Health Technology Assessment (HTA)

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Evolving drug development

- Healthcare today – Scotland and beyond
- Health Technology Assessment (HTA)
- Clinical effectiveness
- Cost effectiveness
- Patient engagement
- Implications for statisticians in drug development

- Population = 5.2 million
- Challenges – financial austerity, ageing population, expensive new treatments/devices
- Taxation based health system, no co-payments, £11billion
- 14 health boards - payers/providers providing primary, community, acute care
- Drug prices set by UK Government in collaboration with Industry
- ~12% spent on prescribing in primary care

Health Technology Assessment (www.eunethta.net)

HTA is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to use of a health technology* in a systematic, transparent, unbiased, robust manner

It aims to inform policy at national, regional or hospital level

*screening , vaccines, diagnostics, medicines, devices, education, rehabilitation….

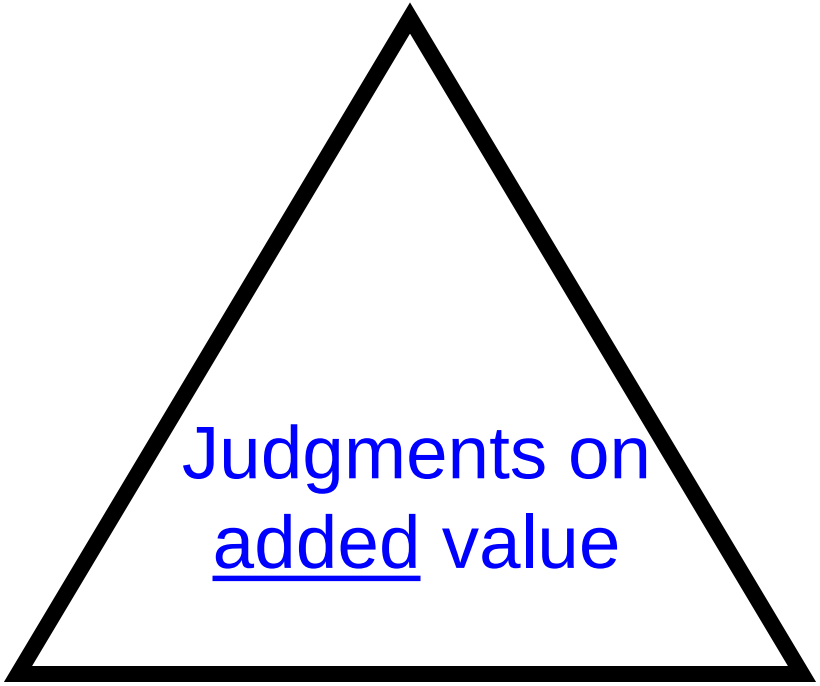
European network for HTA (EUnetHTA)

HTA Core Model

1. Health problem
2. Technical description of technology
3. Safety
4. Clinical Effectiveness
5. Costs and economic evaluation
6. Ethical analysis
7. Organisational aspects
8. Social aspects
9. Legal aspects

HTA: Evidence based decision-making

Scientific publications and submissions
(including evidence from a range of sources)



Needs and preferences of patients & carers

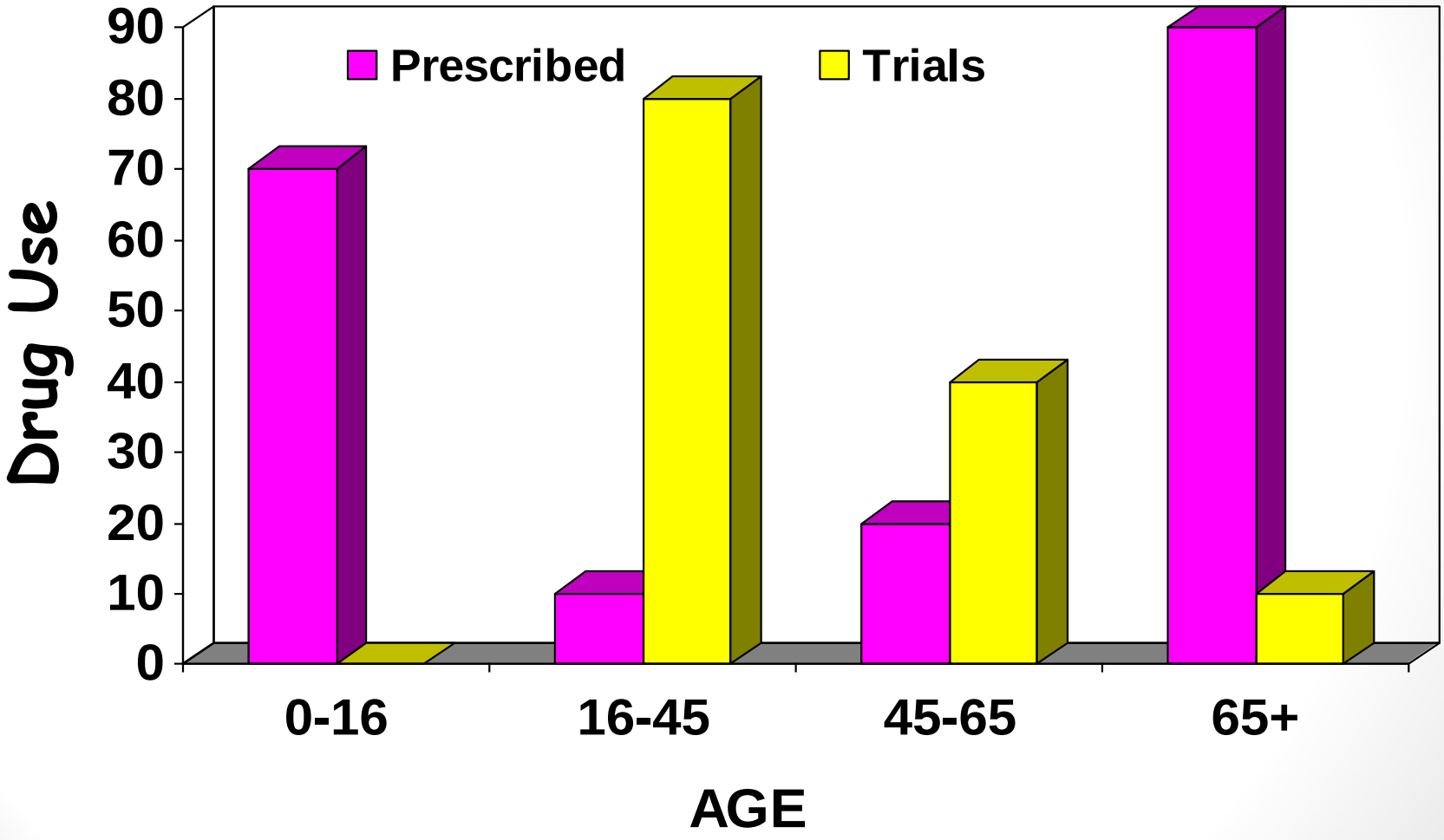
Health professionals' experience

Clinical Effectiveness
(Relative effectiveness/Comparative Effectiveness)

- Evaluation of benefit/risk in a standard clinical setting
  - no upper age restrictions, concurrent medical conditions, polypharmacy
  - compared to best standard care (BSC)
- Measuring outcomes that demonstrate added clinical value
  - long-term – survival, delayed progression
  - QOL (being able to dress, walk, work…)

Evidence : Practice Paradox

(Tom McDonald, MEMO, Dundee)



Mr Average

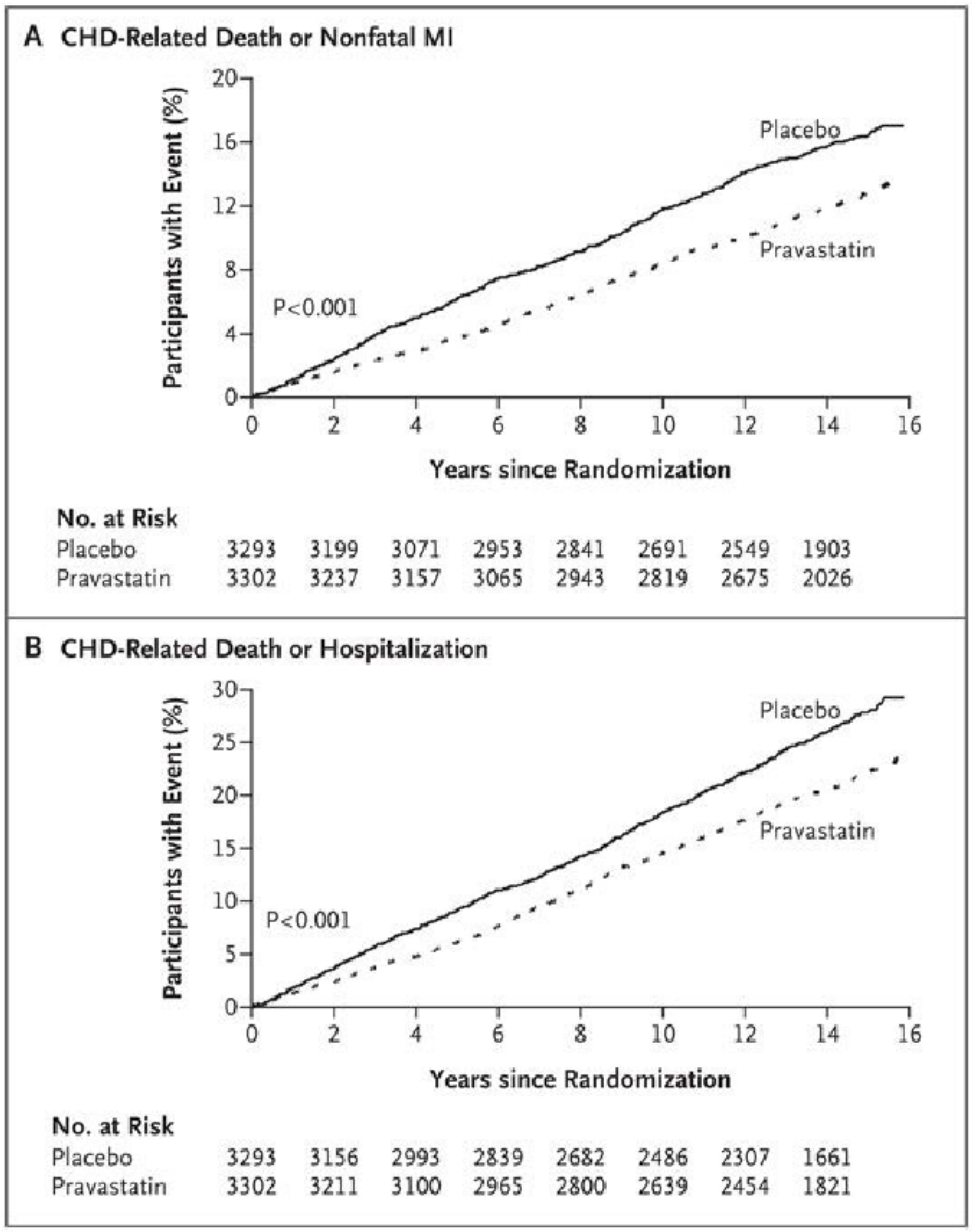
Slide courtesy of Tom McDonald, MEMO, Dundee

Harveian Oration, 2008

"RCTs, long regarded as the "gold standard" of evidence, have been put on an undeserved pedestal..

Observational studies are useful and with care in the interpretation of the results can provide an important source of evidence about both the benefits and harms of therapeutic interventions."

M. Rawlins

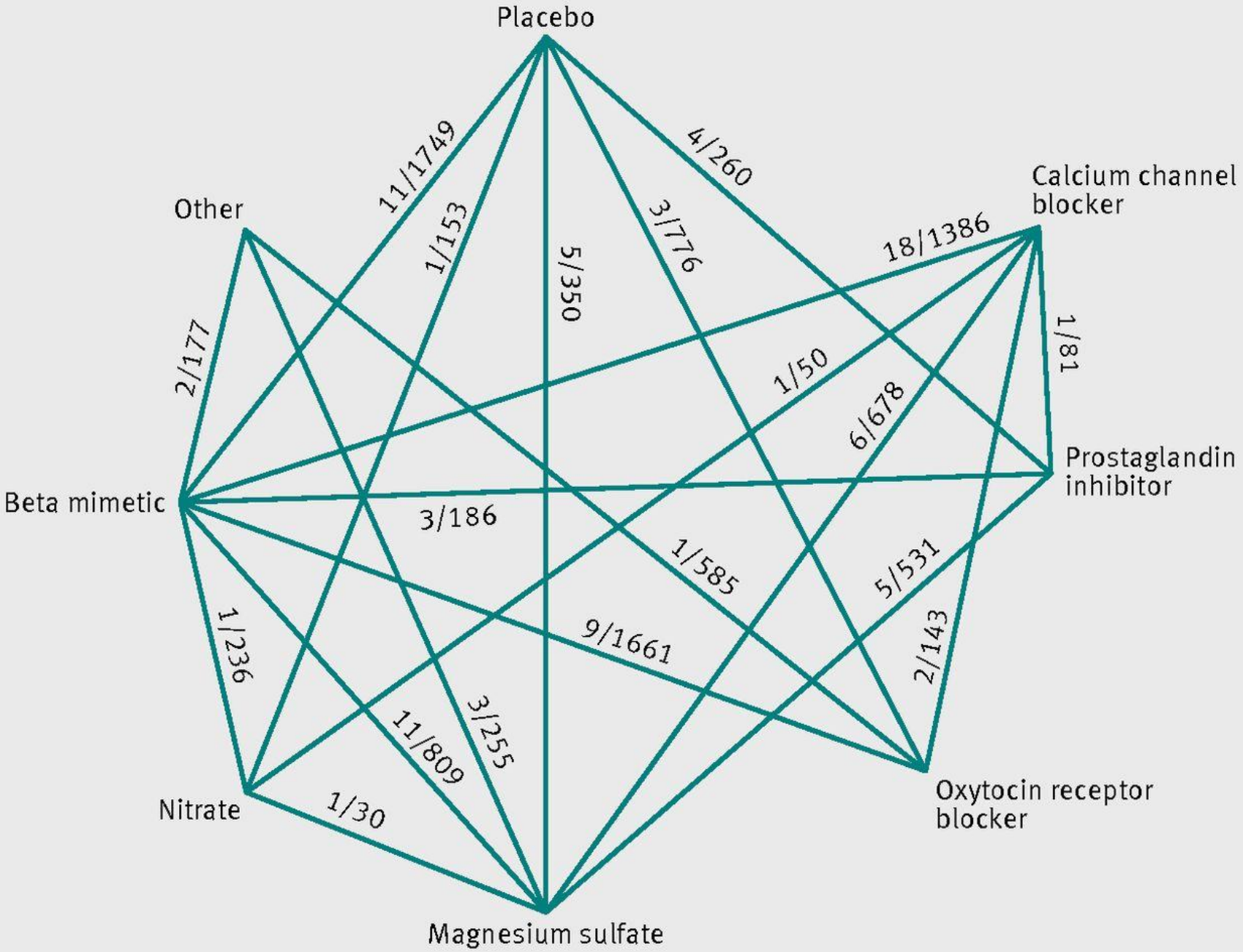


Patients in 5-year WOSCOPS trial (£mi's)

Data linkage identified further coronary events over next 10 years confirming long term benefit of statins (£1,000's)

Slide courtesy of Scott Heald, ISD

Ford I, Murray H, Packard CJ, Shepherd J, Macfarlane PW, Cobbe SM. Long-term follow-up of the West of Scotland Coronary Prevention Study. N Engl J Med 2007;357:1477-1486.


Placebo
Other
Calcium channel blocker
Beta mimetic
Prostaglandin inhibitor
Nitrate
Oxytocin receptor blocker
Magnesium sulfate
11/1749
4/260
1/153
5/350
3/776
18/1386
2/177
1/81
1/50
6/678
3/186
1/585
5/531
1/236
9/1661
2/143
3/255
11/809
1/30

Cost effectiveness (Economic evaluation)

Economic evaluation has been defined as

*‘ the **comparative** analysis of alternative courses of action in terms of both their **costs** and **consequences**’* (Drummond & McGuire, 2001)

An evolving art: modelling what happens to patients in the long-term, in the light of major uncertainties

Guidelines from NICE, SMC, PBAC, CADTH…

Real World Data

Data that are collected outside the controlled constraints of conventional randomised controlled clinical trials to evaluate what is happening in normal clinical practice

ABPI White Paper: The Vision for Real World Data – Harnessing Opportunities in the UK

September 2011

Uses of real world evidence

- Natural history of disease progression
- Epidemiology (for budget impact)
- Patient pathways, unmet needs
- To measure clinical outcomes and resolve uncertainty (safety and effectiveness)
- Patient experience
- Adherence/compliance
- Utilities
- Resource use

Using RWD

- Understand purpose of data collection to identify potential biases and confounding
- Present results with limitations and uncertainties (structural and statistical)
- Need a drug development plan that includes not only clinical studies, but other sources that will demonstrate product value

Scientific Advice for HTA

- Individual Agencies alone and in collaboration with regulatory agencies and EMA
- Tapestry
- Green Park Collaborative
- The lead statistician needs to be involved in these meetings

Patient engagement

International AIDS Society
Stronger Together Against HIV

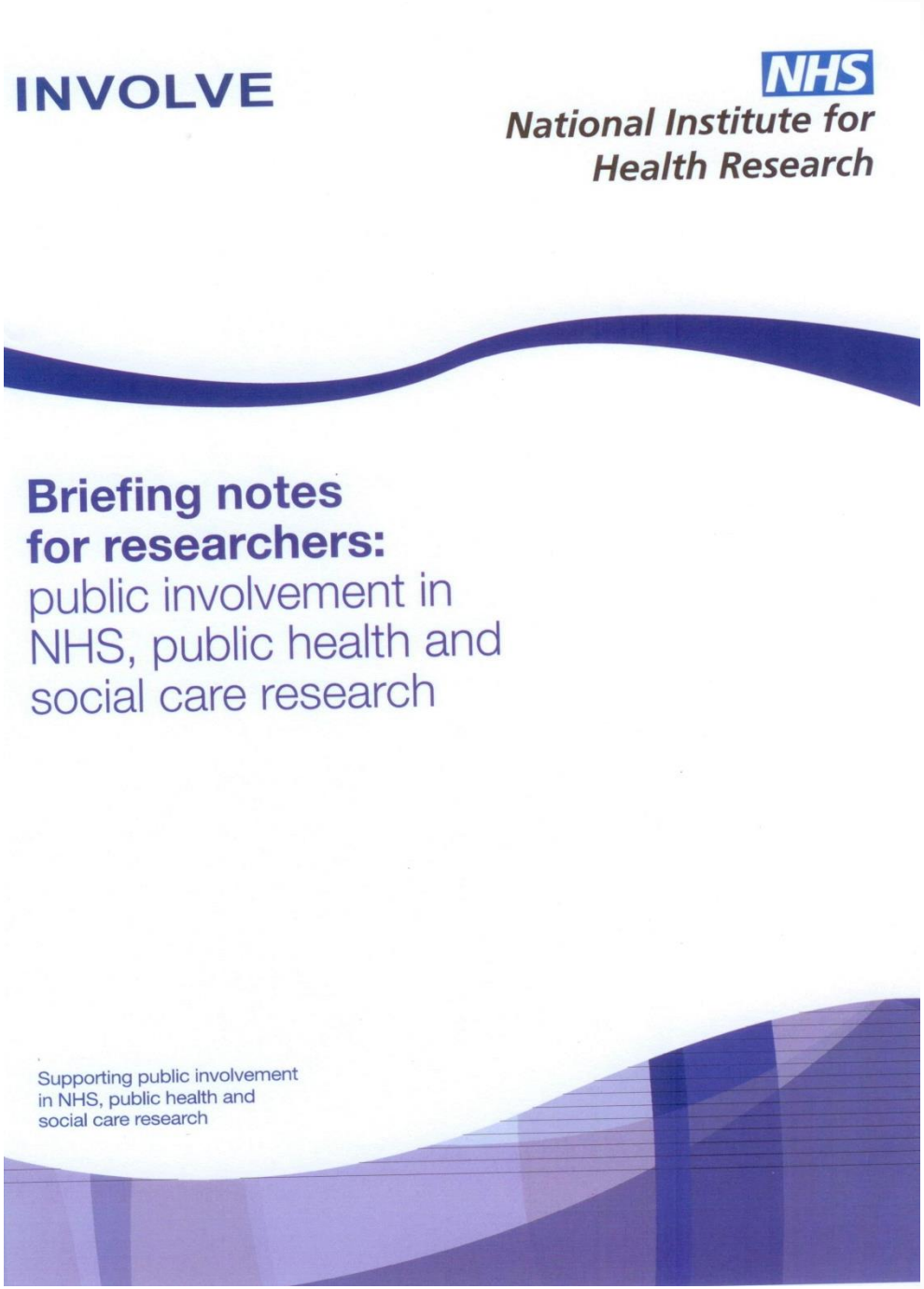
25 YEARS OF COMMITMENT

1992: 8th Conference
A World United Against AIDS
8,000 participants

Patients' and care-givers' experiences

- Living with an illness
  - 'No one knows better what it is like to live with an illness day in, day out, than those who are doing this – the patients and their family and friends who care for them.'
- The technology
  - Their needs and preferences, benefits and unwanted effects

Understanding HTA. Health Equality Europe. 2008
http://www.htai.org/index.php?id=744


INVOLVE
NHS
National Institute for
Health Research
Briefing notes
for researchers:
public involvement in
NHS, public health and
social care research
Supporting public involvement
in NHS, public health and
social care research

HTA influencing drug development

- Pragmatic study designs
- Outcomes that are important to patients
- Duration of treatment
  - Reassessment, how?
  - Continue responders
- Duration of effect
- National studies of resource utilisation
- Identifying patients that benefit most

- *Creating an evidence base to demonstrate added value, not just benefit:risk*

The evolving role of the statistician

~~Clinical~~ Evidence development programme to explain the added value of the product

- Working with patients in phase II
- Phase III trials collecting evidence for HTA
- Building the Network Meta Analyses and economic model from a range of sources
- Critically appraising model inputs
- Assessing uncertainty
- Developing evidence post-marketing for reassessment
- Helping create the HTA submission and responding to queries