The evolution of HTA in Scotland

Karen Facey
Evidence Based Health Policy Consultant
k.facey@btinternet.com

CADTH 25th Anniversary Lecture – June 2014
Evolution of HTA in Scotland

• Scottish context
• The Health Technology Assessment (HTA) model
• Evolving forms of HTA in Scotland
• Evolving processes
• Encouraging Innovation
• Population = 5.2 million

• Challenges
  o financial austerity
  o ageing population
  o expensive new treatments and devices
  o geography

• Taxation based health system, £11billion health budget

• No co-payments for prescription medicines
• 14 health boards - payers/providers providing primary, community, acute care with formularies set by each Area Drugs and Therapeutics Committee

• NHS Forth Valley
• 300,000 people, £450mi
• ~12% spent on prescribing in primary care

• Drug prices set by UK

• Devices negotiated through procurement, national, health board and hospital
The evolution of Scottish science

Maxwell's Equations

1. \( \frac{\nabla \times \mathbf{B}}{\mu} = j + \frac{\partial \varepsilon}{\partial t} (\mathbf{H} \times \mathbf{B}) \)

2. \( \nabla \cdot \mathbf{E} = 0 \)

3. \( \nabla \cdot \mathbf{D} = \varepsilon_0 \rho \)

4. \( \nabla \times \mathbf{E} = \frac{\mathbf{J}}{\varepsilon_0} \)

where \( \mathbf{D} = \varepsilon_0 \mathbf{E} \) and \( \mathbf{B} = \mu_0 \mathbf{H} \)

\( \mathbf{E} \) is the electric field, \( \mathbf{B} \) is the magnetic field.

\( \mathbf{D} \) is the electric displacement, \( \mathbf{H} \) is the magnetic field.

\( j \) is the electric current, \( \varepsilon_0 \) is the charge density.

\( \mu_0 \) and \( \varepsilon_0 \) are constants.

Brownian movement

4. The constitutional formula of succinic acid is
Scottish philosophy

ESSAY ON THE HISTORY OF CIVIL SOCIETY.

By ADAM FERGUSON, LL.D.
Professor of Moral Philosophy in the University of EDINBURGH.

THE FIFTH EDITION.

LONDON:
Printed for T. Cadell, in the Strand; and W. Creech, and J. Bilt, Edinburgh.
M DCC LXXII.

Though the philosopher may live remote from business, the genius of philosophy, if carefully cultivated by several, must gradually diffuse itself throughout the whole society, and bestow a similar correctness on every art and calling.

David Hume
(1711 - 1776)
Scottish technology innovation
Scottish health technologies
Scottish INTERCOLLEGIATE Guideline Network
A National Health Board

- Powers and duties specified in legal statute, **public standards** imposed explicitly upon it

- Influence to lie in persuasion through scientific rigour & transparency of procedures and advice

- Openness, accountability, legitimacy

- Board membership reflects the role of HTA as the bridge between science, professional judgement, public opinion, and the needs of policy makers
Health Technology Board for Scotland
(Into force 1 April 2000)

The Board (HTBS) will exercise the following functions of Scottish Ministers:

the evaluation and provision of advice to the National Health Service in Scotland (NHSScotland) on the clinical and cost effectiveness of new and existing health technologies, including drugs
Health Technologies

Any intervention that may be used to promote health, to prevent, diagnose or treat disease or for rehabilitation or long-term care.

The term encompasses drugs, devices, clinical procedures and health care settings
Health Technology Assessment (INAHTA, 2000)

Health Technology Assessment (HTA) is a multi-disciplinary field of policy analysis, which studies the medical, social, ethical and economic implications of development, diffusion and use of health technology.
HTA

- Clinical effectiveness
- Cost effectiveness and budget impact
- Patients’ perspectives
- Organisational issues
HTA: Evidence based decision-making

Assessment of primary investigations, submissions, literature searches

Needs and preferences of patients and carers

Health professionals’ opinions

Appraisal recommendation/advice

Decision
Delivery of a new service
Reimbursement/formulary listing
Scottish HTAs

1. Comments on NICE Multiple Technology Appraisals
2. Full HTAs on complex issues involving a range of technologies
3. Assessment of all new medicines and new indications via the Scottish Medicines Consortium
4. Rapid assessment of non medicines technologies
Full HTAs
Topics for HTBS Assessment

• Autumn 2000: 66 suggestions ⇒ 3 initial topics
  – Organisation of services for diabetic retinopathy screening
  – Interventions to prevent relapse in alcohol dependency
  – PET imaging in cancer management
HTBS Process for HTA

- Advice
- Review
- Assessment announced
- Evidence gathering
- Filtration
- Other form of evaluation
- Topic Specific Group
- Topic proposal
- Board
- Clarification meetings
- Board
- Open meeting
- Topic Specific Group
- Advice
Relationship with stakeholders

• Open and positive approach
• Early dialogue
• Manufacturers
  – Consensus on data requirements
  – “Clarification” meetings
  – Co-operative approach to data production
• Patients
  – On Expert group: scoping to dissemination
  – Secondary and primary qualitative research
  – Key section of consultation workshop
Health Technology Assessment Report 1:
Organisation of services for diabetic retinopathy screening
Health Technology Board for Scotland

Health Technology Assessment Advice 1:
Organisation of services for diabetic retinopathy screening

Summary - The Health Technology Board for Scotland (HTBS) advises that a national diabetic retinopathy screening programme for Scotland should be established to detect referable (sight-threatening) retinopathy.

- Be quality assured and organised efficiently, taking account of the needs and preferences of people with diabetes and using effective technology.
- Be organised in collaboration with the National Services Division and Clinical Standards Board for Scotland, and fully integrated with other clinical management systems for the care of people with diabetes as outlined in the Scottish Diabetes Framework.
- Be recognised in each local Health Plan.
- Require NHS Boards to identify a named individual who is empowered to take local responsibility for the programme.
- Screen people with diabetes annually using a three-stage process based on non-mydriatic digital cameras, with the use of mydriasis (dilation of pupils with eye drops) and slit lamps, where necessary.
- Use appropriately trained, accredited, and competent professionals for screening and grading with the Scottish Diabetic Retinopathy Grading System recommended by HTBS.
- Enhance existing schemes to achieve the approved quality assured standards.
- Ensure screening is accessible to all people with diabetes, whether they receive community and/or hospital-based diabetic care. Local implementation may include services in diabetes centres, primary healthcare facilities, mobile vans or community optometrists.
- Inform people with diabetes about diabetic retinopathy (including the screening process, its limitations, and possible outcomes). Staff should be supportive and sensitive to each individual's concerns during the screening process.
- Be evaluated as the screening programme is rolled out and reviewed in the light of further research to enable optimal service provision.

April 2002
Status of HTBS Advice

“NHSScotland should take account of advice and evidence from HTBS and ensure that recommended drugs and treatments are made available to meet clinical need.”

Health boards not following HTBS Advice will need to explain their position, which should be a clinical view about whether a treatment is appropriate, not a board going against HTBS Advice.
Scottish Medicines Consortium (SMC)

- Postcode prescribing
- Need for consistency across Scotland
- Avoid duplication of work across Area Drugs and Therapeutic Committees (ADTCs) who create health board formularies
SMC established in 2001 to provide advice for Scotland on status (*clinical and cost effectiveness + patient issues*) of all new medicines, new formulations & new indications as soon as practicable after market launch.

Uses structured evidence submissions from manufacturers and patient organisation.

If a medicine has not been assessed by SMC it should not be used routinely in Scotland.

www.scottishmedicines.org.uk
SMC New Drugs Committee - NDC

- Membership: approximately 20 professionals skilled in *critical appraisal* (assessment)

- Pharmacy and economic assessors from health authorities and universities

- Hospital and primary care physicians

- Public Health Consultants

- Nurses
SMC NDC Process for Full Submissions

• *(No scientific advice, no scoping)*

• Review industry submission and published literature (particularly regulatory report)

• Complete review forms for clinical issues, indirect comparisons, cost effectiveness

• Clarifications with manufacturer throughout the process

• Produce Draft Advice Document
Templates/Guidance for Submission

Working with SMC - A Guide for Manufacturers


Submission Requirements for Full and Abbreviated Submissions

Guidance on Medicines outwith SMC Remit
Guidance regarding medicines outwith SMC remit.

Product Assessment Flowchart

Templates/Guidance Required for a Full Submission

New Product Assessment Form (NPAG) Template for Full Submissions

Budget Impact Template

Guidance to Manufacturers

Guidance for completion of New Product Assessment Form for Full Submissions

Summary of Information to Patients
Template for submission of evidence which may be completed by industry as a supplement for patient and public interest groups making a submission.

Economic Question and Answer Document

Economic Checklist Template

Clinical Checklist Template

Indirect Comparison Checklist Template

Costing – Frequently Asked Questions

Template/Guidance for completion of a Patient Access Scheme

Documentation and Guidance Notes Required for completion of a Patient Access Scheme

Template/Guidance Required for an Abbreviated Submission

Abbreviated Submission Form and Guidance Notes
Template and Guidance to Manufacturers for completion of Abbreviated Submission Form

SMC Policy/Process Statements

Within this section you will find SMC policy statements relating to modifiers, biosimilar medicines, Process for a Resubmission/IRP, Policy for Meetings with Manufacturers and other position statements which you find helpful.

Policy/Process Statements

Further details on how to make a submission
Scottish engagement with patients

- Since 2001 national policy for patient focus and public involvement
- SMC Patient & Public Involvement Group (PAPIG)
  - Development of submission form for patient groups
  - Public Involvement Officer(s) support evidence submissions from patient groups and provides feedback
- Plain English explanations of SMC process
SMC Collaboration with Industry

• User Group Forum chaired by ABPI member
• 11 individual industry members, 1 other from ABPI
• 2 NDC members and SMC secretariat
• Dialogue and joint work to improve processes
  ➢ Evidence submission form
  ➢ Summary Information for Patients
  ➢ Communication
  ➢ Workshops on common pitfalls
  ➢ Horizon scanning database
  ➢ Patient Access (Managed Entry) Schemes
SMC - The Appraisal Committee

- Consortium of the 14 health authorities in Scotland - Health authority managers; clinicians, pharmaceutical advisers, economists from each Area Drug and Therapeutics Committee
- Three public members
- Three members from Industry Association ABPI
- 38 members with equal voting rights and process for management of interests

- Pool of clinical experts
SMC Appraisal Process

• Review company submission, NDC Draft Advice Document and comments on it from industry, clinical expert advice, patient evidence

• Cost/QALY ~ £20,000-£30,000???
• Deliberative decision not a strict threshold
• Depends on
  ➢ unmet need, disease severity, bridge to therapy..
  ➢ substantial improvement.…
  ➢ strength of evidence, uncertainty…..
SMC advice

• SMC advice issued to health boards one month before publication on web site
  ➢ Accepted for general use
  ➢ Accepted for restricted use
  ➢ Not recommended for use

• NHSScotland should take account of advice and evidence from the SMC and ensure that recommended medicines are made available to meet clinical need
SMC and clinicians in the health service

- 14 health board Area Drugs and Therapeutics Committees (ADTCs) have local formularies
- Expected to reach decision on SMC Advice in 90 days, and publish within next 14 days
- If clinicians wish to prescribe a medicine not recommended by SMC they can submit an Individual Patient Treatment Request (IPTR)
SMC and planning in the health service

• Since 2005, SMC issue annual confidential “Forward Look” report
• Estimating potential budget impact at 1 and 5 years after launch for drugs identified in horizon scanning
• Review of actual costs vs budget impact estimates underway
Deadly cost of delays over drug approval

‘Lives being put at risk by deeply flawed system’

HELEN PUTTICK
HEALTH CORRESPONDENT

THE system for approving new drugs for use on the NHS in Scotland is bureaucratic, deeply flawed and could cost lives, according to a Labour MSP.

Dr Richard Simpson, a former doctor, said his constituents are confused by the process and it had created a postcode lottery.

He was speaking as an investigation into access to expensive, potentially life-saving medicines was launched.

“The consistency we have across Scotland is if you have your heart attack in the west, you’ll get your drug; if you’re in the east, you won’t. For my constituents it is confusing.

“We have a system that is deeply flawed at that level.”

Dr Simpson gave the example of the drug Briliq. It was given general approval by the SMC in April last year but is still not available to patients in Scotland.

“The evidence given to the inquiry is that patients in England have been able to access it directly from the manufacturer.”

The approval process for newly licensed medicines is under scrutiny at Holyrood’s Health Committee. During an evidence session, MSPs questioned experts from the SMC and several health boards.

Dr Simpson’s concerns were echoed by Andy Powrie-Smith, director of the Association of the British Pharmaceutical Industry Scotland.

He said the UK is 11th in Europe in terms of access...
Scottish Parliament
Health and Sport Committee

- Understand SMC uses robust procedures and has international standing, but why aren’t products it recommends available in the 14 health boards across Scotland?

- Letter from Chief Medical Officer indicated health board formulary decisions should be transparent by April 2012, but they aren’t.

- Examine general issues regarding the approval process for newly licensed medicines, with submissions from all stakeholders and several round-table discussions, then wide consultation.

Rare Diseases Drugs Fund
For treatments for very rare diseases that are not recommended by SMC where there was an IPTR
£20mi for 2013/2014
Scottish Government response
9 October 2013

Themes raised by stakeholders

• Transparency to aid understanding
• Equity of approach across Scotland
• Person-centred approach
• Timeliness
• Sustainability
Accepted recommendations re SMC process

- SMC to meet in public (from May 2014)
- Pause at any point in process
- Manufacturer to be allowed to give evidence at SMC meeting
- Meetings with manufacturers prior to submissions
- May commission assessment of a medicine for which there have been no submissions

- (£1mi extra for SMC)
Recommendations re the public

• *Cautiously explore establishment of a Citizens Jury*
  • *Explore innovative approaches to increasing public awareness of its role in ensuring timely access to clinically and cost-effective medicines*

• Expand role of SMC’s Patient and Public Involvement Group to engage proactively with patient organisations and the public on SMC’s work
  - Assist in development of Plain English Guides to describe work of the SMC and ADTCs
  - Input to new SMC Annual Report to clearly describe their work to the public and patients
Transparency of ADTCs

- Local response to SMC Advice published in 30 days (max 90 days)
- Two public partnership members on ADTC
- Local Advice easily accessed on web
- Audit of compliance
- National level implementation for some cases e.g. novel, first in class medicines where there is uncertainty of place in therapy
- ??? Smaller number of ADTCs or national body could be considered in future
Altered process for IPTRs

- Concerns that there will still be barriers to accessing new medicines, particularly via IPTRs
- Procedure for accessing drugs in exceptional prescribing circumstances, when all other treatments have been exhausted, should be clearly linked to clinical opinion
- ADTC IPTR systems to be replaced by national, clinically led Peer Approved Clinical System (PACS)
- Centralised patient support from PAPIG
Research in Scotland

Explore diminution in number of phase III trials in Scotland

Work with the pharmaceutical industry to improve the efficiency and effectiveness of NHS clinical research

Create a Scottish Clinical Trials Register to encourage clinician participation
Call for Value Based Assessment

- End of life – medicine used to treat a condition at a stage that usually leads to death within 3 years with currently available treatments
- “Orphan” – Prevalence <2,500/5 million (not necessarily designated orphan)
- “Ultra-orphan” – Prevalence <100/5 million (Carbaglu (hyperammonaemia due to N-acetylglutamate synthase deficiency - Cost/year £41,000 for baby, £245,000 for adult)

60 “not recommended” in this category over 3 years from 2011-2013
Judging options for change

- Robust, transparent, consistent, equitable
- Scientifically rigorous
- Feasible to introduce quickly, feeding into current decision making processes
- Process that will not delay patient access
- Should not destabilise assessment of other new medicines
- Limit scope for gaming and legal challenge
SMC End of Life/Orphans

• QALY weighting
  ➢ NICE uses 1.67
  ➢ Would need weighting of 3 or 4 for those not recommended by SMC in last 2 years

• Stronger Patient And Clinician Engagement (PACE)
  ➢ If NDC propose “not recommended”, manufacturer can invoke PACE meeting
  ➢ Discuss value not captured in QALY (severity of condition, unmet need, impact of carers)
  ➢ Perceived benefits compared with best practice
  ➢ Place in pathway (prescribing advice and continuation)
NICE Value Based Pricing Assessment Consultation

- Burden of illness
  - Proportional shortfall in QALY per Burden of Illness unit as a consequence of having a condition

- Wider societal benefit
  - Not based on producing and consuming resources
  - Absolute shortfall in being able to contribute to society over a lifetime, measured in QALYs

- (Therapeutic improvement and innovation)
SMC Ultra orphans

• Cost consequence, multi-criteria decision analysis, adapted SMC modifiers

• NICE interim process for Highly Specialised Technologies (HST)

  • Framework of explicit criteria, without weighting or scoring (ala HST process) but retaining cost effectiveness

• After 1 year, process for review to be agreed
Potential impact for Scotland

In 2014/15 for new medicines due to receive regulatory approval in these categories

- 1,500 patients
- £70 million
- Plus priority resubmissions in these categories
  - Implications for other medicines?
  - Need Scottish model of value
Statement of Intent for Innovation in Health

Vision
Scotland is a world leading centre for innovation in health through partnership working between Government, NHSScotland, Industry and the research community.
Health and Wealth

• Aims to double economic contribution of Life Sciences to Scottish economy by 2020

• NHS should become a pivotal stimulator of innovative products

• Strong partnership with industry to ensure NHS encourages development, marketing and adoption of medicines (and health technologies) that are better matched to its needs and which are evidence based
Health Innovation Portal

Innovation portal - guide for innovators

About the HIPP Scotland Portal

The portal is an online platform has been designed as a single-point resource to develop stronger partnerships with industry. It provides potential suppliers with information, guidance and support on how to develop ideas and innovations into products and technologies that may be of use to NHS Scotland or to further develop innovative products.

This is the first version of the portal so we would really welcome feedback to help us improve the process.