



回顧澳洲PBAC經驗與台灣展望

洪在華

2007/06/08

Acknowledge



- Nathan CHEN
- Jason LEE
- Carol CHU
- Wennie GUO
- Tim CHIANG

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- What is HTA? HTA in other countries
- Introduction on Australia HTA
- Our view on future “HTA in Taiwan”

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Definition of HTA



- HTA: Health Technology Assessment
- HTA...

*is a structured analysis of a health technology, a set of related technologies, or technology-related issue that is performed for the purpose of providing input to a **policy decision** (US Congress, Office of Technology Assessment 1994)*

*HTA considers the effectiveness, appropriateness and cost of technologies. It does this by asking four fundamental questions: **Does the technology work, for whom, at what cost, and how does it compare with alternatives?** (UK National Health Service R&D Health Technology Assessment Programme 2003)*

HTA...is not the name of an organization

Material Nature of HTA



- Drugs
- Biologics: vaccines, blood products...
- Devices, equipment and supplies: CT scanners, drug eluting stent...
- Support systems: drug formularies, electronic patient record systems...
- Organizational and managerial systems: DRGs, clinical pathways...

Application



- Prevention: immunization, hospital infection control program...
- Screening: Pap smear, PAD...
- Diagnosis: x-ray for possible broken bone,
- Treatment: drugs for cancer pain, stent or no stent, BMS or DES...
- Rehabilitation: exercise program for post-stroke patients, assistive device for severe speech impairment...

Purposes of HTA



TO INFORM

- **Health Care Payers**, providers about whether technologies should be included in health benefits plans, reimbursement (how much to pay)
- **Clinicians** about the appropriate use of health care interventions
- **Health professional associations** about the role of a technology in clinical protocols or practice guidelines
- **Hospitals** about decisions regarding technology acquisition and management
- **Lawmakers and political leaders** about policies concerning technological innovation, research and development, regulation, payment and delivery of health care
- **Investors and companies** concerning venture capital funding, acquisitions and divestitures, and other transactions concerning health care product and service companies

HTA Organization



Country	Agency
Australia	PBAC
Canada	CADTH
UK	NICE
Scotland	SMC
Korea	HIRA
Germany	IQWiG (is obliged to look at HE data)
France	HAS (evidence based medicine)

小 結



- HTA不是一個組織架構的名稱，是一個評估醫療科技的科學方法
- HTA \neq cost-effectiveness analysis
- HTA的範圍、用途可以很廣，不同的單位，規格可以不同
 - 政府單位：screening program的評估，組織架構，評估方法
 - 保險單位：新藥是不是值得給付，付多少，要訂給付規定嗎？現有的品項要不要重新檢討？
 - 醫院：新的醫療技術與現有的比較，要不要引進新的醫療技術，clinical pathway的制定
- 組織架構，評估方法的導入，漸進式/一次到位？那一種比較合適？

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History of PBAC



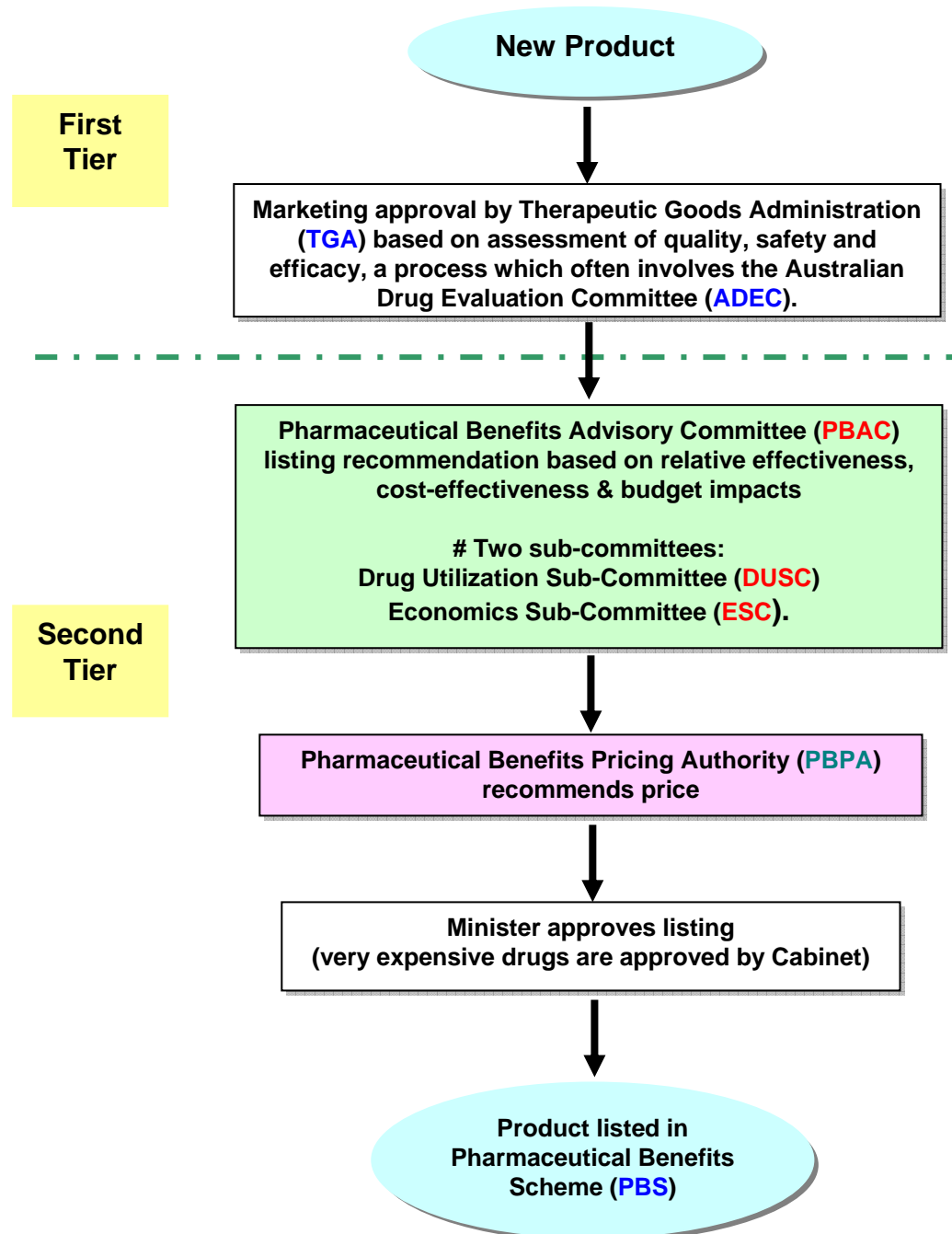
- 1944 Pharmaceutical Benefits Act – a person “shall” not be disqualified from receiving any pharmaceutical benefit by reason of his sickness having been caused by his own misconduct
- 1947 – Formulary Committee – Director General of Health (Chair) & 6 others: 3 MDs, 2 pharmacists and 1 pharmacologist
- 1949 scheme to provide 139 “life-saving and disease preventing drugs”
- 1953 National Health Act – PBAC formed as an independent statutory committee but not authority ie it advises the minister
- In 1987, the National Health Act was amended to require the PBAC to take cost-effectiveness into account
- In 1990, advice from the PBAC was based solely on the criteria of effectiveness and safety relative to existing drugs
- From 1993, National Health Act the PBAC must also compare the cost effectiveness of new drugs relative to alternative therapies
- In 2002, Guidelines for the pharmaceutical industry on preparation of submissions to the PBAC
- In 2006, Guidelines for the pharmaceutical industry on preparation of submissions to the PBAC (Draft for consultation)

Basic Principles of PBS



- The Minister may declare a drug listed on the PBS, but only after the **Therapeutic Goods Administration (TGA)** approval and under s101(4) after the **Pharmaceutical Benefits Advisory Committee (PBAC)** recommends it.
- The **Pharmaceutical Benefits Pricing Authority (PBPA)** negotiates price with manufacturer.

Part VII section 85 of the National Health Act 1953



The Pharmaceutical Benefits Advisory Committee (PBAC)



- An independent expert body whose membership includes doctors, other health professionals and a consumer representative, recommends new drugs to be listed.
- Role :
 - Recommends drugs and medicinal preparations to the Minister for Health for funding under the Pharmaceutical Benefits Scheme (PBS).
 - Recommends vaccines for funding under the National Immunisation Program (since 2006).
 - Advises the minister and the Pharmaceutical Benefits Pricing Authority about cost-effectiveness ('value for money').
 - Recommends maximum quantities and repeats on the basis of community use, and any restrictions on the indications where PBS subsidy is available.
 - Regularly reviews the list of PBS items.
 - Advises the minister about any other matters relating to the PBS.

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Economics Sub-Committee (ESC) of the PBAC



- The Pharmaceutical Benefits Advisory Committee (PBAC) established the ESC in December 1993 under section 101A of the National Health Act 1953 to:
 - review and interpret economic analyses of drugs submitted to the PBAC;
 - advise the PBAC on these analyses; and to
 - advise the PBAC on technical aspects of requiring and using economic evaluations.

PS: The members include clinicians, clinical epidemiologists, health economists, biostatisticians and clinical pharmacologists. As part of its terms of reference, ESC is also responsible for revisions of the guidelines

Drug Utilisation Sub-Committee (DUSC) of the PBAC



- The Pharmaceutical Benefits Advisory Committee (PBAC) established the DUSC in 1988 under section 101A of the National Health Act 1953 to:
 - collect and analyse data on drug utilisation in Australia for use by the PBAC;
 - make inter country comparisons of drug utilisation statistics; and to
 - assist in generating information relating to rational use and prescribing of medicines.
 - **Budget Impact Evaluation**

The DUSC secretariat is responsible for publishing the Australian Statistics on Medicines on an annual basis.

PS: The members have a broad range of relevant expertise and mainly come from organisations interested in the evaluation of drug utilisation.

The Pharmaceutical Benefits Pricing Authority (PBPA)

- An independent non-statutory body
- Role :
 - To review the prices of products supplied under the Pharmaceutical Benefits Scheme (PBS)
 - To recommend prices for new items are recommended for listing on the PBS

For pricing reviews, the Pricing Authority currently meets three times per year, in line with the three meetings per year of the PBAC. PBAC meetings are held in March, July and November, PBPA meetings are held in April, August and December.

Factors considered by the PBPA



- ✓ PBPA comments on clinical and cost effectiveness aspects of items
- ✓ The prices of alternative brands of a drug
- ✓ Comparative prices of drugs in the same therapeutic group
- ✓ Costs information, when provided by the supplier of estimated by the PBPA
- ✓ Prescription volumes, economics of scale and other factors such as expiry dating, storage requirements, product stability and special manufacturing requirements;
- ✓ Level of activity being undertaken by the company in Australia, including new investment, production, research and development;
- ✓ Prices of the drug in reasonably comparable overseas countries;
- ✓ Other relevant factors which the applicant company may wish the PBPA to consider; and
- ✓ Any directions of the Minister

How are products listed on PBS?



- Major submissions
 - Executive Summary
 - Section A, (requested listing)
 - Section B, (key clinical evidence)
 - Section C, (translation of evidence to requested listing)
 - Section D, (economic analysis)
 - Section E, (financial implications)
 - Section F, (other important factors e.g. QUM)
- Minor submissions
 - e.g. new formulation, new brand or change in restriction
 - No specific format
 - Economic evaluation not required
 - Not usually reviewed by PBAC subcommittees
 - Deadline approx. 6 weeks after major submissions

What data do the PBAC receive?



- Sponsor's submission
- PBAC secretariat overview
- Executive summary from submission
- ADEC minutes
- TGA delegate's summary
- PES (Pharmaceutical Evaluation Section)/DUSC secretariat evaluations
- Pre-subcommittee responses by the sponsor
- Advice from ESC, DUSC and RWG (Restriction Working Group)
- Pre-PBAC response by sponsor
- Product Information document
- Data on PBS listing of related drugs

Conduct of PBAC meeting

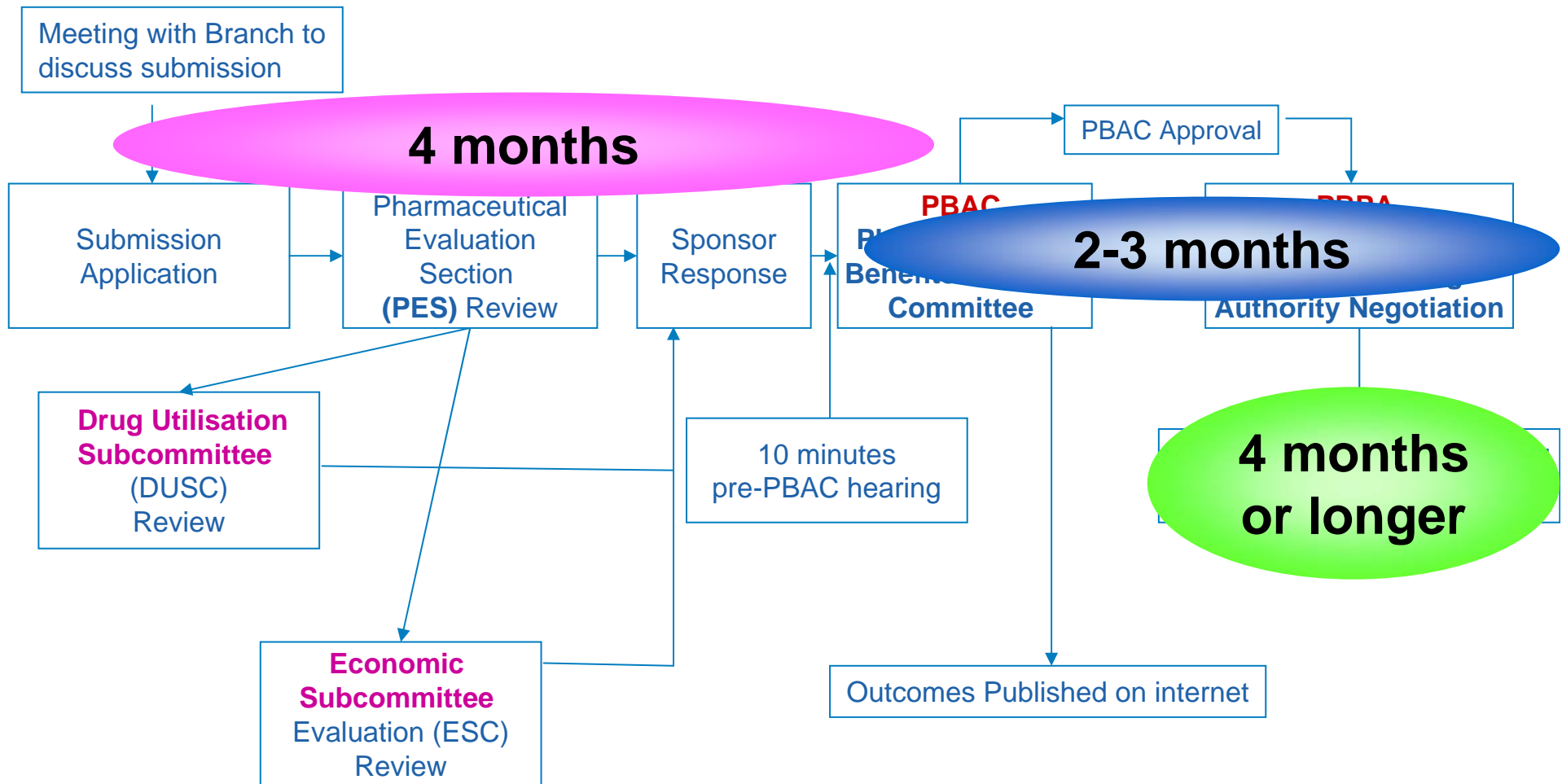


- Two discussants (reviewers) for each item
- First discussant summarises submission and ends with draft decision
- Usually by reference to ESC advice
- Second discussant agrees or adds
- Agenda item open for general discussion
- Formal vote taken, if necessary
- Companies also have an opportunity to present to the committee for 10 minutes

The Reimbursement Process (major submissions)



The existing reimbursement process is complex and lengthy



PBAC process

PBPA process

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PBAC submission process

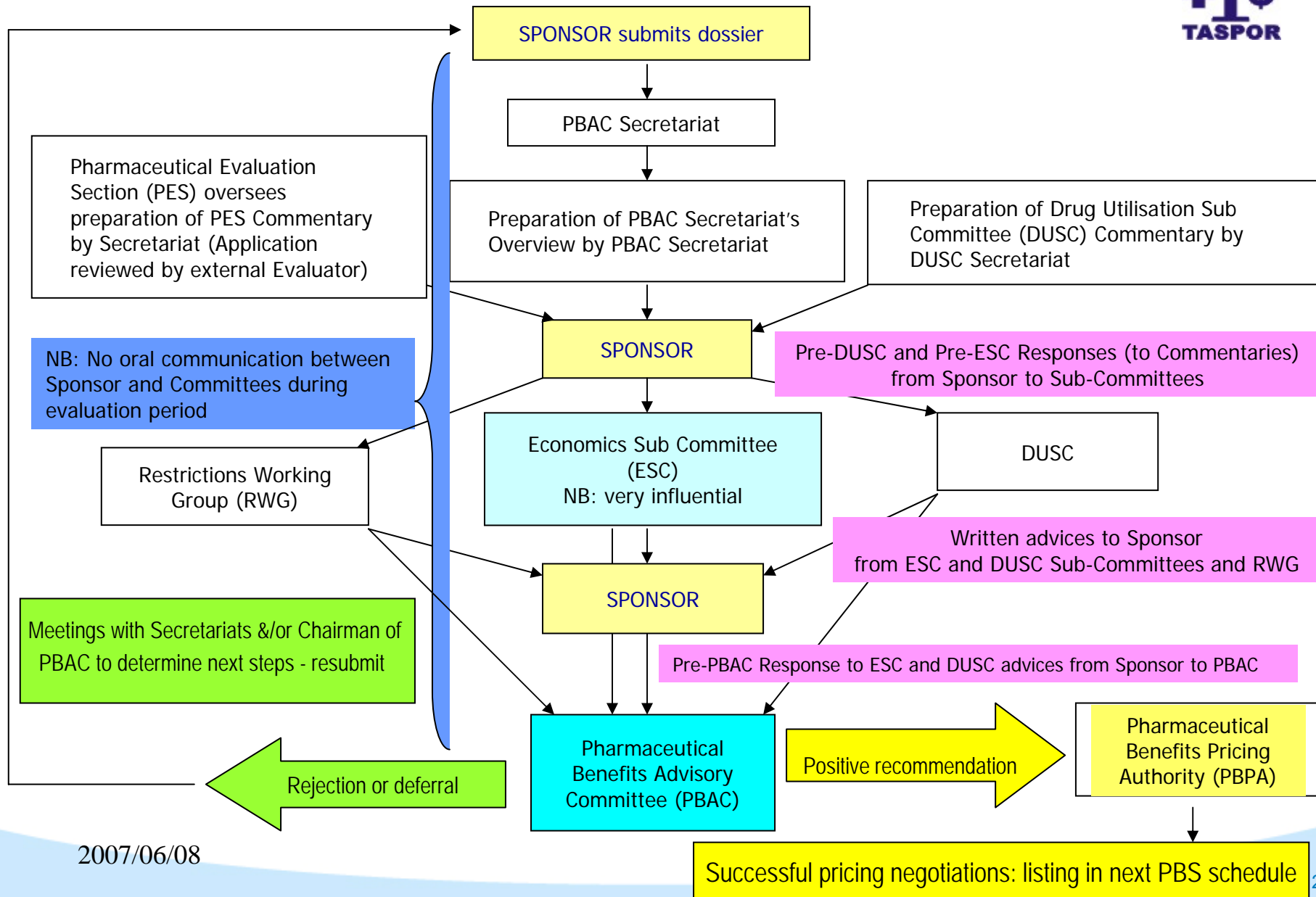


Table: Timeline of PBAC procedures



Action or event	Time relative to PBAC meeting
<ul style="list-style-type: none"> TGA delegate's overview/advice to ADEC and/or ADEC resolution and/or TGA registration granted 	
<ul style="list-style-type: none"> Cut-off date for major submissions to department 	17 weeks before
<ul style="list-style-type: none"> Cut-off date for minor submissions to department 	11 weeks before
<ul style="list-style-type: none"> Departmental papers to sponsors 	6 weeks before
<ul style="list-style-type: none"> Sponsor's pre-subcommittee response to department 	5 weeks before
<ul style="list-style-type: none"> Meeting of subcommittees 	4 weeks before
<ul style="list-style-type: none"> Subcommittee papers to sponsors 	2 weeks before
<ul style="list-style-type: none"> Sponsor's pre-PBAC response to department 	1 week before
<ul style="list-style-type: none"> PBAC meeting 	
<ul style="list-style-type: none"> Verbal advice to sponsor 	half a week after
<ul style="list-style-type: none"> Written advice to sponsor 	3 weeks after
<ul style="list-style-type: none"> Publication of PBAC outcomes on departmental website 	6 weeks after
<ul style="list-style-type: none"> PBAC ratified minutes to sponsor 	10 weeks after
<ul style="list-style-type: none"> Publication of public summary document on departmental website 	16 weeks after
<ul style="list-style-type: none"> 2007/06/08 Publication of public summary document (first time rejections) 	18 weeks after

After positive PBAC recommendation

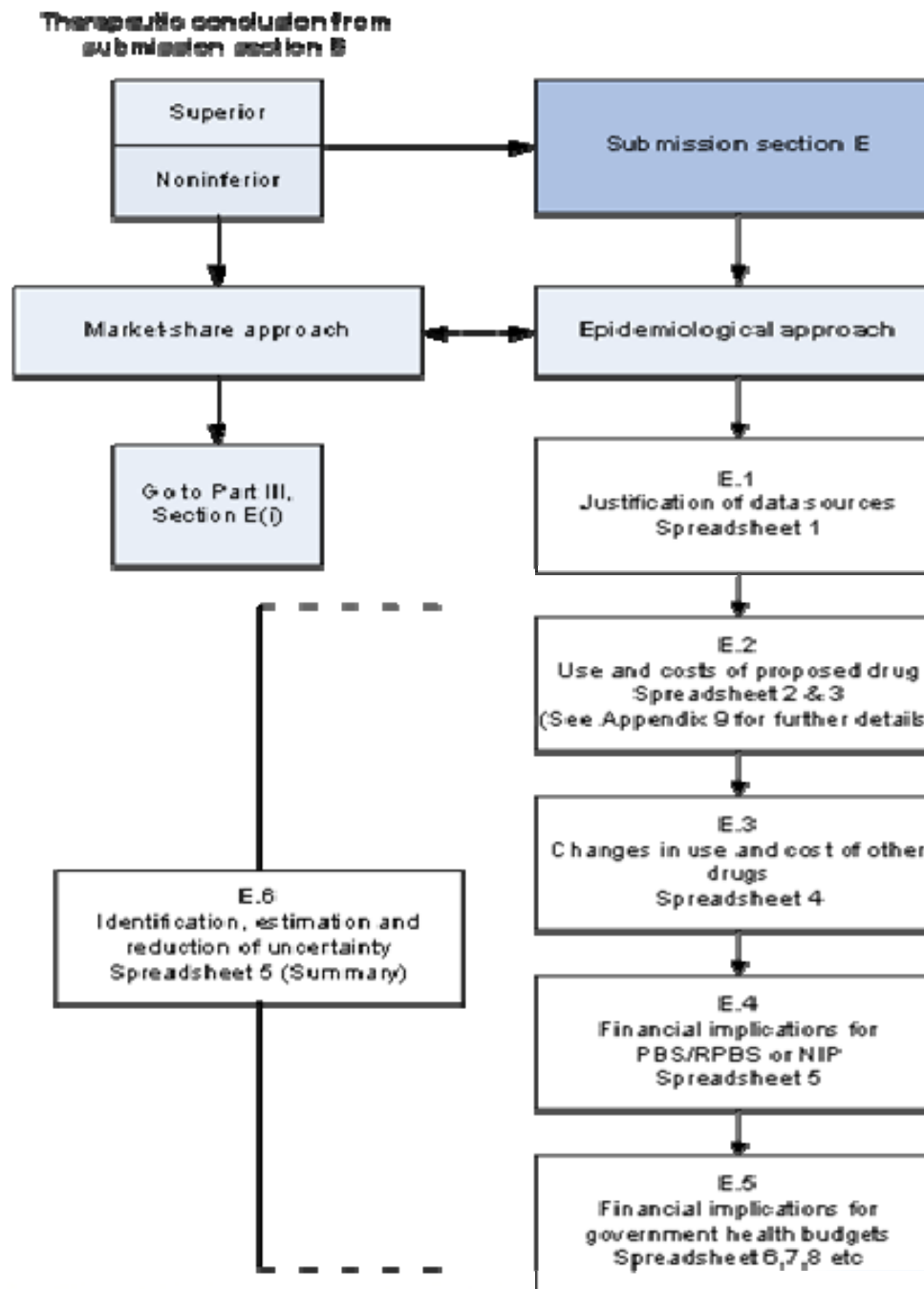
- **Pharmaceutical Benefits Pricing Authority (PBPA)** will review proposed listing (population, price)
- Items presented with draft recommendation and reasons, PBPA may/may not agree. A different price may be proposed.
- Risk share arrangements common where uncertainty over PBS cost and Deed of Agreement will be developed
- Cabinet approval required for PBS costs >\$10m (around NTD276m) (exchange rate 1 Aus ~ 26.7 NTD)

Estimated extent of use and financial implications



- Justification of the selection of sources of data
- Estimation of use and costs of the proposed drug
 - Estimate the number of patients with the medical condition targeted by the proposed drug, the number who would be eligible for the requested restriction and the number of patients likely to take the proposed drug.
- Estimation of changes in use and cost of other drugs
 - Drugs likely to be affected by the listing of the proposed drug
- Estimated financial implications for the PBS/RPBS or the NIP
 - Estimate the net financial implications for the PBS and the RPBS (or the NIP) in each year over five years
- Estimated financial implications for government health budgets
- Identification, estimation and reduction of uncertainty

Key information requests for budget impact



Budget Impact Analysis



$$f(\chi) - f(y) + f(\chi') - f(y')$$

χ : incremental drug cost of the proposed item

y : drug cost replaced by the proposed item
(the proposed item vs competitors)

χ' : incremental cost caused by managing major side effects, concomitant or preventive medicines of the proposed item

y' : cost saved due *to managing major side effects, concomitant or preventive medicines of the competitors*

Categories of data sources



<p>Disease epidemiological data (provide estimates of prevalence or incidence in the population)</p>
<ul style="list-style-type: none">•Australian case or mortality registers estimate the incidence or prevalence of a disease•Large, well-designed Australian studies estimate the incidence or prevalence of a disease•Australian national health surveys estimate the prevalence of a disease
<p>Pharmacoepidemiological data (provide estimates of treated prevalence)</p>
<ul style="list-style-type: none">•Surveys of the treated prevalence of the disease in Australia•Studies using utilisation databases, including PBS/RPBS data
<p>Market data</p>
<ul style="list-style-type: none">•Quantitatively describe the existing market•Estimate relative market shares•Estimate the impact of the requested PBS listing on current treatment paradigms based on similar previous listings.

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Key learning

- HTA 審核機關與藥品審核機關是兩個完全獨立的機構，審核流程亦然
- 1987立法，要求PBAC做決定時應考量cost-effectiveness (C/E), 1990鼓勵送C/E data，1993 C/E是送件之必要條件 – a reasonable learning phase
- 值不值得付/要不要付，與付多少錢分屬兩個不同的單位審核 公開、透明的審核流程與時間表值得學習
- 藥物的合理使用評估亦屬澳洲HTA的範疇
- Budget Impact analysis的範圍不應只侷限在 incremental drug cost

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Our view on future “HTA in Taiwan”

- Mission
 - Short term/ long term
- Organization
- Process
- Application Requirement

Future “HTA in Taiwan” - Objective/Mission



- Mission of “HTA in Taiwan”
 - Establish a structure to do scientific and evidence-based review on health technology-related issues, for the purpose of providing input to policy decision maker
 - Set the foundation for cost-effectiveness evaluation in 5-years time (ex. the right concept, methodology, data sources)

HTA is not a barrier

Future “HTA in Taiwan” - Objective/Mission



- Short term objective
 - As contact window between BNHI and sponsors
 - Training (both internal and external)
 - Build a transparent process and application requirement
 - Establish guidelines related to HTA (ex submission guideline, review guideline)
 - Give recommendation on whether to reimburse a new technology, should be based on evidence
 - Validate budget impact analysis for BNHI to make decision
 - Play as a coordinator to build the foundation for C/E evaluation (to collect or to analyze the current BNHI database, and to publish Taiwan-based Epi., Pharmaco-epidemiology and other data needed)

Periodic communication meeting with stakeholders is necessary.

Future “HTA in Taiwan” - Objective/Mission



Long term objective

- Develop an independent organization and continuous training program (more experienced sponsors and reviewers)
- A reference threshold for Taiwanese decision maker
- Develop Taiwan specific budget impact model, to take into consideration “value of medicine” , instead of incremental drug cost only
- Cost-effectiveness evaluation, Taiwan adaptation
- Initiate Drug Utilization Program (合理的使用)

Periodic communication meeting with stakeholders is necessary.

Future “HTA in Taiwan” - Organization



- 醫藥科技評估組
 - A proper name, instead of HTA
 - An independent organization
 - Independent review, separate from NDA approval process (budget impact should not be a critical factor in NDA approval)
 - Build connection with other HTAs, in line with international rule of game (<http://www.htai.org>)

Future “HTA in Taiwan” - Process



- Transparent review process and timeline

Events	Time
• BoPA informs sponsor on review result after Drug Advisory Committee meeting	
• Pre-submission meeting with HTA	- 12-15 weeks
• Reimbursement dossier submitted to BNHI	0 week
• BNHI transfer submissions dossier to HTA	1 week
• Preliminary review (any missing document), response to sponsor	1 week
• Review after Sponsor's response – verbal/written advice to sponsor	16 weeks
• HTA give recommendation to BNHI and to be discussed at BNHI Drug Advisory Committee	4 weeks
• Written response to sponsor	2 weeks
• Price negotiation	4 weeks
• Publish price on BNHI website	4 weeks

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範例，僅供參考

Current application requirement



- Application form
 - Basic information of the drug
- Ten reference price
 - USA, UK, Japan, Canada, Germany, France, Belgium, Sweden, Switzerland , Australia
- Competitors (not only clinical comparison, differentiate with competitors, but also competitor's price)
- C/E table (focused on clinical trial results)
- 5-year budget impact
- PE (optional)

2007/06/08 Future: evidence-based dossier and review

Available document for reference



台灣藥物經濟學評估方法指南

<http://www.taspor.org/>