial in ensuring the appropriate skills are identified and developed to support this growing area of RW research. The pharmaceutical industry has a responsibility to ensure that the personnel involved in RW data projects locally have the appropriate knowledge level or, alternatively, to secure the necessary support for study design and collection, analysis and subsequent use of these data.

SUMMARY

It is well recognised that data about patients' use of medicines in normal clinical practice, or in settings which reflect the reality of health care delivery – Real World data – are likely to become increasingly important in decisions that affect patients' access to medicines.

The UK is already well placed to lead the world as a centre of excellence for the collection and use of this type of data. The plans announced in December 2011 have been welcomed and help move even closer to this goal. However, it is essential that ongoing consideration is given to the remaining challenges raised here if we are to optimise the benefits to the UK that could be afforded by this opportunity.

Footnotes

1 Kinapse. Commercial Clinical Research in the UK: A report for the Ministerial Industry Strategy Group (MISG) Clinical Research Working Group. November 2008

2 Department of Health. A new value-based approach to the pricing of branded medicines: Government response to consultation. July 2011

Available at: http://www.dh.gov.uk/en/Consultations/Responsestoconsultations/DH_128226 (accessed on 14/12/11)

'COST-PER-QALY IN THE US AND BRITAIN: DAMNED IF YOU DO AND DAMNED IF YOU DON'T'

Dr Adrian Towse

Cost-per-Quality Adjusted Life Years (QALY) is the means by which the value of a medical intervention can be quantified, and is used by the National Institute for Health and Clinical Excellence (NICE) to determine the cost-effectiveness of medicines. This was the subject of the Office of Health Economics' Annual Lecture, given on 15 November in London by Dr Milton Weinstein, Henry J Kaiser Professor of Health Policy and Management at the Harvard School of Public Health.

The lecture was something of a social commentary on the differences in attitudes in the UK and the USA regarding healthcare costs and, in particular, cost-effectiveness analysis costs per QALY. One quote by Dr Weinstein summed this up: 'If you cannot tell from the title, you are the folks who do and we are the folks who don't … In my country we do not touch cost-effectiveness analysis with a 10-kilometre pole: in this country you seem to have a love affair with it'.

Dr Weinstein gives a number of arguments deployed in the USA for not using cost-effectiveness analysis. The most prominent of these is that there is no relation between healthcare expenditures and health outcomes across hospitals in the USA. This, according to Dr Weinstein, is actually true – the association between overall expenditures and outcomes tends to be a 'very fuzzy relationship'. Together with Jonathan Skinner of Dartmouth Medical School, Dr Weinstein recently wrote a paper published in the *New England Journal of Medicine* about what this weak relationship between expenditures and outcomes implies about the need for cost-effectiveness analysis.

What he showed in this paper is that healthcare expenditures are not used most efficiently. There are many situations in which many of the most cost-effective health services and interventions are under-utilised. For example, fewer than half of Americans over the age of 50 have ever had a colorectal screening exam; nor do people get their influenza vaccinations or pneumococcal vaccinations as recommended. For a state to cut its expenditures and improve health outcomes simultaneously, Dr Weinstein concludes it needs to increase the utilisation of highly cost-effective interventions like these and simultaneously cut back on less cost-effective

interventions – and cost-effectiveness analysis is needed to do this.

A study by Skinner and Staiger, available as a National Bureau of Economics research report, looked at the rate of adoption of three highly cost-effective technologies for acute myocardial infarction (MI) – aspirin, beta-blockers and reperfusion. Now almost every hospital is using these to the full, but back in the 1980s and 1990s there was a period where hospitals adopted them at different rates.

Using regression analyses the study looks at the relationship between expenditures and outcomes for acute MI after the hospitals were stratified by their rate of adoption of these cost-effective technologies. The fastest-adopting quintile of hospitals have better outcomes than the slowest and – counter to the opinion that Dr Weinstein spoke of as being widespread in the US – there is a positive relationship between expenditures and outcomes in all the strata. So to cut costs and improve outcomes, hospitals would have had to adopt the cost-effective technologies more rapidly.

Another argument, one that the US Congress has decided to invest in, is that if we do more research on comparative effectiveness of health interventions we can identify the interventions that are useless, leaving enough money saved to pay for everything that is useful. The fact, Dr Weinstein explains, is that it is very hard to prove that something is useless. Randomised trials, if they are feasible, are not intended to prove a negative, and just because you cannot show that an intervention is better than its alternative it is very hard to show that it is exactly equivalent to the alternative. Most interventions do not lend themselves to randomised clinical trials and we have to rely on other sources of evidence, and it is very hard to prove beyond a reasonable doubt that an intervention is absolutely useless.

One argument backed by Dr Weinstein is that QALYs do not reflect everything that people care about in healthcare. For example, there may be value in some genetic testing that tells people what risks they face as they proceed through life, or what risks their child faces. Even if you cannot do anything about it, there is the psychological value of knowing. Caring does not necessarily manifest itself in more QALYs but it is something that people value. Similarly, access to care, equity, and reducing disparities in society are things that people value but which do not reflect themselves in maximising QALYs.

Dr Weinstein was co-chair of the US Panel on Cost-effectiveness in Health and Medicine which reported to the US Government in the 1990s. One of the most important recommendations the panel made is that cost-effectiveness analysis is an aid to decision-making, not a complete procedure for making resource allocation decisions, because it cannot incorporate all the values relevant to such decisions. Dr Weinstein thought that NICE and Britain should be mindful of this, saying that 'sometimes in one's enthusiasm for the cost-effectiveness model – and I am certainly one of the enthusiasts – we need to temper that enthusiasm with the limitations and be mindful of the role that this type of analysis has among many other considerations – ethical, psychological and otherwise'.

Dr Weinstein posed a question - do the British take prescribed guidelines for cost-per-QALY modelling too seriously? The purpose of a model is to inform medical decisions and healthcare resource allocation. Modellers employ quantitative methods to gain qualitative insights. The purpose is not so much the number that comes out as to gain the qualitative insight. The tools of formal analysis are best employed to structure the clinical, epidemiological and economic evidence base in the service of better clinical practice decisions and public health priorities.

Finally, he noted that there is a role for deliberative processes through which individuals and stakeholders, including the general public, can get involved in conversations about how costs and benefits should be traded off against one and another, and with other ethical and psychological factors that people believe should go into decision-making.

STANDING UP FOR ORPHANS

John Irwin
Co-Chair ABPI Orphan Medicines Industry Group

RARE DISEASES IN THE UK

2012 sees the publication of the UK's first Plan for Rare Diseases. This represents an important landmark for the estimated 3.5 million patients in the UK believed to be living with a rare disease. This plan has been delivered in response to a commitment made in the response to the Council of the European Union Recommendation on an action in the field of rare diseases (2009/C 151/02) to 'establish and implement plans or strategies for rare diseases at the appropriate level … in order to aim to ensure that patients with rare diseases have access to high quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan medicines.'

A rare disease is defined by the European Union as one that affects fewer than 5 in 10,000 of the general population. There are between 6,000 and 8,000 known rare diseases and it is believed that 7 per cent of the population will be affected by a rare disease at some point in their lives. Seventy-five per cent of rare diseases affect children