A LITTLE LEARNING: REFLECTIONS ON 10 YEARS OF NICE TECHNOLOGY APPRAISALS

CAROLE M. LONGSON

1. INTRODUCTION

It is now almost 12 years since I joined National Institute for Health and Clinical Excellence (NICE). In those early years, neither I nor anyone else anticipated the importance of the journey we had started in the Technology Appraisal Programme. This very personal review tracks that journey with a few anecdotes and commentary on the input of the health economic community in the UK.

2. THE EARLY YEARS: STORMS AND TEACUPS

Within just a few days of joining NICE, I realised the scale of the task. I thought that grounding in biological science, pharmaceutical R+D and the actual production of submissions to NICE had equipped me with relevant knowledge and skills. How naïve; this grounding was merely the starting point of what was needed. The realisation crystallised for me over initial briefings on the ongoing appraisal of drugs for multiple sclerosis. I had joined NICE at the point that it was concluded that the health economic models produced for the appraisal, from pharmaceutical companies and from the academic assessment team, did not help the appraisal committee decision makers reach conclusions on the drugs with sufficient certainty. The decision: NICE would commission the development of another health economic model. The commission went to the School of Health and Related Research in Sheffield UK (ScHARR).

The first major task after my appointment was to work with the ScHARR health economists to produce an economic model that would provide robust estimates of the cost-effectiveness of the multiple sclerosis (MS) drugs. That could not be too difficult. This was a world-leading research group who had experience in the therapeutic area. I was wrong. I had forgotten that my new environment was not one where the only people scrutinising the science were a critical bunch of like-minded scientists. I was now in a world of transparent, inclusive and interactive health policy decision making.

In its forming principles, NICE committed itself to working in a way that no other NHS health policy organisation before it had worked. The words transparent and inclusive are simple words. Implementing the principle of engagement that lies behind them is not simple at all. I was about to find out how difficult it could get. People wanted to know, in detail, what was wrong with the current models. These stakeholders had submitted the existing models and thought they had produced robust submissions, some with input of leading academic modellers. Why could ScHARR do it any better than they had? How could they interact with the research group? Could they submit data? Could they scrutinise this output? We provided a mechanism for

*Correspondence to: Centre for Health Technology Evaluation, National Institute for Health and Clinical Excellence, Manchester M1 4BD, UK. E-mail: carole.longson@nice.org.uk

Copyright © 2011 John Wiley & Sons, Ltd.
engagement with stakeholders during model development, convening meetings and allowing submission of
data and analysis to the modelling group. However, at that time, I was ill-equipped to deal with this intensity
of interaction. I learnt quickly that having tea available helped when the conversation became tricky. ‘I think
it’s time for refreshments’ became a frequent comment during the exchanges.

Many commentators have covered the NICE appraisal of drugs for MS. Most reflect on the Department of
Health risk sharing scheme, initiated as a result of the NICE guidance. Views expressed have mainly been
about the usefulness, or otherwise, of this type of arrangement and whether the data emerging from the scheme
will be useful to establish value with greater certainty. In my view, many commentaries miss the point
somewhat: the significance and importance of the actual decision of the appraisal committee. A decision that,
with the evidence available, the drugs could not be recommended as a clinically and cost-effective use of NHS
resources. NICE came under huge challenge to justify this decision to a very a knowledgeable and articulate
group of stakeholders. The scrutiny we came under during the multiple sclerosis appraisal was a formative
experience for the appraisal programme. It formed our understanding of the level of process detail needed to
interact professionally with informed stakeholders.

3. DISSONANCE IN ACADEMIA

The UK had a well-developed and groundbreaking health technology assessment programme long before NICE
was established. The HTA programme, part of the NHS Research and Development programme, produced reports
using state of the art techniques. The UK HTA programme was, and continues to be, one of the world leaders in
HTA. It was an obvious conclusion that NICE would need the expertise and resources of the HTA programme to
deliver the evidence assessments to the technology appraisal committee. Back in 2000, the input of health econom-
ic on HTA reports was regarded by most as a bolt on to the robust, well-tested methodology of systematic review-
ing. Many systematic reviewers were sceptical of the views of health economists to maximise the use of all relevant
evidence by developing a structured framework (a model) to present and explore the decision at hand.

In the early years, the clinical and economic sections of some HTA reports for NICE sometimes lacked
coherence and cohesion: a direct consequence of the dissonance in approach. This was not helpful for NICE.
What should we do? Follow the tried and tested approach of most of the HTA community or side with the
‘interloping health economists’ invading the space of a well-respected system. The decision parameters were
clear. What does the NICE decision maker on the appraisal committee need? What does NICE need to be able
to justify the decision to stakeholders. The conclusion: a new type of HTA, NICE HTA, was needed in this new
world of transparent, HTA-informed decision making. We needed a report structure that enabled us to map out
the issues for the decision maker and stakeholder, we needed to extrapolate current evidence into the future to
predict downstream outcomes, we needed to demonstrate that we have been inclusive in our use of available
evidence, and we needed to be clear when we had moved too far beyond the available evidence, thus creating
too much uncertainty for the decision maker.

To achieve this, we all needed to adopt a mindset prevalent in the health economic community. This was
absolutely crucial to the long-term sustainability of the NICE technology appraisal programme. Note that I
emphasise the mindset. The issue is not about the relative importance of various disciplines. The issue is not about
expertise. At the time NICE was established, the health economic community structured their thoughts in a way
that was more aligned to what was needed. A good example of this was that back in 2000, the use of Bayesian
statistics in health economics was already becoming widely adopted, reflecting the decision making focus of the
community, but that these methods were not and are still not widely adopted within the broader HTA community.

4. ON BALANCE OR BEYOND DOUBT

At the start of the appraisal programme, the evidence base submitted for NICE appraisal did not always allow
us to meaningfully explore the question at hand: in particular, economic analysis often was presented either
very scantily or as gobbledygook, understandable only to the few card-carrying health economists engaged
with the process. Our appraisal committee decision makers needed to be very brave. They could not hide behind an opaque evidence base. The products being appraised were on the market. There was no possibility of deferring the majority of decisions. We needed a way to be able to navigate through the confusion. The health economic community provided a solution. Let (probabilistic) analysis of uncertainty help. If decision makers know how certain their conclusions are, then surely this would be sufficient to convince stakeholders of the validity of the decision. The acceptance of this premise depends on the decision. Most stakeholders accept uncertainty if, on the balance of probabilities, a decision is in line with their expectations. However, it is different for a decision that goes against this. NICE and its decision makers need to able to justify these decisions beyond doubt. The skew in the degree of interrogation, analysis and scrutiny required for negative decisions continues and is entirely understandable. Probabilistic analysis is still not well understood. Anything the health economic community can do to help translate the complexity of probabilistic modelling into a language understood by a mass audience would be a significant contribution.

5. AD IUDICUM

Being a member of a NICE technology appraisal committee must be one of the most challenging tasks in UK public life, and great admiration and huge appreciation must go to the people who give up their time to undertake what must, at times, seem like a thankless task. The members of the appraisal committee people inject a crucial dose of pragmatism and common sense into what appears a very technocratic process. The technical nature of our work must not obscure the essence of a technology appraisal—an independent group of people reviewing evidence, deliberating and then debating with stakeholders, and reaching a conclusion that can withstand intense scrutiny.

6. IN CONCLUSION: THE THREE RS OF NICE TECHNOLOGY APPRAISAL

The key points of my journey through NICE technology appraisals can be summed up in three words: relentless, robust and reasonable. All three conditions need to be fulfilled for a successful technology appraisal: relentless in understanding both what the evidence implies and the views and opinions of stakeholders, robust to withstand intense scrutiny, and reasonable so that people can appreciate the conclusions reached, even if they disagree with them.

I have a personal debt of gratitude to the health technology assessment and health economic community in the UK for helping steer the NICE technology appraisal programme on a sound course. I have two wishes for the future. First, the term ‘health economic model’ is a poor descriptor for the analytical framework that explores the NICE appraisal decision problem. It gives the impression that interrogation of ‘the model’ is a pursuit only for health economists. Quite often, the term puts people off, reducing the likelihood for involvement of an informed, non-expert audience. It would be very helpful for the health economic community to develop a more meaningful and engaging lay descriptor for ‘the model’.

Second, as health economists, think outside the box. Think about how much more impact you would have to be viewed as the problem solver, not as the health economist. The essence of a technology appraisal is unravelling a complex, multi-dimensional question. The technical pursuit is fuzzy: more like meteorology than health science. You have the tools required for the task: perhaps a re-branding is necessary!

A little learning is a dangerous thing;
drink deep, or taste not the Pierian spring:
there shallow draughts intoxicate the brain,
and drinking largely sobers us again.

Alexander Pope
Essay on Criticism Part ii