Coverage with evidence development in NHSScotland

Discussion Paper

11 November 2008
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1 Executive summary

Coverage with evidence development is the term given to a policy which allows conditional funding of a promising health intervention while more conclusive evidence is gathered to address uncertainty regarding its clinical or cost effectiveness.

Such policies have been explored in a number of countries, with varying degrees of success, and could be of potential benefit in Scotland, allowing patients earlier access to promising new treatments.

A steering committee was formed and organised a workshop to determine if Scotland might benefit from some form of coverage with evidence development on drugs and other health technologies. Many stakeholders were represented at the workshop.

The consensus of opinion at this time is that a policy of coverage with evidence could be feasible in NHSScotland provided that procedures are in place to ensure that the research which is commissioned resolves uncertainty and influences decision making. Reproducible criteria for when to use coverage with evidence would be needed and the timeliness and funding of the research would have to be ensured. Consideration would have to be given to safety and access issues. Mechanisms would have to be in place for updating advice based on the evidence gathered.

In conclusion, some form of coverage with evidence development could be feasible in NHSScotland if there is clarity from the outset on the form of the model, the framework is designed to best exploit existing data capture mechanisms in Scotland and there is genuine collaboration between all stakeholders.
2 Introduction

An increasingly common barrier to effective decision making in healthcare systems is the conclusion of health technology assessments (HTAs) and systematic reviews that ‘further research is needed’. A recent article in the BMJ states that ‘If there is not enough evidence for conventional systematic reviews and technology assessments to find benefit, the default decision is that a new technology, whether drug, device, or surgical procedure, is not “covered” and thus not paid for’ Kamerow, 2007\(^1\). However, it is argued that a middle ground between whether or not to fund the implementation of a new technology is needed in situations in which these have suggestive but not definitive evidence of benefit.

The phrase ‘coverage with evidence development’ was first used in the United States of America (USA) in 1995 as the name of a Medicare policy which provided cover for a procedure with no supportive randomised evidence of benefit. Medicare covered the cost of the procedure on the condition that patients consented to be randomised into a multi-centre trial sponsored by the National Institutes for Health comparing the procedure with optimal medical treatment.

Since then, a variety of ‘coverage with evidence development’ approaches have been adopted worldwide. Recognising that a model of coverage with evidence development could work in Scotland, Professor Andrew Briggs, Lindsay Chair in Health Policy and Economic Evaluation at the University of Glasgow formed a steering committee to take this forward. Members of the steering committee are listed in Appendix 1.

A workshop entitled ‘Coverage with Evidence Development’ was organised by NHS Quality Improvement Scotland (NHS QIS), the Scottish Medicines Consortium (SMC) and the University of Glasgow. The workshop posed the question ‘Can Scotland benefit from coverage with evidence development on drugs and other health technologies?’ Its aim was to consider coverage with evidence development as an approach to the use of promising technologies for which the evidence remains uncertain in the context of its practical application in Scotland. The workshop consisted of presentations from all perspectives with plenty of time for discussion. The agenda detailing all speakers and presentations is included as Appendix 2.

\(^1\) Kamerow D, Paying for promising but unproven technologies BMJ 2007; 335:965
Attendance at the workshop held at Delta House, Glasgow on Friday 19 September 2008 was by invitation only and included organisations and individuals with an interest in the topic. There were representatives from the NHSScotland Chief Executives’ Group, NHSScotland Chief Pharmacists’ Group, Scottish Government Health Directorates, CSO, ISD, University of Aberdeen (HSRU and HERU), MHRA, NICE, ABPI and ABHI.

This report summarises the discussions that took place at the workshop and raises issues for consideration if a strategy for coverage with evidence development is developed in NHSScotland. ‘Risk sharing’ and ‘market access’ schemes (see Glossary) were not considered.
Learning from others’ experiences

3.1 Ontario, Canada

The Ontario Health Technology Advisory Committee (OHTAC) was established in October 2003 to address the need to rationalise the diffusion and uptake of new technologies in a complex healthcare system. Its mandate is to provide evidence-based examination of proposed health technologies in the context of existing clinical practice and provide advice and recommendations to Ontario practitioners and the broader health care system and Ministry of Health and Long Term Care (MOHLTC). The committee receives secretariat and methodological support from the Medical Advisory Secretariat (MAS) of MOHLTC.

Cognisant that large, multi-year, funding decisions should be made with as much certainty of effectiveness and cost effectiveness as possible and that non-drug technologies in particular often lack robust evidence from pre-market studies to inform decision making, OHTAC developed a model of coverage with evidence development. In the absence of high quality evidence regarding the effectiveness of a health technology under review, and to prevent this becoming a barrier to creating a policy out of the existing evidence, OHTAC may recommend that a field evaluation be conducted. Field evaluations are designed to obtain evidence while meeting the care needs of informed, consenting patients, who may benefit.

MAS facilitates the start up of these studies, which are conducted in partnership with various clinical and academic research institutions around Ontario including the Program for the Assessment of Technologies in Health (PATH) at McMaster University, Toronto Health Economic and Technology Assessment Collaboration (THETA) at University of Toronto, Academic Health Science Centres or provincial disease-specific agencies.

Field evaluation provides an opportunity to address uncertainty arising from evidence-based analyses while still allowing access. In Ontario, field evaluations address many kinds of uncertainty related to policy decision making and the type of evaluation differs accordingly, including post-market pragmatic studies, registry studies, other observational studies and randomised controlled trials. Each field evaluation is subject to Research Ethics Board approvals and requires patient informed consent.

Current indications for such conditionally funded field evaluations include:

- large potential investment
• potentially disruptive effects
• quality controls desirable prior to unrestricted diffusion
• uncertainty regarding safety, low quality of evidence or generalisability.

Ten field evaluation studies have been completed since 2003, of which eight have informed policy in a highly significant way (see Table 1). A further 24 field evaluation studies are ongoing.

**Table 1 Completed field evaluations and associated policy development**

<table>
<thead>
<tr>
<th>Field evaluation</th>
<th>Result</th>
<th>Policy decision</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug eluting stents</td>
<td>Only effective in high risk</td>
<td>Funded 34–36% conversion from bare-metal (compared with 90% elsewhere)</td>
</tr>
<tr>
<td>Endovascular aortic aneurysm repair</td>
<td>No endoleak, CE dominant for high risk</td>
<td>Increase funding to improve access</td>
</tr>
<tr>
<td>Ontario Diabetes Economic Model</td>
<td>Downstream outcomes and costs based on multiple variables</td>
<td>Decisions re drugs and CE for DECs. Evaluation tool for diabetes strategy.</td>
</tr>
<tr>
<td>HPV as adjunctive screening tool</td>
<td>Poor compliance re colposcopy for ASCUS</td>
<td>Awaited – presumably delay until broader compliance issues dealt with</td>
</tr>
<tr>
<td>Safety evaluation of in-room air cleaners</td>
<td>Inappropriate use avoided. Safety and maintenance standards established.</td>
<td>Report and standards communicated to all hospitals</td>
</tr>
<tr>
<td>Poll of 1,000 women re acuity and treatment preference for stress urinary incontinence</td>
<td>Prevalence of high acuity 43,000. Invasive surgery an issue.</td>
<td>Conversion to mid-urethral slings doubled access in first 3 years. Exploring increased funding.</td>
</tr>
<tr>
<td>RCT of PET in staging lung cancer pre-surgical resection</td>
<td>PET reduces futile thoracotomy rates</td>
<td>Immediate open-ended access to PET funded for this indication</td>
</tr>
</tbody>
</table>

Several years after the inception of field evaluations, the Ontario Health System includes experts who continue to strongly support the process; the main advantage being that results are associated with immediate buy-in. However, lessons have been learned:

• these are complex processes involving multiple partners with long lead and execution timelines
• RCTs are difficult to defend for insured services and there must be
flexibility in design to address accrual and access issues

- public and professional pressure can be intense and engaging end-users (for example by publishing in respectable journals) is most likely to result in adherence

- conditionally funding field evaluations is not about delaying uptake or saving money, although the latter may be a spin off.

Sources Levin et al. 2007; http://www.health.gov.on.ca/english/providers/program/ohtac/ohtac_mn.html

3.2 NICE

The National Institute for Health and Clinical Excellence (NICE) was set up by the United Kingdom Department of Health in 1999 as the independent organisation responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health. NICE, in its technology appraisals, makes recommendations, based on a review of the clinical and economic evidence, on the use of new and existing medicines and treatments within the NHS.

The NHS in England is legally obliged to fund and resource medicines and treatments recommended by NICE, technology appraisals. However, as an alternative to a simple ‘yes’ or ‘no’ decision, NICE can recommend ‘Approval only in the context of research’ (OIR). In the case of promising interventions not yet supported by sufficiently robust evidence to justify an unqualified recommendation, NICE will:

- recommend that further research is carried out to see whether the potential promise of the intervention can be realised

- indicate in broad terms the questions this research should address

- advise clinicians that, in the meantime, they should only use the health technology as part of a well-designed programme of research to answer these questions.

Approximately one in 20 of NICE technology appraisal recommendations published between 1999–2007 were OIR. However, experience has shown that an OIR recommendation is perceived as essentially a ‘no’ and commissioners are not motivated to fund additional research. Funding for such research has been public, private or a mixture of both.

A recent review concludes that, when used sparingly, OIR has provided an important alternative to a ‘yes’ or ‘no’ decision on the appropriateness of
technologies. The main challenges encountered have been: developing clear and reproducible criteria for when to use coverage with evidence development; ensuring the timeliness and funding of the research studies they recommend; safety and access issues; and ensuring that mechanisms are in place for updating advice based on new evidence.

3.3 United States of America

Unlike the UK, the USA does not have a universal healthcare system and only access to emergency services regardless of ability to pay is mandated by law. The majority of citizens have some form of healthcare insurance either provided by their employer, paid for individually or provided by government programmes. There are several publicly-funded healthcare programmes which help provide for the elderly, disabled, children, veterans, and the poor.

Medicare is one such government funded programme which provides health insurance coverage to people who are aged 65 and over, or who meet other special criteria. Medicare has to pay for all “reasonable and necessary care”. In deciding whether a service or technology meets the reasonable and necessary rule, Medicare stipulates that there must be ‘adequate evidence to conclude that the item or service improves net health outcomes’. Costs are not considered, though evidence is reviewed ‘more carefully’ for high-cost interventions. Since 2006, Medicare will cover the costs of most drugs for FDA-approved indications (Medicare Part D).

In 2005, Medicare proposed a draft policy termed ‘coverage with evidence development’ to provide cover for a procedure with no supportive randomised evidence of benefit. The policy was formally introduced in 2006. However, the first time Medicare required that beneficiaries of a specific technology were enrolled into a clinical study for the technology to be covered, was in 1995, in the case of lung reduction surgery for patients with emphysema. The trial was sponsored by the National Institutes for Health. In recent years, the policy of coverage with evidence development has been applied on a number of cases in the USA.

Indeed, the recent decision that Medicare would continue to pay for CT scans in spite of the lack of evidence that these provide measurable medical benefit highlights a discrepancy between the USA and UK approach: a recommendation in the USA is likely to be to fund treatment with conditions attached (‘yes, but…’), while in the UK the recommendation would be not to fund treatment until more evidence is available (‘no, but…’).

2 http://www.cms.hhs.gov/CoverageGenInfo/03_CED.asp
3.4 Sweden

In Sweden, the Dental and Pharmaceutical Benefits Agency (TLV) make reimbursement decisions and establish the price of drugs. Most drugs are reimbursed without restrictions and the remaining drugs are either reimbursed with conditions or denied reimbursement. Between October 2002 and July 2008 17% of drugs were reimbursed with conditions.

There are several reasons for reimbursement with conditions:

- treatment is only cost-effective for a subgroup, in which case reimbursement would be limited
- to reduce uncertainty by demand and gain additional evidence on efficiency, effectiveness in real life or information about current treatment pattern
- to reduce uncertainty in the usage/uptake of the new treatment (risk of use in additional indications) – follow prescription pattern.

TLV undertakes two forms of economic analyses: ex ante (before the drug is on the market) and ex post (includes drugs on the market). The ex ante process results in a decision within 4 months for new pharmaceuticals. It needs modelling studies based on RCT results and observational studies (Swedish treatment pattern) and follow-up data from real life studies to confirm modelling results and to follow prescription patterns. A review of follow-up studies found that the research questions were not clear enough and that these studies were often of low quality. Consequently TLV will develop and publish guidelines for follow-up studies and need to learn to interpret data from observational studies.

The ex post evaluation process often takes between 12–15 months and reconsiders cost effectiveness of existing technology; old treatments that prove to be no longer cost effective are rapidly withdrawn to create space for new cost-effective technologies.
4 Stakeholders’ perspectives

4.1 Health technology assessors (including Scottish Medicines Consortium)

The Scottish Medicines Consortium (SMC) provides advice to NHS boards and their Area Drug and Therapeutics Committees (ADTCs) across Scotland about the status of all newly licensed medicines, all new formulations of existing medicines and new indications for established products (licensed from January 2002). SMC advises NHSScotland on the costs and benefits of new medicines after they have been licensed by the Medicines and Healthcare products Regulatory Agency/European Medicines Evaluation Agency. This advice is made available as soon as practical after the launch of the product involved.

For every medicine assessed, SMC issues one of three possible recommendations: accepted; accepted for restricted use; or not accepted for use within NHSScotland.

Table 2 presents the 10 most common reasons for a medicine failing to be accepted and considers whether or not a recommendation for coverage with evidence could be useful.

Table 2 Reasons for non-acceptance

<table>
<thead>
<tr>
<th>Reason medicine not accepted by SMC</th>
<th>Could a CED recommendation be useful?</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 Comparator does not reflect current practice – eg placebo studies or comparator off-label</td>
<td>Direct clinical data may be useful</td>
</tr>
<tr>
<td>2 Poor clinical data in economic evaluation</td>
<td>Additional short term clinical data not required but good long-term effectiveness</td>
</tr>
<tr>
<td>3 Over optimism in resources/QALY</td>
<td>Additional clinical data not required</td>
</tr>
<tr>
<td>4 No economic evaluation</td>
<td>Unsure if additional clinical data required – often clinical study is weak</td>
</tr>
<tr>
<td>5 No health outcome or QALY</td>
<td>Additional clinical data not required</td>
</tr>
<tr>
<td>6 Inadequate sensitivity analyses – given SMC timing uncertainty of evidence is expected so robust sensitivity analyses are vital</td>
<td>Direct clinical data may be useful if uncertainty is in clinical data (but usually problem is period beyond the study)</td>
</tr>
<tr>
<td>7 No transparency of economic case - issues is not complexity but one of clarity on the parameters and pathways</td>
<td>Additional clinical data not required</td>
</tr>
<tr>
<td>8 New medicine not cost effective</td>
<td>Additional clinical data not required</td>
</tr>
<tr>
<td>9 Data not generalisable to Scottish practice in terms of patient selection, resource use and costs and clinical pathways</td>
<td>Direct clinical data useful</td>
</tr>
<tr>
<td>10 Poor indirect comparison</td>
<td>Direct clinical data may be useful (but easier to improve methodology)</td>
</tr>
</tbody>
</table>
CED may address generalisability and comparator issues. However if such a recommendation was to be given by SMC, careful consideration would need to be given to the kind of study in terms of:

- setting – single centre, national or regional?
- patients – entry criteria, study size, sub-groups for specific clinical characteristics
- comparator treatments – if comparator not appropriate for a clinical trial is it ethical for a CED study?
- administered by whom? Strong interest group or any?
- unbiased so randomisation (or some other method of handling bias) needed
- outcomes – clinical effect, short- and long-term patient outcomes, short- and long-term resource use
- duration
- questions to be answered
- what registry /audit data already available?
- what variation in Scottish practice?
- robust, pre-specified protocol, data collection and analysis
- stopping criteria – especially if intervention works.

4.2 Industry

Industry would be supportive of the principle of CED, recognising that it is potentially very valuable in providing patients with earlier access to new medicines. Furthermore, CED could reduce potential bias against ‘promising but less well proven’ technologies, in particular where there is high medical need (e.g., orphan disease, high need area with poorly validated surrogate endpoint). Increased HTA assessment at launch puts increased focus on the unavoidable uncertainty at that point and CED could be part of the solution in specific cases.

Owing to complexities that would arise from such a policy, CED may be
limited to exceptional use, where other less complex market access schemes are not applicable. Examples of where it might be used follow

- at the launch of a promising new technology
  - where medical need is high
  - it would otherwise fail to be accepted by health technology assessment processes (eg SMC)
  - the main issue is data uncertainty which, if resolved, would inform cost effectiveness.

- when a product is available, but more information is required in specific sub-groups of patients, generally within the licence
  - a registry could address the need.

CED would not be appropriate when it is already agreed that the therapy in question is cost effective. For example, when multiple, cost-effective therapies are already available within a disease area, CED is not the appropriate methodology for comparing and deciding which is best; a post-marketing trial could be more appropriate.

Systematically collecting evidence while coverage is assumed could result in a potential waste of resources from implementing non-beneficial technology. However, without CED, implementation of effective treatment may be delayed, an equally ‘wasteful’ situation. A case by case evaluation is needed on this balance.

While pharmaceutical companies may fund those aspects of a CED study which are not based on usual practice ie the incremental costs, there are likely to be concerns raised. For example, will CED schemes reduce the incentives on industry to fully evaluate their products? This is thought to be unlikely as developments are generally at a global level. Will the potential for CED schemes incentivise SMC and others to limit ‘recommended’ decisions and ask for CEDs? Again, this is thought to be unlikely as these schemes are a significant burden, but it is certainly a concern to industry. Can the CED study meet its aims within the relatively small Scottish population base or is collaboration with other populations required? What are the pharmaceutical company’s and the NHS’ obligations to patients at the end of a scheme if the study results are not supportive of the initial claim?

To address these and other issues, development of any CED scheme must be highly collaborative across all relevant parties.
4.3 Scottish Government

The Scottish Government has a desire to ensure that patients get access to new effective medicines as soon as possible. In 2006–2007, £1.17 billion was spent on drugs in Scotland (10% of the total healthcare budget). While the centralised Swedish policy of disinvesting in older drugs that are no longer cost effective is not employed in Scotland, prescribing efficiency initiatives and formulary management are important in the management of resources.

A key strength of the SMC as perceived by the Scottish Government is that advice is issued soon after a medicine is launched. In cases where the cost per QALY is too high, the Scottish Government is seeking to provide a transparent method of ensuring that market access schemes are considered.

Given that SMC do not have an OIR recommendation, a policy of coverage with evidence development would provide a way forward in an environment of high uncertainty around clinical or cost effectiveness. This would equip decision makers with evaluation addressing uncertainty, clinicians would gain experience of the technology and patients would get access to medicines that appear beneficial but for which there are evidence gaps.

However, such a scheme would be complex and possibly expensive to set up. A clear set of intentions must be established if adopting this strategy to address the following concerns and ensure coverage with evidence development would answer the necessary questions:

- if the findings of the study are negative it would be more difficult to withdraw coverage than to refuse it initially
- patient safety may be compromised by exposure to something that may be ineffective
- patient consent and choice issues.

Coverage with evidence is worthy of further consideration within the wider context of spending on health, however policy space is required without pressure to rush a decision. The impact of new Pharmaceutical Price Regulation Scheme (PPRS) and market access framework should be taken into account.
4.4 Clinicians

Investigator-initiated trials have been successful in answering clinical questions in cases of unmet clinical need, where health boards have had concerns about cost and clinical effectiveness and industry wanted sales to recover development costs. An example is the Scottish Prospective Evaluation of Clinical Effectiveness of anti-TNF therapy in rheumatoid arthritis (SPECTRA), undertaken by clinicians in partnership with industry and health boards. The study of 178 patients confirmed the clinical effectiveness of anti-TNF therapy, proved that the principles of prescribing guidelines were acceptable to clinicians and adhered to closely and facilitated access to effective therapy in advance of SMC/NICE guidance.

This example illustrates how effective such collaboration can be, however large clinical trials require multi-centre collaboration and there is a perception among clinicians that bureaucratic barriers to involvement in clinical research are ever increasing. Furthermore, biologic drug costs are prohibitive for most research funders.

A clinician posed the question of whether the SMC has a role in supporting and facilitating large-scale investigator-initiated studies, perhaps by approving the use of drugs in patients who participate in such research and by delaying the final decision of the unrestricted use of the drug until the study results are available.

It was suggested that industry could be asked to engage with SMC prior to submission and that SMC could interact with the clinical community to identify areas of uncertainty in evidence and develop protocols for evidence coverage research that will reduce this uncertainty.

4.5 Scottish Health Technologies Group

The Scottish Health Technologies Group (SHTG) was established in 2007 in response to the need for more support and assistance for NHS boards in planning the introduction of new technologies recommended by NICE and others. It is an advisory group whose role is to provide evidence to support planning and decision making using members’ networks to communicate information. SHTG is a cross-cutting group of people from NHS boards and membership includes chief executives, NHS QIS, Scottish Government Health Department, health economists and a lay representative.

The remit of SHTG includes horizon scanning, assessment and facilitating implementation and dissemination of evidence-based recommendations. As such, improvement in outcomes research may be useful for SHTG.
4.6 Information Services Division (ISD)

In considering coverage with evidence development, the question arises of whether Scotland has or can get the information systems necessary to support such a complex undertaking.

Information Services Division (ISD), part of NHS National Services Scotland, is Scotland's national organisation for health information, statistics and information technology (IT) services. Scotland has some of the best health service data in the world, combining high quality data, consistency, national coverage and the ability to link data to allow patient based analysis and follow up.

ISD is currently modernising its IT, with the creation of the Scottish Health Information Service (SHIS), a strategy for delivering a Secondary Uses Service (SUS) for the NHS in Scotland. One of the objectives of SHIS is to deliver an NHSScotland data Warehouse which holds national and local data, is accessible to authorised customers, and is subject to appropriate information governance.

Over 28 million records for 4.7 million people are stored and, by recording the Community Health Index (CHI) number unique to each patient, the system has the advantage of being able to link together data for patients. Ways in which the stored information is used include clinical outcome indicators, resource allocation, survival analysis, changing patterns of care and case/control/COHORT studies.

The eHealth Strategy launched in June 2008 aims to change the way in which information and related technology are used within NHSScotland in order to improve the quality of patient care. In parallel, funding has been awarded from Wellcome to build an electronic patient record and research infrastructure. Once the e-Health research infrastructure is in place, it may not be dissimilar to that needed to facilitate coverage with evidence development. A challenge will be to have local and national datasets which interface.

ISD has already demonstrated its existing ability to retrieve information for follow up studies (e.g. WOSCOPS long-term follow up). Lots of audit information is already being collected, but not being used – is the wrong information being collected or is the information not being used effectively?
5 Considerations for NHSScotland

5.1 Risks and benefits

Table 3 summarises the perceived risks and benefits associated with developing a policy of coverage with evidence within NHSScotland.

Table 3 Risks and benefits of coverage with evidence development in Scotland

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Risks</th>
</tr>
</thead>
<tbody>
<tr>
<td>Earlier patient access to effective treatment</td>
<td>Financial – what will be funded and by whom?</td>
</tr>
<tr>
<td>Acting to reduce uncertainty and gaining better evidence</td>
<td>Setting up the wrong type of study that doesn’t answer the question – Scotland is a small country and studies may require a large number of participants</td>
</tr>
<tr>
<td>Spending money wisely – better value healthcare</td>
<td>Re-inventing the wheel – similar schemes have already been developed internationally</td>
</tr>
<tr>
<td>Scotland is a small country so there are more direct links to the clinical community and political system</td>
<td>How to prioritise areas for study?</td>
</tr>
<tr>
<td>Expectations from academics, the public, politicians and policy makers</td>
<td></td>
</tr>
<tr>
<td>Capacity and engagement</td>
<td></td>
</tr>
<tr>
<td>Intellectual rights</td>
<td></td>
</tr>
</tbody>
</table>

5.2 Ethical considerations

Any study initiated as part of coverage with evidence development would be subject to approval by the relevant ethics boards. Inequity of access, incentives and adjustment of funding depending on the outcome of these studies are among the ethical issues that will have to be considered.

5.3 A model for Scotland

Coverage with evidence development could work in NHSScotland if the terminology and the questions are clearly defined from the outset and there is collaboration between all stakeholders.

It should be understood that coverage with evidence development is not about getting the cost right; it is not a market access scheme. Instead, coverage with evidence development should be considered as a means of helping policy makers take decisions in the face of uncertainty around clinical and cost effectiveness, generalisability, safety and outcomes.

Reproducible criteria should define what evidence is needed to address particular uncertainties but the system should have a flexible approach to
funding, responsibilities and impact. Health technologies which are found to be no longer cost effective should be withdrawn.

Whatever model is adopted, it is of paramount importance that procedures are in place to ensure that the research that is commissioned resolves uncertainty and influences decision making.

5.4 Next steps

An evolutionary rather than a 'big bang' approach to implementing some form of coverage with evidence development in Scotland is suggested, perhaps beginning with drug therapies where uncertainty remains identified during SMC processes. A pilot study could show what can be achieved and may help market the concept to all concerned, including patients and the public.

A two stage process to achieve this is proposed. Over the next 6 months, the working group lead by the University of Glasgow will produce proposals to develop an appropriate study design and economic model to facilitate evidence collection and evaluation for two topics, one of which should be non-pharmaceutical. The intention is to submit these proposals to the Chief Scientist Office for consideration for initial funding. On completion of this stage, further funding and appropriate collaboration would then be sought to undertake the data collection and populate the economic model thereby addressing the existing uncertainty regarding clinical and/or cost effectiveness.

Development of an international registry database of coverage with evidence projects, similar to that already in existence for HTAs, would minimise duplication of effort between countries.

Any steps towards coverage with evidence development should be taken in full partnership with all relevant stakeholders and link with other initiatives and policy drivers eg Health Efficiency Access and Treatment (HEAT) targets and any communication regarding developments in this area should be made easily accessible ie will be free from jargon and will be readily understood by clinicians, managers, policy makers and the public alike.
6 Acknowledgements

NHS QIS is grateful to colleagues from SMC, Glasgow University and Scottish Government for their input to the workshop and this report; the presenters at the workshop; Marina Logan and Doreen Pedlar for administrative support; and Susan Downie for preparation of this manuscript.
## Appendices

### Appendix 1 Members of the steering committee

<table>
<thead>
<tr>
<th>Name</th>
<th>Job title</th>
<th>Organisation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Prof Andrew Briggs</td>
<td>Lindsay Chair in Health Policy and Economic Evaluation</td>
<td>Faculty of Medicine, University of Glasgow</td>
</tr>
<tr>
<td>Dr Sara Davies</td>
<td>Public Health Consultant</td>
<td>Scottish Government Health Department</td>
</tr>
<tr>
<td>Dr Harpreet Kohli</td>
<td>Medical Advisor</td>
<td>NHS Quality Improvement Scotland</td>
</tr>
<tr>
<td>Dr Ken Paterson</td>
<td>Chairman</td>
<td>Scottish Medicines Consortium</td>
</tr>
<tr>
<td>Dr Karen Ritchie</td>
<td>Lead Health Service Researcher</td>
<td>NHS Quality Improvement Scotland</td>
</tr>
</tbody>
</table>
Appendix 2 Workshop agenda

Coverage with Evidence Development Workshop

Organised by NHS Quality Improvement Scotland, Scottish Medicines Consortium and the University of Glasgow

NHS QIS, Delta House 6th Floor,
50 West Nile Street, Glasgow, G1 2NP
Friday 19 September 2008, 10.00 – 15.30

09:30 COFFEE

09:50 Session 1 - Experience of Coverage with Evidence Development
Chaired by Professor Andrew Briggs

10:00 Experience of Ontario
Dr Leslie Levin – Head Medical Advisory Secretariat, Ontario Ministry of Health & Long Term Care

10:20 ‘Only in Research’ - a NICE example
Dr Fergus Macbeth - Director National Collaborating Centre for Cancer, Cardiff & Consultant Oncologist

10:30 Experience of USA
Dr Kalipso Chalkidou - Director, Policy Consulting, NICE

10:40 Experience of Sweden
Dr Ulf Persson - Research Director, IHE Sweden

10:50 Discussion

11:30 COFFEE

11:45 Session 2 – Stakeholders Perspectives on Coverage with Evidence Development
Chaired by Dr Ken Paterson

11:45 Evidence Developers
Ms Joyce Craig - Lead Health Economist, NHS QIS

11:55 Pharmaceutical Industry
Dr Frances MacDonald, ABPI

12:05 Scottish Government Health Department
Professor Bill Scott – Chief Pharmaceutical Officer, Scottish Government

12:15 Clinician
Dr Duncan Porter, Consultant Rheumatologist, NHS Greater Glasgow & Clyde

12:25 Scottish Health Technologies Group
Dr Sara Davies – Public Health Consultant, Scottish Government

12:35 Discussion

13:00 LUNCH
13:45 Session 3 - ISD Perspective and Group Work
  Chaired by Dr Harpreet Kohli

13:45 ISD: Current and Future Data Infrastructure and Analysis
  Ms Mary Sweetland, Deputy Director ISD)

14:00 Discussion

14:15 Group work session

15:15 Closing remarks – Professor Andrew Briggs

15:30 COFFEE AND FINISH
**Glossary**

<table>
<thead>
<tr>
<th>ABHI</th>
<th>Association of British Healthcare Industries</th>
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<tr>
<td>ABPI</td>
<td>Association of the British Pharmaceutical Industry</td>
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<tr>
<td>Academic Health Science Centres</td>
<td>A partnership between one or more universities and healthcare providers focusing on world-class research, clinical services, education and training. Sunnybrook Health Sciences Centre located in Toronto is an example.</td>
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<tr>
<td>ADTC</td>
<td>Area Drugs and Therapeutic Committee</td>
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<tr>
<td>CED</td>
<td>Coverage with evidence development</td>
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<tr>
<td>Clinical effectiveness</td>
<td>The evaluation of the balance between benefits and risks in a standard clinical setting using outcomes of importance to the patient.</td>
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<td>Community Health Index (CHI)</td>
<td>The CHI number is a unique patient identifier that is allocated to every patient registered with a GP in Scotland. It is entered onto a database that underpins a wide range of patient-care processes in Scotland. There are strict controls on access to patient identifiable details.</td>
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<tr>
<td>Cost effectiveness</td>
<td>Used in its broadest form, this term encompasses all forms of economic analysis.</td>
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<tr>
<td>CSO</td>
<td>Chief Scientist's Office</td>
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<td>FDA</td>
<td>Food and Drug Administration</td>
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<td>Health technology assessment (HTA)</td>
<td>A multidisciplinary field of policy analysis, which studies the medical, social, ethical and economic implications of development, diffusion and use of the technology.</td>
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<tr>
<td>HERU</td>
<td>Health Economics Research Unit</td>
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<td>HSRU</td>
<td>Health Services Research Unit</td>
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<tr>
<td>ISD</td>
<td>Information Services Division</td>
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<td>IT</td>
<td>Information technology</td>
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<td>Market access scheme</td>
<td>See ‘Risk sharing scheme’.</td>
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<tr>
<td>Term</td>
<td>Definition</td>
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<tr>
<td>Medicare</td>
<td>USA insurance programme which pays for healthcare for elderly people and some disabled patients.</td>
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<td>MHRA</td>
<td>Medicines and Healthcare products Regulatory Agency</td>
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<td>MOHLTC</td>
<td>Ministry of Health and Long Term Care</td>
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<td>NHS Quality Improvement Scotland (NHS QIS)</td>
<td>NHS QIS has been established to lead in improving the quality of care and treatment delivered by NHSScotland. To do this, it sets standards and monitors performance, and provides NHSScotland with advice, guidance and support on effective clinical practice and service improvements.</td>
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<tr>
<td>NICE</td>
<td>National Institute for Health and Clinical Excellence</td>
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<td>NSD</td>
<td>National Services Division</td>
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<td>OHTAC</td>
<td>Ontario Health Technology Advisory Committee</td>
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<tr>
<td>OIR</td>
<td>Only In the context of Research</td>
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<td>Risk sharing scheme</td>
<td>An agreement between the NHS and the manufacturers in which the manufacturers have agreed that their product will deliver certain outcomes and if these outcomes are not delivered the price that the NHS pays will fall, hence the risk sharing. These schemes are designed to allow greater patient access to new medicines and speed their uptake.</td>
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<td>Scottish Executive</td>
<td>Formerly the name of the Scottish Government. See Scottish Government.</td>
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<tr>
<td>Scottish Government</td>
<td>The devolved government for Scotland, with responsibilities including health policy and the administration of NHSScotland. Until September 2007, the devolved government was named the Scottish Executive.</td>
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<td>SEHD</td>
<td>Scottish Executive Health Department. The former name of the Scottish Government Health Directorates. See Scottish Government.</td>
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<td>SHIS</td>
<td>Scottish Health Information Service</td>
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<td>SHTG</td>
<td>Scottish Health Technologies Group</td>
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<td>SMC</td>
<td>Scottish Medicines Consortium</td>
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<td>Systematic review</td>
<td>Synthesis of original studies such as controlled trials and random controlled trials.</td>
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<tr>
<td>UK</td>
<td>United Kingdom</td>
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<tr>
<td>USA</td>
<td>United States of America</td>
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