



# HTA for improving quality

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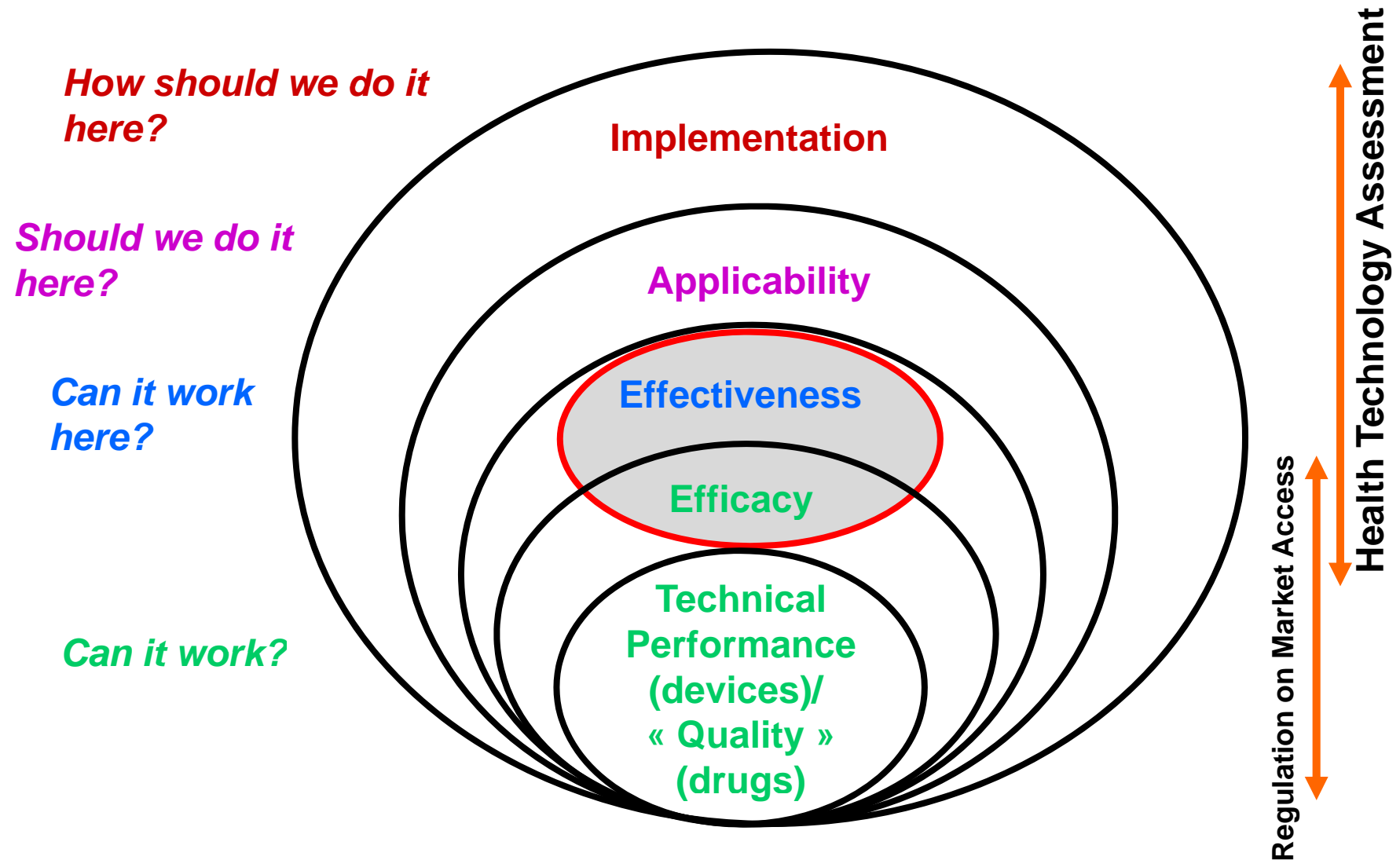


## HTA and the broader quality agenda

**“... quality of care is that component of the difference between efficacy and effectiveness that can be attributed to care providers, taking account of the environment in which they work”**

**(Brook & Lohr 1985)**

# Layers of questions when deciding upon health technologies



## Efficacy

- explanatory trials
- highly selected populations
- comparator: placebo
- outcomes: clinical, morbidity, mortality, adverse effects
- ‘what it says on the packet’

## Effectiveness

- pragmatic trials
- few exclusions
- comparator: ‘current (best) practice’
- outcomes: patient-focused, down-stream resources
- ‘the real life effect’

## Efficacy

- explanatory trials
- highly controlled
- population: healthy
- comparator: placebo
- outcomes: clinical, morbidity, mortality, adverse events
- 'what is in the packet'

**Licensing**

## Effectiveness

- pragmatic trials
- few controls
- comparator: 'current (best) practice'
- outcomes: patient focused, down-stream

**Coverage**

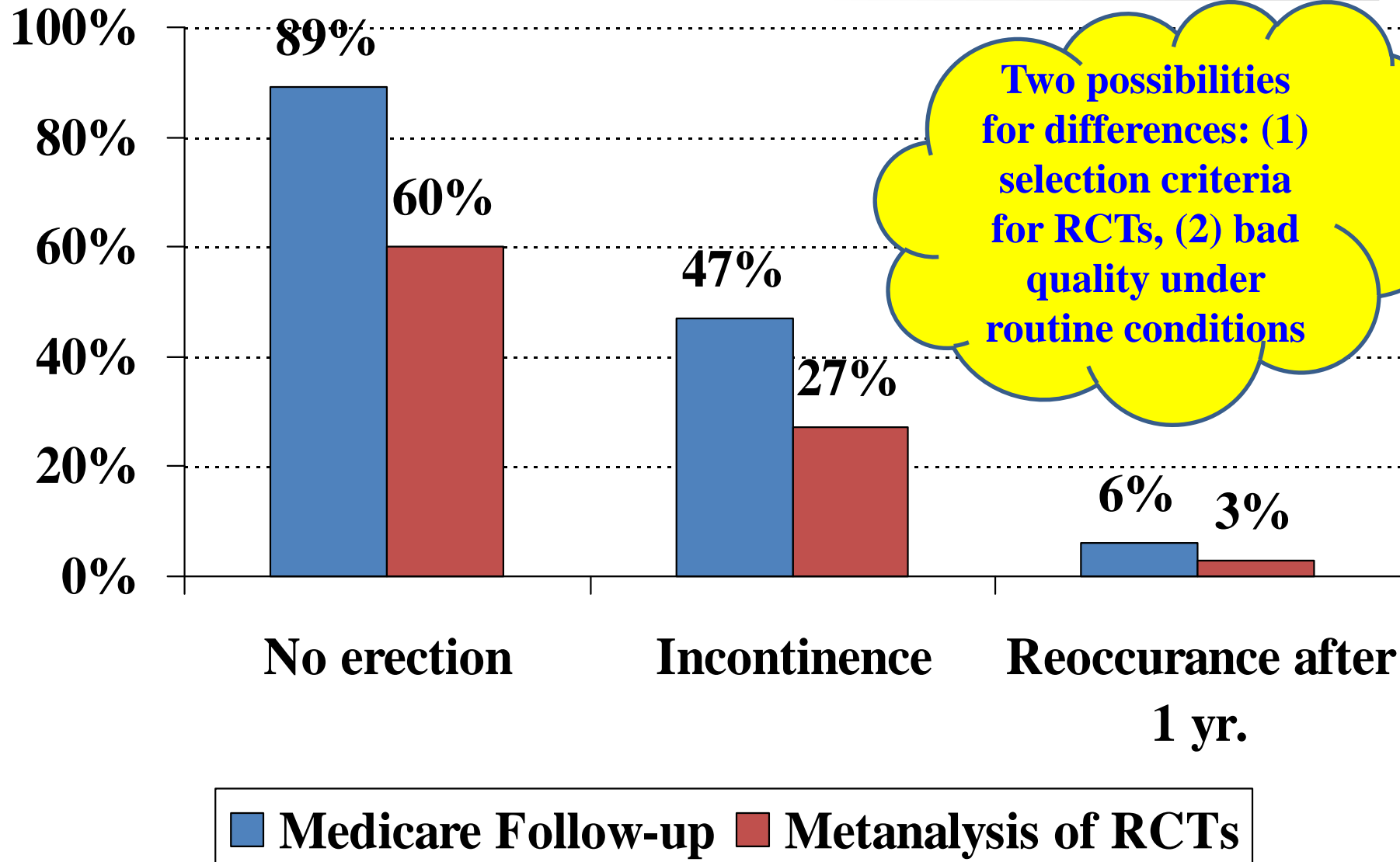
**Evidence Gap**

# Final outcomes versus surrogate parameters

|   |                           |  |   |   |
|---|---------------------------|--|---|---|
| Coronary thrombosis (thrombolysis)      | Quality-adjusted survival | Number surviving                       | Number with specified level of left ventricular function  | Number achieving coronary reperfusion   |
| Unstable angina (various interventions) | Quality-adjusted survival | Number surviving                       | Number with myocardial infarction                         | Number with adequate relief of pain   |
| Stable angina (various interventions)   | Quality-adjusted survival | Number with acceptable quality of life | Number who can walk a specified distance                  | Number with adequate relief of pain   |
| Asthma (various drugs)                  | Quality-adjusted survival | Number surviving                       | Number with adequate control of bronchial hyperreactivity | Number achieving a target level of airways function                             |
| Depression (various drugs)              | Quality-adjusted survival | Number avoiding suicide                | Quality of life (may be improved by drugs)                | Number achieving a target Hamilton or Montgomery-Asberg Depression Rating Scale |
| Hypertension (various drugs)            | Quality-adjusted survival | Number avoiding stroke                 | Quality of life (may be worsened by drugs)                | Number achieving a target blood pressure  |

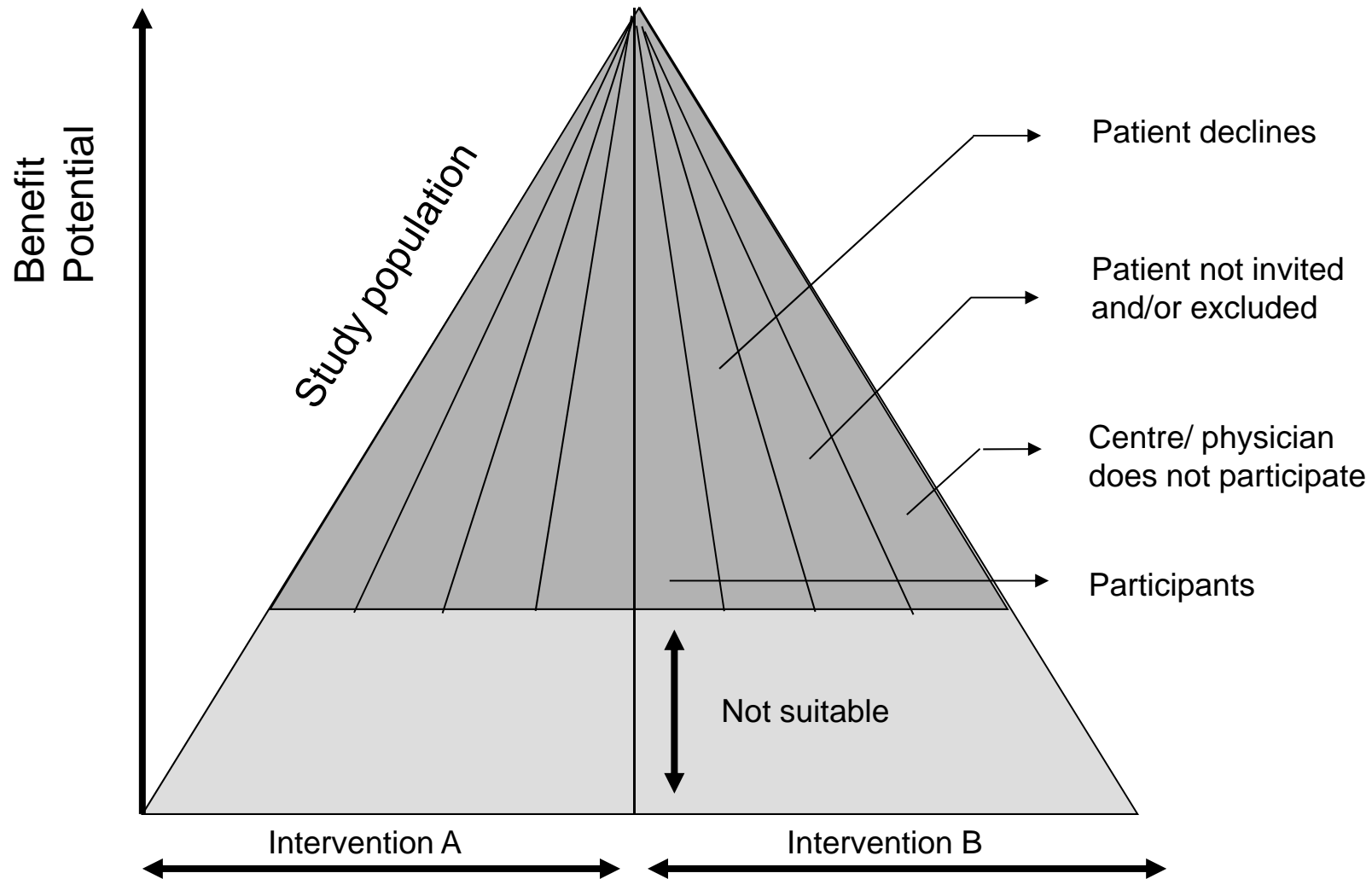
source: PBAC

# Example: Outcomes of prostatectomy



Data from: Fowler FJ, Roman A, Barry MJ, Wasson J, Lu-Yao G, Wennberg JE (1993). Patient-reported complications and follow-up treatment after radical prostatectomy - the national Medicare experience 1988-1990. Urology 42: 622-9

# RCT selection -> RCT participation



Based on: McKee M et al. BMJ 1999;319:312-315



# RCT selection -> RCT participation

Benefit Potential

Population

**On average, only 15% of actual patients meet RCT inclusion criteria.  
To what degree can we generalize results to the other 85%?**

Participants

Not suitable

Intervention A

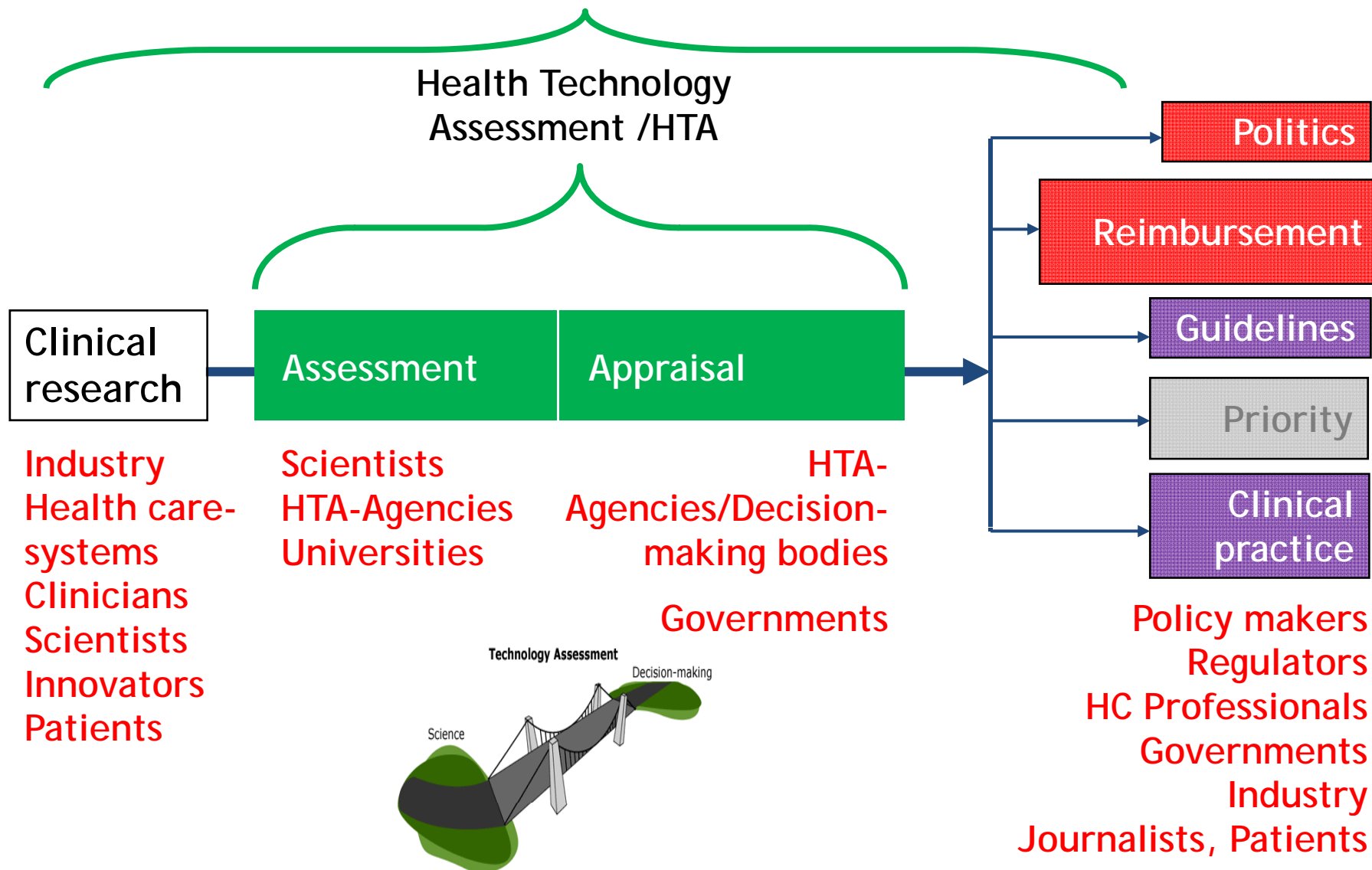
Intervention B

Based on: McKee M et al. BMJ 1999;319:312-315

## Preferred study designs (in 11 countries doing drug HTA)

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- preferably “head-to-head” randomized controlled trials (direct comparisons)
- majority favours final outcome parameters (change in mortality, morbidity, quality of life) and studies in “natural” and country specific setting
- **But: available are often 24-week RCTs against placebo with highly selected patients and providers conducted in a mixture of countries**



Most health care systems have some form of a uniform/ standard/ minimum package of benefits to which the persons covered are entitled; can be

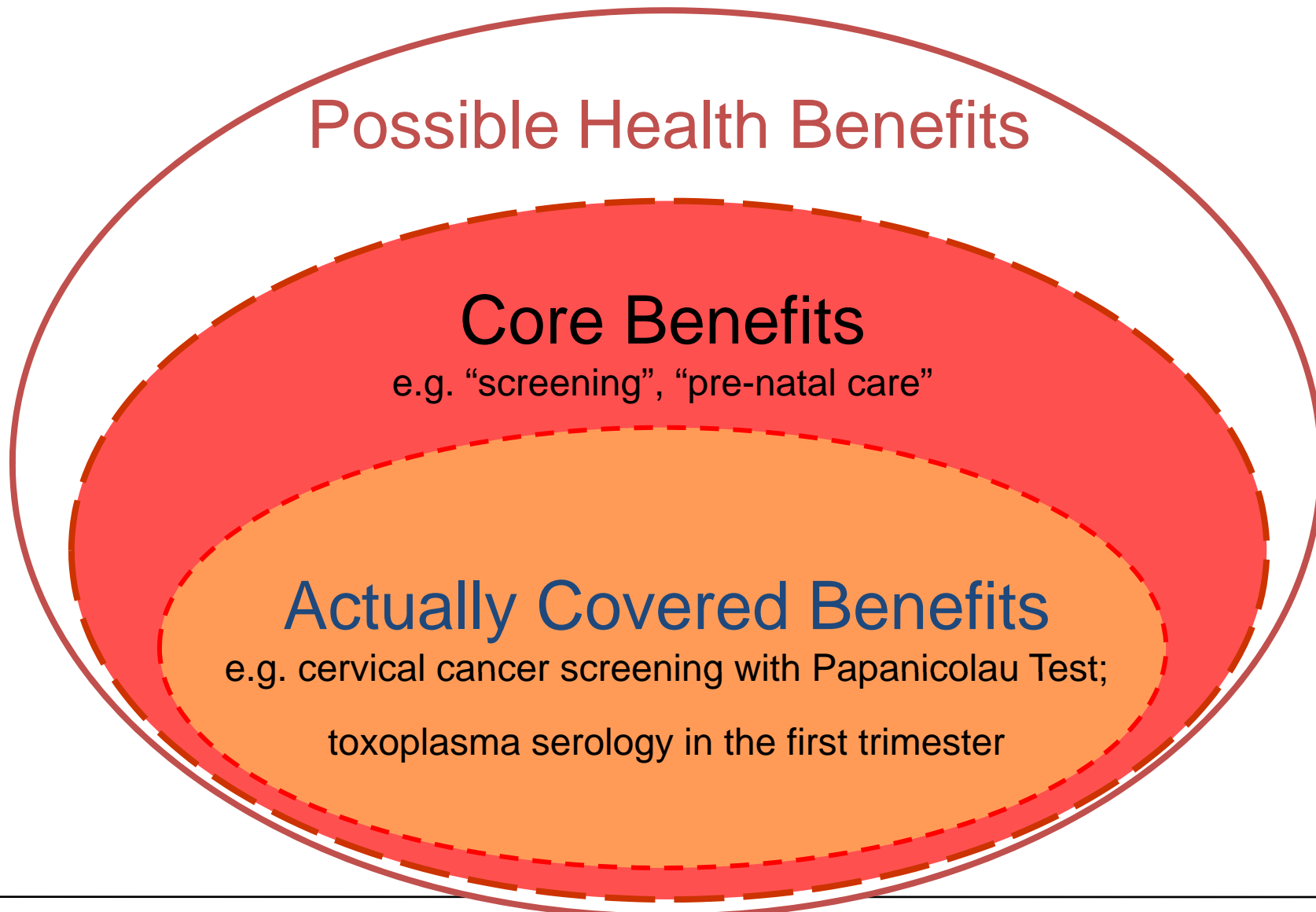
- implicit, i.e. based on traditions and routine
- explicit, i.e.
  - a list states all the benefits available under the statutory system (rarely)
  - separate lists exist for various sectors (more often)
  - and/or negative lists
  - often primarily intended as fee schedule

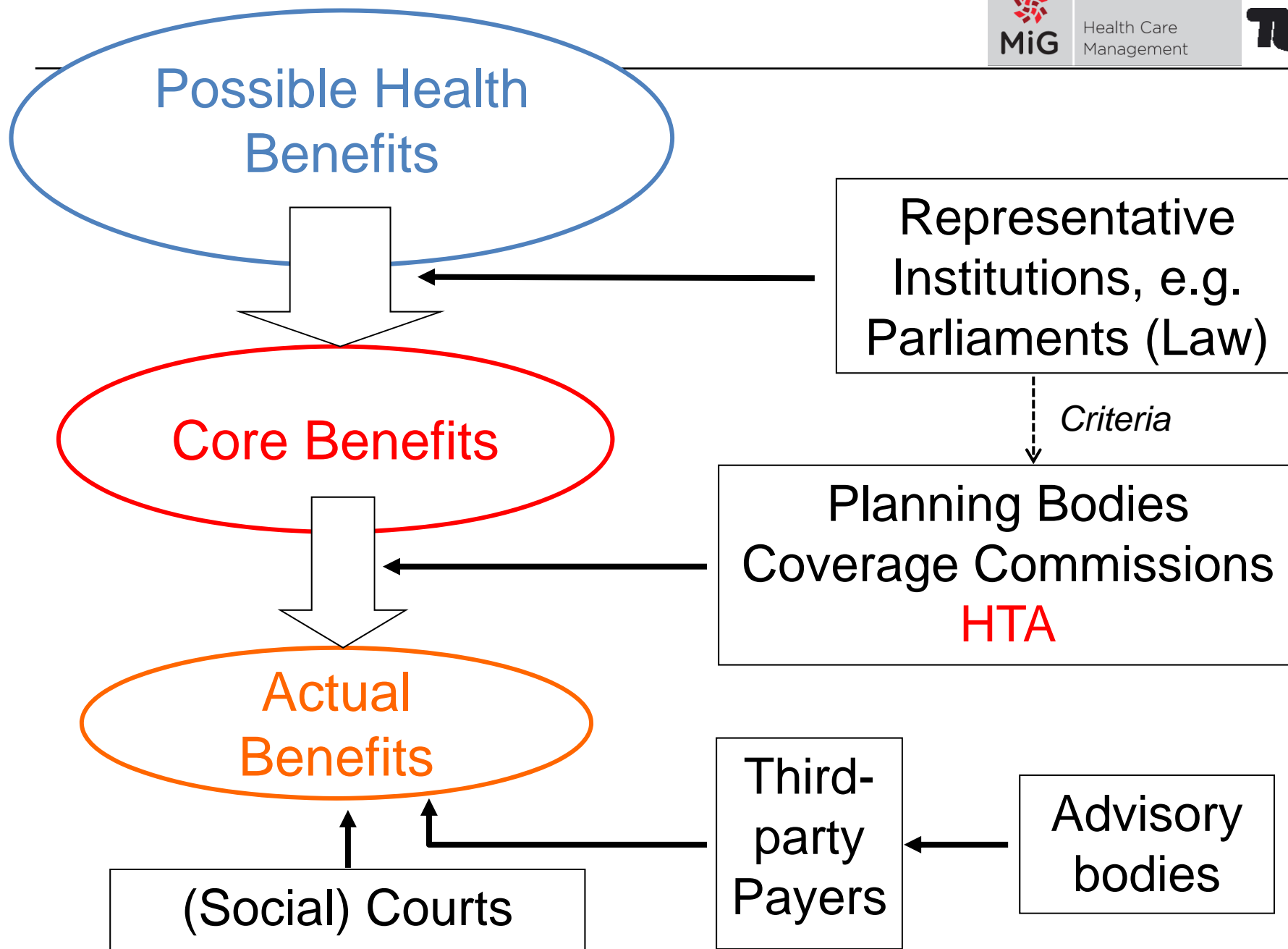
Main questions: WHAT is included/ excluded? WHO decides?

Based on WHICH CRITERIA? → HTA

# What is included in the benefit package?

## A model





# Example: Outpatient benefit catalogues (I)

| Country | Name of taxonomy                                   | Applied geographical area                                     | Taxonomy (and grouping criteria)  | Actors involved indecision making   | Criteria for in-/exclusion of benefits  |
|---------|--|---|---|---|---|
| Denmark | Health Care Reimbursement Scheme Fee Schedule      | National  | Services are grouped according to medical specialty and for GPs additionally in basket, supplementary, laboratory and miscellaneous services. Each service has an item number. It is referred to the respective legislation decree specifying the benefit, certain goods, procedures or in rare cases indications | <ul style="list-style-type: none"> <li>• National level (law, general framework)</li> <li>• Ministry for the Interior and Health (approval)</li> <li>• Counties (budgeting, health plan)</li> <li>• Healthcare Reimbursement Negotiating Committee and health professional associations (negotiate catalogue)</li> </ul>                        | Need  |
| France  | Common Classification of Medical Procedures (CCAM) | National  | Lists all medical procedures reimbursable and excluded. Grouping criteria: anatomic classification, medical specialties   | <ul style="list-style-type: none"> <li>• National level (law, general framework)</li> <li>• Ministry of Health (approval)</li> <li>• National Union of Health Insurance Funds (in- and exclusion of services)</li> <li>• High Health Authority (advisory body on in- and exclusion of services)</li> </ul>                                      | Effectiveness, safety   |
| Germany | SHI-EBM, SHI-BEMA, SHI-BEL-II                      | National  | Services are grouped according to the medical specialty allowed to provide the service. Each service is assigned a numeric code in accordance with the subjection of the catalogue  | <ul style="list-style-type: none"> <li>• National level (law, general framework)</li> <li>• Federal Joint Committee (approval of new benefits)</li> <li>• Valuation Committee (negotiates EBM)</li> <li>• Dental Valuation Committee (negotiates BEMA, BEL-II)</li> </ul>   | Diagnostic and therapeutic expedience, medical necessity and cost-effectiveness |
| Hungary | Governmental decrees and reimbursement catalogues  | National  | Similar services are listed in groups. Governmental decrees relate to different areas of care (e.g., dental care, specialist services). Items in reimbursement catalogues are listed with the respective ICPM code and a point value  | <ul style="list-style-type: none"> <li>• Legislation at the national level (law, general framework, budgeting)</li> <li>• Ministry of Welfare (decrees, approval) - National Health Insurance Fund</li> <li>• Administration, especially (prepares decisions)</li> <li>• Payment Codes Updating Committee (reimbursement catalogues)</li> </ul> | Costs, effectiveness  |
| Italy   | National contract for specialist outpatient        | National benefit package, regions include additional services | Contract for primary care describes obligations of GP. Individual services are not further itemized. Decree on specialist outpatient services lists services in three sections: available, availability restricted to specific indications, excluded  | <ul style="list-style-type: none"> <li>• Government at national level (sets decree, negotiates contract)</li> <li>• Representatives of GPs (negotiate contract)</li> <li>• Ministry of Health (transfers contract into law)</li> <li>• Governmental regional level (negotiates additional contracts)</li> </ul>                                 | Effectiveness, costs  |

# Example: Outpatient benefit catalogues (II)

| Country         | Name of taxonomy   | Applied geographical area   | Taxonomy (and grouping criteria)   | Actors involved in decision making   | Criteria for in-/exclusion of benefits   |
|-----------------|--|---|--|--|--|
| The Netherlands | Health Insurance (Treatment and Services) Decree; Diagnose Behandeling Combinaties (DBC; DRG-like system); Jan. 2005                                       | National  | GP services are regulated in generic terms only by decree, DBC catalogue (111,527 DBCs) combine information on diagnosis and treatment for medical specialists. DBCs are on three different lists determining the status for tariff negotiations or excluding DBCs from the benefit package. Grouping criteria: medical specialty, product group | <ul style="list-style-type: none"> <li>• Legislation at the national level (law, general framework)</li> <li>• Ministry of Health (decrees)</li> <li>• DBC-Maintenance Organization (DBC-System)</li> <li>• Physicians (priority setting)</li> </ul>   | Costs, effective-ness  |
| Poland          | and catalogue of benefits  | National  | Catalogue lists all services covered under social health insurance scheme. Services are linked to the respective regulation/law. Grouping criteria: area of care, medical specialty  | <ul style="list-style-type: none"> <li>• Legislation at the national level (law, general framework)</li> <li>• Ministry of Health (regulations)</li> <li>• National Health Fund (catalogue)</li> </ul>   |  |
| Spain           | Royal Decree 63/1995   | National with regional differences  | Services are listed explicitly in decree. In some cases, services are restricted to specific patient groups. Decree lists services in 5 areas of care (e.g., primary care, specialized care, pharmaceutical care) which are further subdivided.  | <ul style="list-style-type: none"> <li>• Legislation at the national level (law, general framework)</li> <li>• Federal Government (decree)</li> <li>• Inter-territorial Council and Council of the State (inclusion of new benefits)</li> <li>• Clinicians (provision of services relating to entitlements defined by decree)</li> </ul> | Safety, efficacy, efficiency   |
| UK (England)    | <ul style="list-style-type: none"> <li>• National Service Framework</li> <li>• General Medical Services Contract</li> <li>• Clinical Guidelines</li> </ul> | <ul style="list-style-type: none"> <li>• National</li> <li>• National with possible variation at PCT-level</li> <li>• National</li> </ul> | Health Resource Groups are linked to procedures. Currently only 48 HRGs are in use. Guidelines recommend services to be used on certain indications  | <ul style="list-style-type: none"> <li>• Legislator at national level (law, general framework)</li> <li>• NHS Confederation and General Practitioners Committee (negotiate contract)</li> <li>• Primary Care Trusts (PCT) (negotiate additional contracts)</li> <li>• NICE (clinical guidelines)</li> </ul>                              | <ul style="list-style-type: none"> <li>• Need, effectiveness</li> <li>• Need, costs</li> <li>• Need, costs, effectiveness</li> </ul> |



# Example: explicit exclusions

Explicit exclusions from health baskets in studied countries<sup>a</sup>

| Source (S), Exceptions (E)                  | D  |          | DK |      | E  |      | F  |      | H  |      | I  |      | NL |                | PL |      | UK |      |
|---|----|----------|----|------|----|------|----|------|----|------|----|------|----|----------------|----|------|----|------|
|   | S  | E        | S  | E    | S  | E    | S  | E    | S  | E    | S  | E    | S  | E              | S  | E    | S  | E    |
| Service / Item                              |    |          |    |      |    |      |    |      |    |      |    |      |    |                |    |      |    |      |
| Cosmetic Surgery                            |    | n.e.     |    | n.e. | LF | M    |    | n.e. | LF | n.s. | LF | M    | LF | M              | QL | M    | QL | M    |
| Medical Examinations/<br>Certifications     |    | n.e.     |    | n.e. | LF | RC   |    | n.e. | LF | RC   | LF | Ch   |    | n.e.           | LF | RC   |    | n.e. |
| OTC-Drugs / OTC-Aids                        | LF | Ch, D, M |    | n.e. | QL | n.s. |    | n.e. |    | n.e. | QL | n.s. | LF | CD             |    | n.e. | QL | M    |
| Complementary Medicine*                     |    | n.e.     |    | n.e. |    | n.e. |    | n.e. | LF | n.s. | LF | n.s. | LF | n.s.           | QL | CD   |    | n.e. |
| Unconventional therapies*                   | QL | M        |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | n.s. |    | n.e.           | QL | n.s. |    | n.e. |
| Thermal Medicine / Spa*                     |    | n.e.     |    | n.e. | LF | n.s. |    | n.e. |    | n.e. | LF | n.s. |    | n.e.           | QL | n.s. |    | n.e. |
| Sterilisation                               |    | n.e.     |    | n.e. |    | n.e. |    | n.e. | LF | M    |    | n.e. | LF | n.s.           | QL | D    |    | n.e. |
| Abortion                                    |    | n.e.     |    | n.e. |    | n.e. |    | n.e. | LF | M    |    | n.e. |    | n.e.           |    | n.e. |    | n.e. |
| IVF   |    | n.e.     |    | n.e. |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | <sup>2/3</sup> |    | n.e. |    | n.e. |
| Ritual circumcision                         |    | n.e.     |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | n.s. | LF | n.s.           |    | n.e. |    | n.e. |
| Bone densitometry                           | QL | M        |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | M    |    | n.e.           |    | n.e. |    | n.e. |
| Physiotherapy                               |    | n.e.     |    | n.e. |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | Ch, CD         |    | n.e. |    | n.e. |
| Sex-Change Surgery                          |    | n.e.     |    | n.e. | LF | M    |    | n.e. |    | n.e. |    | n.e. |    | n.e.           | QL | n.s. |    | n.e. |
| Contact Lenses                              | LF | M        |    | n.e. |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | M              |    | n.e. |    | n.e. |
| Refractive Surgery                          | QL | M        |    | n.e. |    | n.e. |    | n.e. |    | n.e. | LF | M    |    | n.e.           |    | n.e. |    | n.e. |
| Psychoanalysis                              |    | n.e.     |    | n.e. | LF | n.s. |    | n.e. |    | n.e. |    | n.e. |    | n.e.           | QL | n.s. |    | n.e. |
| Other Specific<br>Procedures/Technologies** | QL | M        |    | n.e. |    | n.e. | QL | M    | LF | n.s. | LF | n.s. | LF | n.s.           | QL | n.s. | QL | M    |

<sup>a</sup> Denmark (DK), France (F) Germany (D), Hungary (H), Italy (I), Netherlands (NL), Poland (PL), Spain (E), England (UK)

LF: Stated in Health Basket Legal Framework, QL: Quasi-Law (Clinical Guidelines/Recommendations, Service Implementation Guidelines, Contracts, Negative Lists)

n.e.: no explicit exclusions, n.s.: not stated

M: Medical necessity, special clinical circumstances (i.e., accident, malformation, disease), Ch: Children, Youth (<18), CD: Chronic disease, D: disabled persons, RC: certifications related to health care, <sup>2/3</sup>: 2nd and 3rd attempt

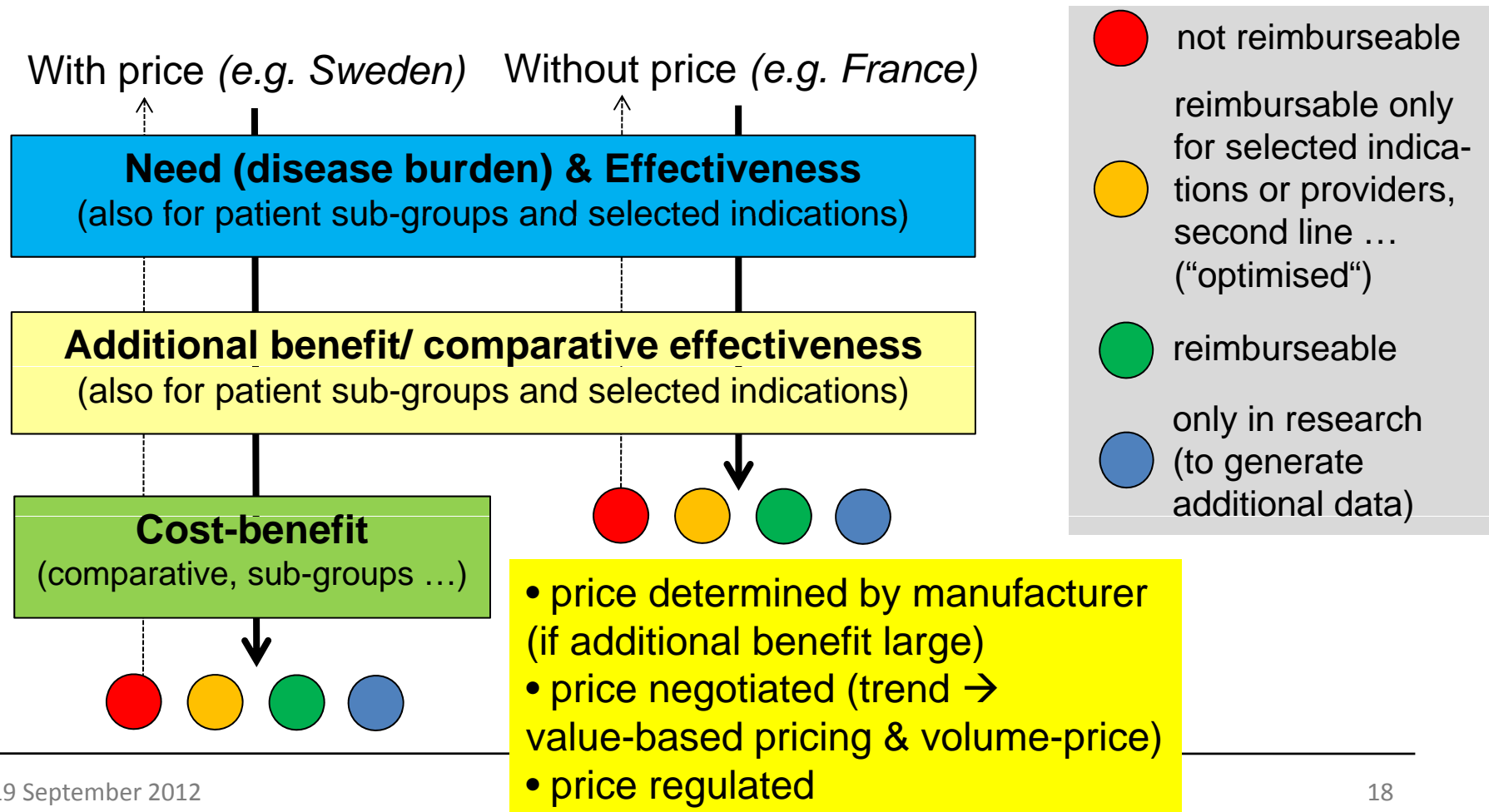
\* At least one intervention explicitly excluded, \*\* Mainly specific screening, unconventional therapies, or specific devices

# HTA direct after market launch

*(currently existing mainly for drugs)*








**New drug/ device/ intervention**  
 → „single technology assessment“:

Important input = structured information (dossier of manufacturer/ promoter)



## More than YES or NO:

### Decision Options (e.g. in Switzerland)

| Coverage<br>(reimbursement)   | Decision   |
|---|--|
| Yes    | reimbursement without conditions                     |
| Yes    | reimbursement for specific indications               |
| Yes    | in centers which have to fulfil certain requirements |
| Yes   | in centers + evaluation registers                    |
| Yes  | in evaluation (by benefit commission)                |
| No   | in evaluation (by applicant)                         |
| No   | refusal  |

Source: Swiss Federal Office for Social Security (SFOSS)

## More than YES or NO:

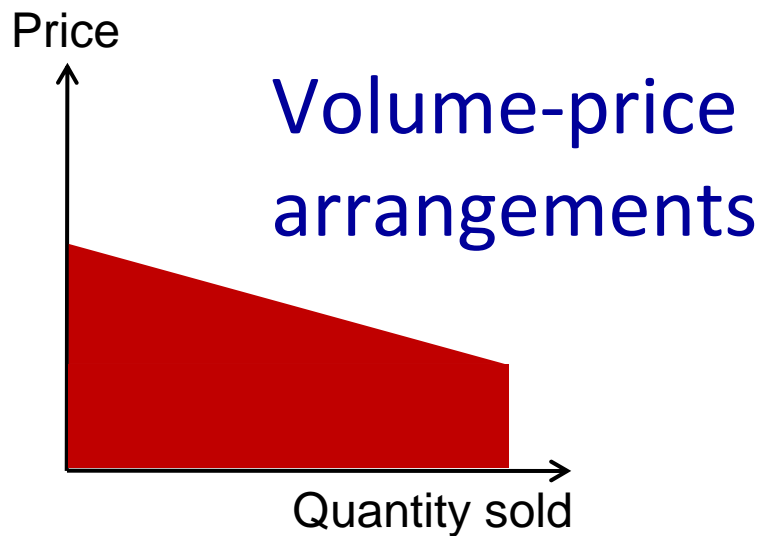
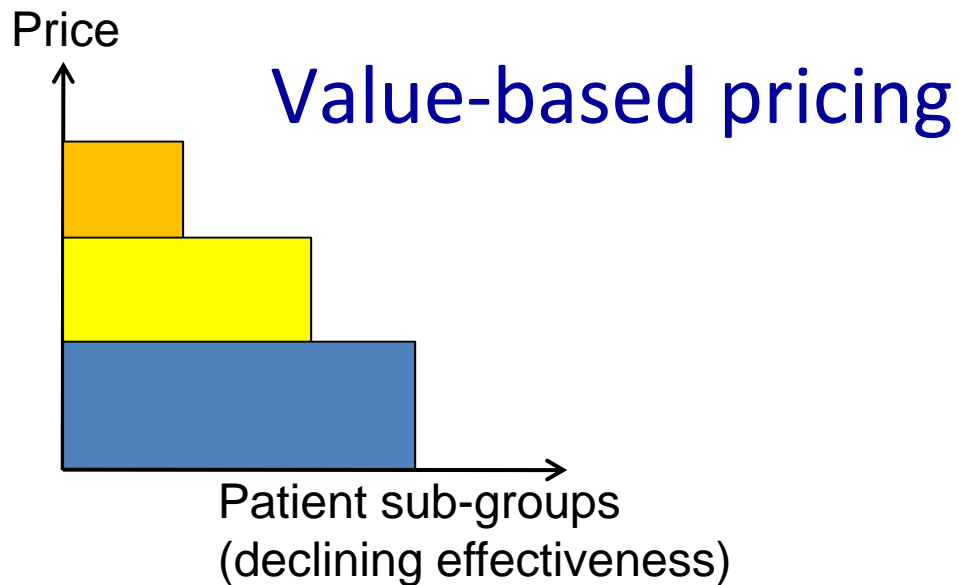
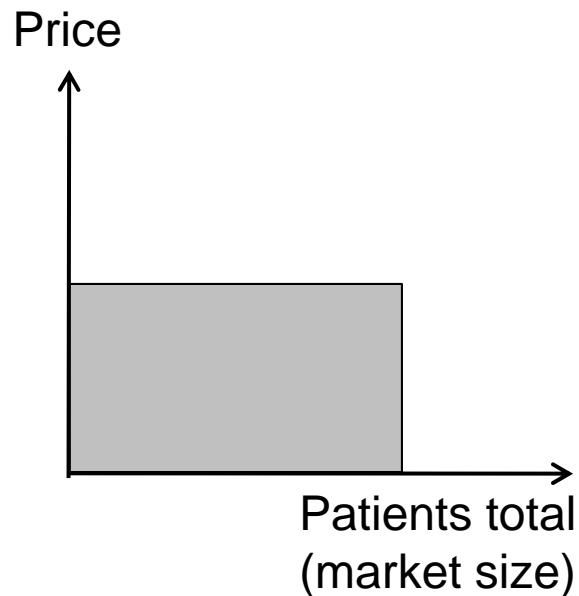
### Actual decisions in England

### (results of NICE “Technology Appraisals” 3/2000-8/2009)

| Decision         | Number | Percent |                      | Number | % of opt. rec. |
|------------------|--------|---------|----------------------|--------|----------------|
| Unrestricted     | 98     | 29%     |                      |        |                |
| Optimised        | 188    | 55%     | By patient group     | 158    | 80%            |
| Only in Research | 21     | 6%      | By price             | 53     | 27%            |
| Not recommended  | 31     | 9%      | By continuation rule | 34     | 17%            |
| Non-submission   | 4      | 1%      | By regimen           | 7      | 4%             |
| Total            | 342    | 100%    | By setting           | 4      | 2%             |

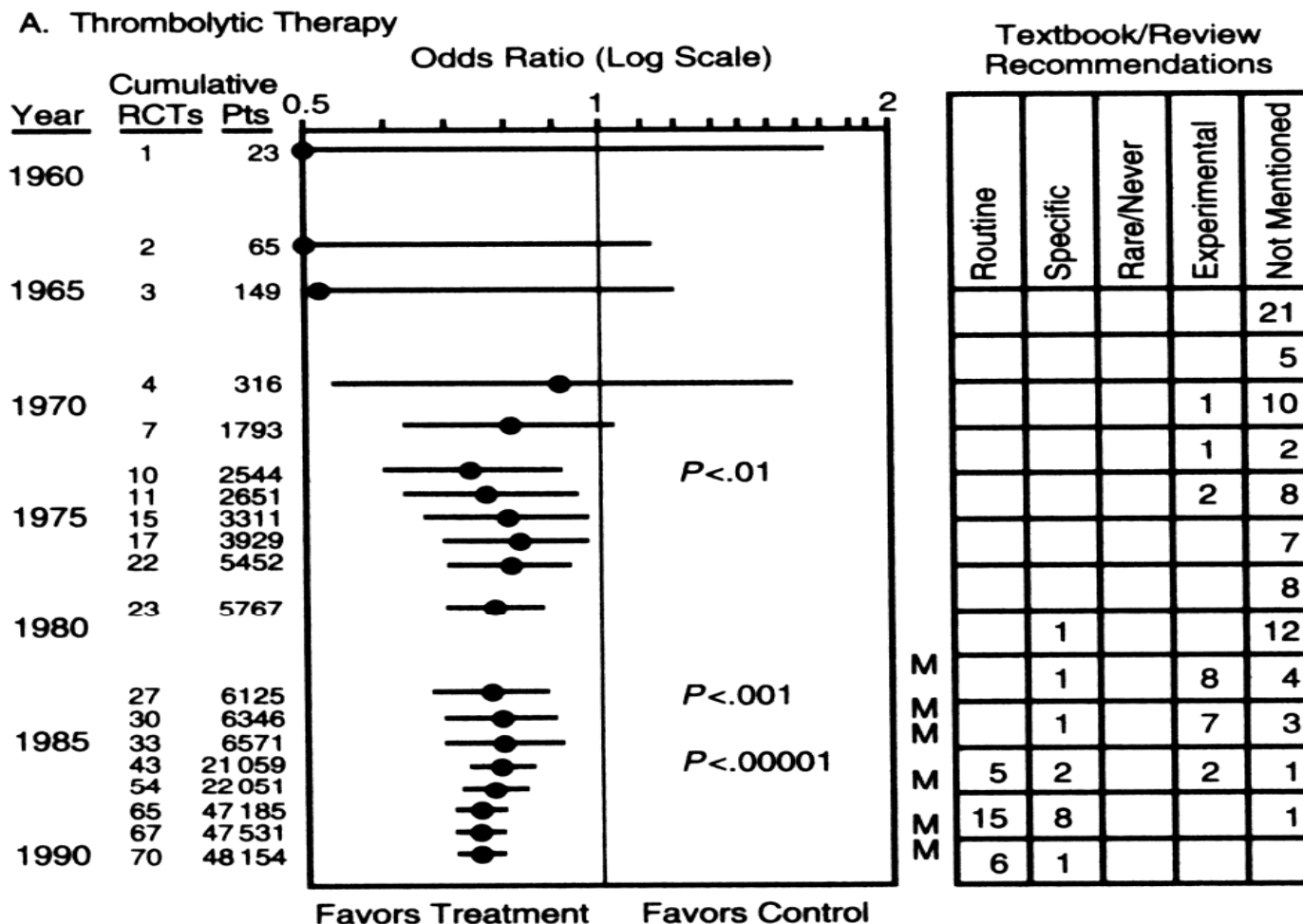
342 individual recommendations in 166 technology appraisals

# Value-based pricing and volume-price arrangements



# Reaching physicians/ clinicians earlier:

## The case of 13 years of delay in thrombolytic therapy



Antman EM, Lau J, Kupelnick B, Mosteller F, Chalmers TC. JAMA 1992, 268:240-8

... but not confusing them ...



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# STATINS

They're the wonder drug some believe could eradicate heart disease. But are they safe enough to hand out to millions of new patients?

**YES** SAYS PROFESSOR RORY COLLINS

**NO** SAYS PROFESSOR TOM SA

**commentary**

## Is science dangerous?

Does society need protecting from scientific advances? Most emphatically not, so long as scientists themselves and their employers are committed to full disclosure of what they know.

**Lewis Wolpert**

The idea that knowledge is dangerous is deeply embedded in our culture. Adam and Eve were forbidden to eat from the biblical Tree of Knowledge, and in Milton's *Paradise Lost* the serpent addresses the Tree as the "Mother of Science". The archangel Raphael advises Adam to be "loosely wive" when he tries to question him about the nature of the Universe. Indeed, western literature is filled with images of scientists meddling with nature, with disastrous results. Scientists are portrayed as a wilder group, unconcerned with ethical issues.

But is science in fact dangerous, and do scientists have special social responsibilities? It is essential to recognize that reliable scientific knowledge has no moral or ethical value.

**Dazed and confused**  
If the experts can't agree, what chance for the rest of us?

### Reckless medical scans

For a prime example of medical screening that has proliferated beyond reason, consider the alarming case of full-body computed tomography scans to detect cancer, cardiovascular disease and other conditions. Narrowly targeted CT scans aimed at particular organs are undeniably valuable when used to pin down a diagnosis in sick patients. But full-body scans to screen healthy individuals for hidden disease have never been shown to be effective, and now there is evi-

patients to undergo needless further tests. The U.S. Food and Drug Administration and the American College of Radiology, among other expert groups, oppose full-body scans in people with no disease symptoms.

A study published in May found that radiologists and emergency-room physicians were largely unaware of how much radiation the scans delivered, and now a study by Columbia University researchers highlights the risk of dying from radiation-induced cancer. A single

RADIO, TV, the Internet, even mobile phones constantly bombard us with snippets of news from around the globe. Trouble is who and what to believe? Science has always been lucky in this regard. Peer-reviewed journals ensure that protocols are followed, discussions rational and conclusions justified. Which makes it all the more disconcerting when news comes from the

### Making decisions about hormone replacement therapy

Janice Rytner, Ruth Wilson, Karen Ballard

Many women will at some stage consider taking hormone replacement therapy, but uncertainty about the risks and benefits makes this decision difficult



... let them develop (and use)

clinical guidelines according to international standards ...

AGREE

### *Six domains*

- 23 items
- 4-point Likert Scale

Overall  
assessment

User guide

1. Scope & purpose (3)
2. Stakeholder involvement (4)
3. Rigour of development (7)
4. Clarity & presentation (4)
5. Applicability (3)
6. Editorial independence (2)





## ... or encourage them to adapt clinical guidelines developed elsewhere

### **ADAPTE Framework**

The ADAPTE framework is a systematic approach to aid in the adaptation of guidelines produced in one setting to be used in a different cultural and/or organizational context. The ADAPTE framework is based on the following core principles:

- Respect of evidence-based principles for guideline development;
- Reliable and consistent methods to ensure the quality of the adapted guideline;
- Participation of key stakeholders to foster acceptance and ownership of the adapted guideline;
- Explicit consideration of context to ensure relevance for local practice and policies;
- Transparent reporting to promote confidence in the recommendations of the adapted guideline;
- Flexible format to accommodate specific needs and circumstances;
- Respect for and acknowledgement of source guidelines material.