HEALTH CARE RESEARCH CONSULTANCY

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JOB NO: 07/03582

PROJECT PE

AN EXPLORATORY SURVEY ON

PHARMACO-ECONOMIC EVALUATIONS

- SUMMARY REPORT -

CONFIDENTIAL

PREPARED FOR: IMSA

PREPARED BY: VBH HEALTH CARE RESEARCH CONSULTANCY
MAY 2007

1 RESEARCH MOTIVATORS

IMSA (Innovative Medicines of South Africa) put together a task team to explore perceptions about pharmaco-economic evaluations (PE) in South Africa. The team devised a questionnaire to meet this goal, with the initial intention being that the team would handle the distribution of the questionnaires / the collection of the required data.

However, concerns were expressed that respondents might refuse to participate in the survey if they felt that the information was being collected by someone with a vested interest in the topic. The decision was thus taken to have an independent third party collect the information to meet the research objectives.

VBH was approached to assist in this regard and contained in this document are the findings from this survey.

2 RESEARCH OBJECTIVES

2.1 Overall Aim

The overall aim of this research project was summarised as:

> "*To assess the primary conceptual motive and perceived role of PE assessment in South Africa and to establish how PE evaluations are currently applied in South Africa*".

3 RESEARCH STRUCTURE

3.1 Design and Methodology

The information required to meet the research objectives was obtained via a self completion questionnaire. Client identified target respondents and VBH interviewers approached these people and invited them to participate in this survey. Questionnaires were emailed, faxed or personally delivered to people willing to participate in the survey.

The questionnaire was largely structured, with respondents indicating the extent to which they agree with / the importance of batteries of statements pertaining to PE and its applicability in the South African context, or rating the importance of various issues.

3.2 Coverage and Sample

Client provided the names of 44 people for inclusion in the sample, a total of 26 returns ultimately being received.

3.3 Fieldwork

Fieldwork for this survey was conducted from 29 March to 11 May 2007.

3.4 The Questionnaire

A copy of the questionnaire used for this survey can be found in Appendix I of this report.

4 RESEARCH FINDINGS

The data from this survey was subjected to very simple analyses:

Measuring level of agreement / relevance / importance

- where respondents had to indicate the extent to which they agree with a battery of statements, both the number of respondents and the proportion of the sample indicating that they "strongly agree", "agree", "disagree" and "strongly disagree" was calculated. These figures have been shown in the tables that follow, along with a top two box score (i.e. the proportion strongly agreeing / agreeing) and a bottom two box score (i.e. the proportion disagreeing / strongly disagreeing). In addition, by assigning the following numeric value to the rating scale, 1 = strongly disagree, 2 = disagree, 3 = agree and 4 = strongly agree, an average score out of 4 was also calculated as an indicator of level of agreement (the higher the score, the higher the level of agreement). This score has also been shown for each statement

- the same principle as above was applied to data captured when respondents had to indicate the level of relevance / importance attached to a battery of statements.

In the tables that follow, attributes have been listed from highest to lowest level of agreement / most to least importance, etc, based on the top two box score.

Ranking statements in terms of overall importance

- in some instances, respondents were asked to rank a battery of statements in terms of overall importance. Here, the number of respondents ranking each attribute in each position was calculated, and based on this, a weighting figure was calculated. This weighting figure shows the relative importance of one attribute over the next and dictates the overall rank order

Open-ended questions

- as the sample was so small and responses to the open-ended questions were generally fragmented, individual verbatim quotes have simply been listed.

4.1 The Applicability of PE Evaluation in South Africa

The extent to which the respondents agree with given statements relating to the applicability of PE evaluation in South Africa is as follows:

BASE = 26	STRONGLY AGREE	AGREE	DISAGREE	STRONGLY DISAGREE	NO OPINION	Top two box score % STRONGLY AGREE/AGREE	Bottom two box score % DISAGREE/ STRONGLY DISAGREE	AVE SCORE OUT OF 4
PE should be mandatory for ALL new biologicals	17 65%	8 31%	1 4%	- -	- -	96%	4%	3.6
All health technologies, including devices, techniques, etc. should be subjected to PE	14 54%	11 42%	1 4%	- -	- -	96%	4%	3.5
PE should not be done in isolation and should be part of a bigger health economics assessment, where all interventions and expenses are evaluated	12 46%	12 46%	2 8%	- -	- -	92%	8%	3.4
PE should be mandatory for ALL newly marketed medicines	13 50%	10 38%	3 12%	- -	- -	88%	12%	3.4
PE assessment should be mandatory for drugs indicated for the treatment of PMB conditions	11 42%	11 42%	2 8%	2 8%	- -	85%	15%	3.2
PE should be mandatory for ALL state tender products	11 42%	10 38%	3 12%	1 4%	1 4%	81%	15%	3.2
PE should be mandatory for ALL existing products	4 15%	8 31%	11 42%	3 12%	- -	46%	54%	2.5
PE assessment should ONLY be mandatory for drugs within selected therapeutic areas/categories	- -	4 15%	13 50%	8 31%	1 4%	15%	81%	1.8
PE should be mandatory ONLY for new generic drugs	1 4%	2 8%	6 23%	14 54%	3 12%	12%	77%	1.4

4.2 The Role of PE Evaluation in South Africa

The extent to which the respondents agree with given statements relating to the role of PE evaluation in South Africa is as follows:

BASE = 26	STRONGLY AGREE	AGREE	DISAGREE	STRONGLY DISAGREE	NO OPINION	Top two box score % STRONGLY AGREE/AGREE	Bottom two box score % DISAGREE/ STRONGLY DISAGREE	AVE SCORE OUT OF 4
PE provides value in assessing health outcomes in disease management	7 27%	14 54%	3 12%	- -	2 8%	81%	12%	3.2
PE provides an ethical guidance for healthcare policy formulation	6 23%	13 50%	7 27%	- -	- -	73%	27%	3.0
PE provides a guide in setting clinical practice guidelines	5 19%	11 42%	8 31%	1 4%	1 4%	62%	35%	2.8
PE ensures equitable distribution of payers' health care resources	5 19%	11 42%	8 31%	1 4%	1 4%	62%	35%	2.8
PE provides an explicit benchmark for price setting of new drug entities	3 12%	13 50%	8 31%	1 4%	1 4%	62%	35%	2.7
PE is an effective means to control drug costs	1 4%	15 58%	8 31%	2 8%	- -	62%	38%	2.6
PE evaluation of a drug positively influences doctors' prescribing patterns	2 8%	12 46%	8 31%	2 8%	2 8%	54%	38%	2.6
PE is the most important part of health economic evaluations	2 8%	12 46%	10 38%	1 4%	1 4%	54%	42%	2.6
PE is the easiest type of cost-effectiveness intervention a payer can make	- -	12 46%	8 31%	2 8%	4 15%	46%	38%	2.5
PE plays a crucial role in setting payers' budget caps	2 8%	8 31%	15 58%	- -	1 4%	38%	58%	2.5

4.2.1 Other Roles PE could Play in the South African Health Care Arena

Responses obtained when asked what other role they see PE evaluation playing in the South African health care arena are as follows:

Figures in brackets = No. of mentions (Base = 26)

- Nothing further specified (13)

- A very limited / minimal role (3)

  - makes doctors aware that added benefit comes at a price
  - to see the true cost of health technologies, medicines, etc in the SA market with the SA population and local prevalence, with the local cost of hospitalisation, special investigations, etc. But at this point in time new entities are seldom compared to current treatment with local costs
  - there is a lack of understanding and effective application
  - PE basics need to be implemented before moving forward
  - must assist in ensuring local studies and local data availability.

Other comments received at this time as to the role that PE evaluations could play in the South African health care arena are as follows:

- Better utilisation of limited resources, driving down the cost burden of medical aid premiums, increasing choice of drug care option, understanding compromise from the patient's perspective

- All given statements regarding the role of PE are important but it will only add value if applied correctly

- Negotiate better prices from a third party funding perspective with pharmaceutical manufacturer

- Eliminates any potential possibility for perverse incentives as well as ensures that EBM principles are being adhered to

- Currently PE is used as a hurdle rather than a means to obtain more information for critical decision making. It can be used as a vital means to direct product positioning. It can inform healthcare risk management

- The principles that underpin PE studies are premised on the application of explicit economic rules with a view to informing clinical decision-making and resource allocation. These economic rules are based on well established (normative) or philosophical principles that inform welfare economics. It is critical that when conducting PE studies one should fully appreciate the extent to which one is deviating from these principles. In my view, pharmaco-economics lends credence to the importance of applying clear and transparent principles when making decisions in health care. One wishes that such an approach could resonate through the entire health care system at all levels of decision making

- To be able to know outcomes of drugs / technologies and of course the cost to achieve that

- Evaluation of other costs e.g. technical equipment, prostheses and of course hospital and oncology costs

- The only role it plays and will play is to justify the cost of expensive drugs for the multinational companies.

4.3 Evaluation of PE Submissions

The extent to which the respondents agree with given statements relating to the evaluation of PE submissions is as follows:

BASE = 26	STRONGLY AGREE	AGREE	DISAGREE	STRONGLY DISAGREE	NO OPINION	Top two box score % STRONGLY AGREE/AGREE	Bottom two box score % DISAGREE/ STRONGLY DISAGREE	AVE SCORE OUT OF 4
PE submissions should be evaluated by Managed Care Companies (MCOs)	8 31%	11 42%	3 12%	3 12%	1 4%	73%	23%	3.0
PE submissions should be evaluated by Pharmaceutical Benefit Managers (PMBs)	4 15%	14 54%	2 8%	4 15%	2 8%	69%	23%	2.8
PE submissions should be evaluated by Independent Private Consultants	6 23%	11 42%	7 27%	1 4%	1 4%	65%	31%	2.9
PE submissions should be evaluated by Government	8 31%	8 31%	5 19%	2 8%	3 12%	62%	27%	3.0
PE submissions should be evaluated by the Council of Medical Schemes	5 19%	10 38%	7 27%	1 4%	3 12%	58%	31%	2.8
PE submissions should be evaluated by Medical Scheme Administrators	2 8%	11 42%	7 27%	5 19%	1 4%	50%	46%	2.4
PE submissions should be evaluated by DUR-pharmacists	2 8%	8 31%	10 38%	4 15%	2 8%	38%	54%	2.3

4.4 Standardisation of Requirements for PE submissions

The extent to which the respondents feel that it would be relevant to incorporate procedures from each of these sources into a South African guideline is as follows:

BASE = 26	EXTREMELY RELEVANT	SOMEWHAT RELEVANT	NOT VERY RELEVANT	IRRELEVANT	DON'T KNOW	Top two box score % EXTREMELY / SOMEWHAT RELEVANT	Bottom two box score % NOT VERY RELEVANT / IRRELEVANT	AVE SCORE OUT OF 4
Local / South African Expert Opinion	15 58%	8 31%	1 4%	1 4%	1 4%	88%	8%	3.5
National Institution for Health and Clinical Excellence (NICE) from the United Kingdom (UK)	14 54%	9 35%	3 12%	- -	- -	88%	12%	3.4
Canadian Co-ordinating Office for Health Technology Assessment (CCOHTA) guidelines	8 31%	14 54%	- -	- -	4 15%	85%	-	3.4
Australian Pharmaceutical Benefit Advisory Committee (PBAC)	10 38%	11 42%	3 12%	- -	2 8%	81%	12%	3.3

4.5 Preferred Health Outcome Measures for PE submissions

The health outcome measures below have been listed from most to least important, based on an importance ranking exercise:

BASE = 26	No respondents ranking in each position 1 - 5					WEIGHT	RANK ORDER
	RANK 1	RANK 2	RANK 3	RANK 4	RANK 5		
Cost-effectiveness	14	3	5	3	-	103	**1**
Quality Adjusted Life Years (QALYs)	3	8	2	8	4	73	**2**
Cost Utilities	4	3	8	4	6	70	**3**
Cost Minimization	2	5	7	5	6	67	**4**
Budget Impact	2	6	3	5	9	62	**5**

4.5.1 Respondents' Definitions of Cost-effectiveness

Respondents were asked to explain how they would define cost effectiveness and what factors they would take into consideration when assessing cost-effectiveness. Three respondents gave no response here, whilst comments received from the other respondents were as follows:

- ICERS that could be incorporated into QALYs
- Extra costs needed to obtain additional benefit. Budget impact is important – this will reflect the number expected to benefit
- Cost-effectiveness is value for money, but it doesn't imply affordability and doesn't necessitate / ensure reimbursement of the specific product. What is cost-effective for an individual patient is not necessarily cost-effective for a medical scheme population
- Cost-effectiveness is not "cheap". It is determining for that particular "cost" what health outcome is produced. Also, how this relates to long term costs / total costs i.e. hospitalisation as opposed to looking at the "cost of the drug" only
- It should be in relative terms – against a comparator of reasonable clinical positioning. A "threshold" is required for consideration above which a drug isn't cost-effective. Factors taken into account for a funder would be costs and benefits from a third party payer's perspective
- Cost-effectiveness analyses include a measure of effectiveness that should preferably be QALYs. This is also the international standard but is sometimes difficult to implement as most of the applications go to the funders who are not responsible for indirect costs. This is of course wrong, as health care costs influence the society as a whole
- Cost-effectiveness analysis is a technique for comparing the relative value of various clinical strategies in terms of cost and effectiveness. Factors considered when assessing cost-effectiveness include a comparison against the "golden standard". Use local cost data. We do the analysis from the right perspective (the funder's perspective) for the purpose of the study. Are the assumptions made acceptable? Was sensitivity analysis done adequately? Who funded the analysis? Are the studies published in accredited peer-reviews journals?
- I have assumed cost benefit definition: looking at all the costs that are incurred in the programme

- Cost to measure clinical consequences of different intervention that can be measured – e.g. mmHg reduction of BP

- Confidence with which scientific assumptions can be accepted is most important when assessing any type of PE analysis

- Cost-effectiveness is "value for money" i.e. does the benefit or the additional benefits above other treatment justify the cost (or the additional cost above that of other treatment)? Factors considered when assessing cost-effectiveness include:

    - Effect of the intervention (e.g. BP reduction, mortality reduction) versus the effect of other intervention (either the same drug class or other treatment)

    - Cost of intervention as compared to the cost of other treatment / intervention

- A product is cost-effective when the cost of the intervention is justified by the relative benefit compared with an appropriate reference / standard care. Factors considered when assessing cost-effectiveness include:

    - Disease burden

    - Numbers needed to treat (NNT)

    - Relevance of selected comparator

    - Definition of payment thresholds

    - Absolute benefit as determined by appropriately conducted clinical trails

    - Actual price of the intervention

- Value (therapeutic effect) per rand spent. Therapeutic effect and the clinical significance of such effect versus SEP of dose required to achieve such an effect

- Cost-effectiveness is a phrase used broadly to define PE but in fact is one of 4 types of HE. It refers to 2 or more interventions with outcomes of similar measure (e.g. life years) but different cost value (i.e. in rands)

and different outcomes (e.g. different number of life years). The most important factors are outputs i.e. clinical outcomes and inputs i.e. direct costs

- Something is cost-effective if the outcome of a specific treatment is measurable in terms of clinical efficacy as well as financial affordability
- Cost-effectiveness is based on estimating the incremental costs and benefits of a particular treatment compared to its next best treatment in the context of a particular setting and with access to an explicit threshold. Factors to be considered include:
    - Evidence of clinical effectiveness
    - Clinical effectiveness of alternative treatment
    - Epidemiological data
    - Unit cost (if applicable)
    - Cost of alternative treatment
    - Explicit threshold
    - Preference-based health related quality of life measures
- Efficacy of drug in treating the condition as measured in quality of life scores and QALYs
- Does acquisition cost of the drug justify outcome / benefits with minimal monitoring and follow-up?
- Cost-effectiveness can only be defined as a number that is less than a threshold that is accepted as the threshold cost-effectiveness. In our research, we have calculated thresholds for both the public and private sector and a CUA that produces results below this threshold might be seen as cost-effective. We propagate a five level approach to cost-effectiveness, namely clinical effectiveness, value for money, budget impact, finding options and marketing impact
- The therapeutic cost of achieving a certain predefined clinical outcome of two drugs of the same generic molecule. Price, effectiveness, utilisation and safety and taken into account when assessing cost-effectiveness
- Cost effectiveness is the relationship of cost to a single common effect. It is often used by decision makers to compare different resource allocation

options in like terms, i.e. quantitatively and objectively. Factors taken into account depend on the perspective of the study. There are very few factors that always have to be taken into account

- Cost effectiveness expresses the value of an intervention as the ratio of its **effectiveness** (please note, not “efficacy”) to the resources utilised to produce these benefits. Technically, it assumes that there are significant differences among comparators as far as effectiveness is concerned

- Outcomes relative to expenditure. I have never had high regard for QALYs as this is open to manipulation

- The effect divided by the acquisition cost.

4.6 Critical Success Factors for Appropriate Implementation of PE Submissions

The importance attached to each factor is shown in the table below:

BASE = 26	EXTREMELY IMPORTANT	SOMEWHAT IMPORTANT	NOT VERY IMPORTANT	IRRELEVANT	DON'T KNOW	Top two box score % EXTREMELY / SOMEWHAT IMPORTANT	Bottom two box score % NOT VERY IMPORTANT / IRRELEVANT	AVE SCORE OUT OF 4
Transparency in the PE studies submitted by the manufacturer	25 96%	1 4%	- -	- -	- -	100%	-	4.0
Adequate resources and capacity for the review process	23 88%	2 8%	1 4%	- -	- -	96%	4%	3.8
Availability of local epidemiological data for South Africa	19 73%	6 23%	1 4%	- -	- -	96%	4%	3.7
Provision of formal training on PE	19 73%	6 23%	- -	1 4%	- -	96%	4%	3.7
Relevance of PE models to the South African population	16 62%	9 35%	1 4%	- -	- -	96%	4%	3.6
Availability of a central repository of data for the country	17 65%	8 31%	1 4%	- -	- -	96%	4%	3.6
Relevance of PE models to current treatment guidelines	14 54%	11 42%	1 4%	- -	- -	96%	4%	3.5
Relevance of PE models to the South African medical scheme population / medical scheme members	12 46%	12 46%	1 4%	1 4%	- -	92%	8%	3.3
Availability of centres of excellence for PE	17 65%	6 23%	3 12%	- -	- -	88%	12%	3.5
Availability of a legal framework that governs the application of PE in South Africa	9 35%	11 42%	6 23%	- -	- -	77%	23%	3.1

4.6.1 Ranking of Clinical Success Factors for Appropriate Implementation of PE Submissions

The factors below have been listed from most to least important, as calculated based on an importance ranking exercise:

BASE = 26	No respondents ranking in each position 1 - 10										WEIGHT	RANK ORDER
	RANK 1	RANK 2	RANK 3	RANK 4	RANK 5	RANK 6	RANK 7	RANK 8	RANK 9	RANK 10		
Adequate resources and capacity for the review process	10	6	2	1	0	2	0	1	2	2	196	**1**
Availability of local epidemiological data for South Africa	4	4	2	5	2	4	0	1	3	1	169	**2**
Relevance of PE models to the South African population	1	0	5	3	6	5	2	3	1	0	151	**3**
Transparency in the PE studies submitted by the manufacturer	4	2	1	1	5	1	7	2	2	1	147	**4**
Relevance of PE models to the South African medical scheme population / medical scheme members	2	4	1	2	2	6	4	1	4	0	147	**4**
Relevance of PE models to current treatment guidelines	0	1	6	3	5	3	4	2	0	2	147	**4**
Provision of formal training on PE	2	2	4	2	3	2	3	3	1	4	139	**7**
Availability of centres of excellence for PE	1	2	3	4	2	2	2	5	3	2	133	**8**
Availability of a legal framework that governs the application of PE in South Africa	2	1	1	2	1	1	2	5	7	4	103	**9**
Availability of a central repository of data for the country	0	4	1	3	0	0	2	3	3	10	98	**10**

4.7 Measures Considered in the Review of PE Submissions

The importance attached to each measure is shown in the tables below:

BASE = 26	EXTREMELY IMPORTANT	SOMEWHAT IMPORTANT	NOT VERY IMPORTANT	IRRELEVANT	DON'T KNOW	Top two box score % EXTREMELY / SOMEWHAT IMPORTANT	Bottom two box score % NOT VERY IMPORTANT / IRRELEVANT	AVE SCORE OUT OF 4
Therapeutic effects of the drug or technology assessed	24 92%	2 8%	- -	- -	- -	100%	-	3.9
Relationship between the treatment effects and costs (cost vs effects) of the drug or medical technology assessed	18 69%	8 31%	- -	- -	- -	100%	-	3.7
Relationship between the treatment effects and total economic cost associated with the use of the drug or medical technology under review	14 54%	12 46%	- -	- -	- -	100%	-	3.5
Impact of the cost of medicines or technology (that is under review) on total cost to the scheme (cost vs budget)	16 62%	9 35%	1 4%	- -	- -	96%	4%	3.6
Impact of the cost of medicines or technology (that is under review) onto the total healthcare budget for the country	9 35%	15 58%	1 4%	- -	1 4%	92%	4%	3.2

Continued below

BASE = 26	EXTREMELY IMPORTANT	SOMEWHAT IMPORTANT	NOT VERY IMPORTANT	IRRELEVANT	DON'T KNOW	Top two box score % EXTREMELY / SOMEWHAT IMPORTANT	Bottom two box score % NOT VERY IMPORTANT / IRRELEVANT	AVE SCORE OUT OF 4
Clinical guidelines as set by professional bodies	10 38%	13 50%	3 12%	- -	- -	88%	12%	3.3
Years of clinical experience with the medicine or new technology	8 31%	15 58%	3 12%	- -	- -	88%	12%	3.2
Standardised threshold levels for chronic conditions	8 31%	14 54%	4 15%	- -	- -	85%	15%	3.2
Impact of the cost of medicines or technology (under review) on the patient's out-of-pocket expenses	10 38%	12 46%	3 12%	- -	1 4	85%	12%	3.2
Standardised league tables for selected chronic conditions	4 15%	13 50%	8 31%	- -	1 4	65%	31%	2.7
Reputation and credibility of the manufacturer	4 15%	11 42%	7 27%	3 12%	1 4%	58%	38%	2.5
Cost of the medicine or new technology only	1 4%	13 50%	10 38%	2 8%	- -	54%	46%	2.5

4.7.1 Ranking of Measures Considered in the Review of PE Submissions

The factors below have been listed from most to least important, as calculated based on an importance ranking exercise:

BASE = 26	No respondents ranking in each position 1 - 12												WEIGHT	RANK ORDER
	RANK 1	RANK 2	RANK 3	RANK 4	RANK 5	RANK 6	RANK 7	RANK 8	RANK 9	RANK 10	RANK 11	RANK 12		
Therapeutic effects of the drug or technology assessed	14	1	4	1	1	1	1	0	1	1	1	0	258	**1**
Relationship between the treatment effects and costs (cost vs effects) of the drug or medical technology assessed	6	7	4	1	3	1	1	1	1	1	0	0	247	**2**
Relationship between the treatment effects and total economic cost associated with the use of the drug or medical technology under review	4	6	5	3	3	0	0	3	0	0	1	1	233	**3**
Impact of the cost of medicines or technology (that is under review) on total cost to the scheme (cost vs budget)	0	2	4	9	4	3	2	0	1	0	1	0	214	**4**
Clinical guidelines as set by professional bodies	1	5	2	4	2	2	2	2	2	2	1	1	192	**5**
Impact of the cost of medicines or technology (that is under review) onto the total healthcare budget for the country	1	1	3	3	3	4	3	2	3	1	0	2	177	**6**
Years of clinical experience with the medicine or new technology	0	0	2	1	4	4	2	6	4	1	2	0	154	**7**
Impact of the cost of medicines or technology (under review) on the patient's out-of-pocket expenses	0	2	0	0	2	5	6	3	3	2	2	1	147	**8**
Standardised threshold levels for chronic conditions	0	1	1	1	1	4	2	1	5	3	6	1	125	**9**
Cost of the medicine or new technology only	0	0	1	1	0	2	4	1	3	5	3	6	101	**10**
Standardised league tables for selected chronic conditions	0	1	0	0	3	0	1	4	0	4	5	8	91	**11**
Reputation and credibility of the manufacturer	0	0	0	2	0	0	2	3	3	6	4	6	89	**12**

4.8 Resources Required for Effective PE Evaluations

The extent to which the respondents agree that given statements apply to their organisations' systems that are in place for the evaluation of PE submissions is as follows:

BASE = 26	STRONGLY AGREE	AGREE	DISAGREE	STRONGLY DISAGREE	NO OPINION	% STRONGLY AGREE/AGREE	% DISAGREE/ STRONGLY DISAGREE	AVE SCORE OUT OF 4
When performing PE evaluations on new chemical entities, my company does so by engaging / discussing pertinent issues with the relevant company during the review process	7 27%	12 46%	4 15%	- -	3 12%	73%	15%	3.1
My organisation has relevantly qualified human resources to perform effective PE analysis	8 31%	10 38%	7 27%	1 4%	- -	69%	31%	3.0
When performing PE evaluations on generic medicines, my company does so by engaging / discussing pertinent issues with the relevant company during the review process	4 15%	11 42%	3 12%	2 8%	6 23%	58%	19%	2.9
My organisation effectively performs PE	8 31%	5 19%	10 38%	1 4%	2 8%	50%	42%	2.8
My organisation has a documented policy on how to perform PE evaluations	6 23%	7 27%	8 31%	3 12%	2 8%	50%	42%	2.7
My organisation has agreed upon time frames for completion of PE evaluations	5 19%	8 31%	8 31%	3 12%	2 8%	50%	42%	2.6
My organisation has a guideline for the communication of PE evaluations results to all relevant parties	3 12%	9 35%	8 31%	4 15%	2 8%	46%	46%	2.5

4.9 Closing Comments

The final question allowed respondents the opportunity of recording any additional comments they wished to add about PE evaluation, the assessment thereof and the role that this plays in the South African health care arena. Eleven respondents made no further comment. Those who did comment noted the following:

- PE is poorly understood by most role players in the industry
- PE needs to be explained more widely and the underlying principles made clear for decision makers
- At this point PE plays little role as the new entities are seldom compared to current treatment with local costs
- The importance of PE evaluations cannot be stressed enough. However, we require a “central” or “industry” body doing these and the information made available to all stakeholders who can then utilise the info and convert it to their requirements. Duplication needs to be avoided. Who this body should be – non-biased / independent made up of key players and well respected KOLS to get one version of the truth
- The lack of local data clearly means that current best efforts in PE and HE must be a mix of best available and most useful data to input. While this is a limitation, it should not alter the requirements for this process for all new pharmaceutical and technologies
- PE is an additional cost to the system and will flounder without adequate funding. Generic medicines are only used for comparators and seldom / never for initial analyses
- I think that PE evaluations should play a far more extensive role than currently is the case. SA needs more trained people to perform and evaluate PE analysis
- The value of PE in assisting with the allocation of scarce resources is sometimes overstated; it is only one tool and must be seen as that. Although PE can assist in setting fair drug prices, it is a double-edged sword in that pharmaceutical manufacturers can also exploit it to their advantage

- In the current setting, PE evaluations are used to justify the high cost of expensive interventions. Until affordability within the South African context is taken into account and appropriate thresholds of “willingness to pay” are defined, PE analyses will not add significantly to the manner in which funding decisions are made within the South African private health care sector

- It would be ideal if private funders can make use of a mutual PE process so that reimbursement decisions can be standardised between funders

- We require open dialogue and many more resources and training. PE is critical in future decision making

- Although it has improved a lot, I don’t believe that enough emphasis is being placed on the value of PE in health care in SA at present

- PE is currently not performed at all

- Clinical outcomes data is difficult to obtain especially with new molecules. One must use overseas data if available, but then the problem is that this is not representing SA conditions

- Transparency on decision-making policies from funders and other decision makers who are making use of PE evidence or who require the submission of PE evidence is required. Unless such transparency can be provided, PE analyses will remain hurdles and as such, “ grudge” research obligations!

- Your questions are framed in a way which does not leave much room for disagreement.

APPENDIX I

QUESTIONNAIRE

HEALTH CARE RESEARCH CONSULTANCY

PO Box 1979 Northcliff 2115 Tel/Fax (011) 467 6569/71 Reg No.CK90/08527/23

Project PE – An exploratory survey on pharmaco-economic evaluations

VBH Health Care Research Consultancy, a specialist health care marketing research company, has been commissioned to conduct a survey aimed at gaining insight into the views and perception of the health care industry on the implementation of pharmaco-economic evaluation as a tool for making decisions on the reimbursement of medicines and medical technologies.

The objective of this study is to assess the **primary conceptual motive** and **perceived role** of pharmaco-economic assessment in South Africa. In addition, we would also like to establish how pharmaco-economic evaluations are currently **applied** in South Africa, as well as to ascertain areas / resources required to execute pharmaco-economic evaluations in various sub-sectors in South Africa.

This survey is being conducted on behalf of Innovative Medicines South Africa (IMSA). IMSA represents six research-based pharmaceutical companies in South Africa and the organisation believes in constructive engagement with, and the enhancement of understanding between, role-players in the health sector.

As a stakeholder intrinsically involved in the formulation of health care and funding policies and practices, your views and ideas on this topic will add value in harmonising interaction and communication between the payers and the manufacturers.

There is an incentive payment as a token of our appreciation for the time that you spend completing this questionnaire. On receipt of your completed questionnaire, we will contact you to arrange payment thereof.

As members of SAMRA (South African Marketing Research Association), VBH Heath Care Research Consultancy abides by the SAMRA code of conduct, part of which guarantees respondent anonymity for participation in our surveys. Responses from this survey will be collated and reported collectively to our Client – IMSA will never be able to link any specific responses to any one organisation. IMSA would, however, like to share the findings of this survey with participants. Should you wish to receive feedback of results, please sign the relevant space on the last page of the attached questionnaire. We also encourage all participants to forward any information that may add more value to the study.

Completed questionnaires should either be faxed to VBH at 011 467 6569, or emailed to lesley@vbh.co.za. The deadline for return of completed questionnaires is Monday, 30 April 2007.

Should you have any queries with regards to this survey, please do not hesitate to contact Lesley van Buuren or Trudi Hanson on 011 467 6569.

Yours faithfully
Lesley Van Buuren
(Managing Member VBH Health Care Research Consultancy)

QUESTIONNAIRE

Demographic Information

Name of Institution: _____

Your name: _____

Current Position: _____

Years of Experience in Current Position: _____

Question 1. The Applicability of Pharmaco-Economic (PE) Evaluation in South Africa

Please indicate, by marking the appropriate box, the extent to which you agree with each of the following statements relating to the applicability of pharmaco-economic evaluation in South Africa. Please note that you can only mark one box per statement.

	Strongly Agree	Agree	Disagree	Strongly Disagree	No Opinion
PE should be mandatory for ALL newly marketed medicines	❒	❒	❒	❒	❒
PE should be mandatory for ALL existing products	❒	❒	❒	❒	❒
PE should be mandatory ONLY for new generic drugs	❒	❒	❒	❒	❒
PE should not be done in isolation and should be part of a bigger health economics assessment, where all interventions and expenses are evaluated	❒	❒	❒	❒	❒
All health technologies, including devices, techniques, etc. should be subjected to PE	❒	❒	❒	❒	❒
PE assessment should ONLY be mandatory for drugs within selected therapeutic areas/categories	❒	❒	❒	❒	❒
PE assessment should be mandatory for drugs indicated for the treatment of PMB conditions.	❒	❒	❒	❒	❒
PE should be mandatory for ALL new biologicals	❒	❒	❒	❒	❒
PE should be mandatory for ALL STATE Tender Products	❒	❒	❒	❒	❒

Question 2. The Role of Pharmaco-Economic Evaluation in South Africa

Please indicate, by marking the appropriate box, the extent to which you agree with each statement regarding the **CURRENT** role that pharmaco-economic evaluation plays in the South African healthcare arena. Please note that you can only mark one box per statement.

	Strongly Agree	Agree	Disagree	Strongly Disagree	No Opinion
PE provides an ethical guidance for healthcare policy formulation	❒	❒	❒	❒	❒
PE provides an explicit benchmark for price setting of new drug entities	❒	❒	❒	❒	❒
PE provides a guide in setting clinical practice guidelines	❒	❒	❒	❒	❒
PE provides value in assessing health outcomes in disease management	❒	❒	❒	❒	❒
PE evaluation of a drug positively influences doctors' prescribing patterns	❒	❒	❒	❒	❒
PE is an effective means to control drug costs	❒	❒	❒	❒	❒
PE ensures equitable distribution of payers' health care resources	❒	❒	❒	❒	❒
PE plays a crucial role in setting payers' budget caps	❒	❒	❒	❒	❒
PE is the most important part of health economic evaluations	❒	❒	❒	❒	❒
PE is the easiest type of cost-effectiveness intervention a payer can make	❒	❒	❒	❒	❒

What other role do you see pharmaco-economic evaluation playing in the South African health care arena?

Question 3. Evaluation of Pharmaco-Economic Submissions

The following is a list of parties / organisations that are currently involved in the evaluation of pharmaco-economic submissions. Please indicate, by marking the appropriate box, the extent to which you agree that each listed party should be conducting pharmaco-economic evaluations. Please note that you can only mark one box per statement.

	Strongly Agree	Agree	Disagree	Strongly Disagree	No Opinion
PE submissions should be evaluated by Government	❒	❒	❒	❒	❒
PE submissions should be evaluated by Pharmaceutical Benefit Managers (PBM's)	❒	❒	❒	❒	❒
PE submissions should be evaluated by DUR-pharmacists.	❒	❒	❒	❒	❒
PE submissions should be evaluated by Medical Scheme Administrators	❒	❒	❒	❒	❒
PE submissions should be evaluated by the Council of Medical Schemes	❒	❒	❒	❒	❒
PE submissions should be evaluated by Managed Care Companies (MCO's)	❒	❒	❒	❒	❒
PE submissions should be evaluated by Independent Private Consultants	❒	❒	❒	❒	❒

Question 4. Standardisation of Requirements for Pharmaco-Economic Submissions

The countries listed below follow country-specific procedural guidelines for the submission of pharmaco-economic dossiers. Supposing a practical guide was to be developed to assist in standardising pharmaco-economic submissions in South Africa. Please indicate, by marking the appropriate box, how relevant you believe it would be to incorporate procedures from each of these sources into a South African guideline. Please note that you can only mark one box per option.

	Extremely relevant	Somewhat relevant	Not very relevant	Irrelevant	Do not know
Canadian Co-ordinating Office for Health Technology Assessment (CCOHTA) guidelines	❒	❒	❒	❒	❒
Australian Pharmaceutical Benefit Advisory Committee (PBAC)	❒	❒	❒	❒	❒
National Institution for Health and Clinical Excellence (NICE) from the United Kingdom (UK)	❒	❒	❒	❒	❒
Local / South African Expert Opinion	❒	❒	❒	❒	❒

Question 5. Preferred Health Outcome Measures for Pharmaco-Economic submissions

Listed below are some health outcome measures for pharmaco-economic submissions. Please could you rank these from most to least important, by writing a "1" next to the option that you believe is the most important outcome measure, a "2" next to the outcome that you believe is the second most important measure ... up to "5" being the fifth most important measure. Please note that you can only use each number from 1 – 5 once.

	RANKING FROM 1 - 5
Cost-effectiveness	
Cost minimization	
Cost utilities	
Quality Adjusted Life Years (QALYs)	
Budget impact	

How do you define cost-effectiveness? What factors do you take into consideration when assessing cost-effectiveness?

Question 6a. Critical Success Factors for Appropriate Implementation of Pharmaco-Economic submissions

The following is a list of factors that could influence the implementation of pharmaco-economics in South Africa. Please could you indicate, by ticking the appropriate box, how important you feel each factor is. Please note that you can only mark one box per factor.

	Extremely Important	Somewhat Important	Not very Important	Irrelevant	Don't Know
Adequate resources and capacity for the review process	❒	❒	❒	❒	❒
Availability of local epidemiological data for South Africa	❒	❒	❒	❒	❒
Relevance of PE models to current treatment guidelines	❒	❒	❒	❒	❒
Relevance of PE models to the South African population	❒	❒	❒	❒	❒
Relevance of PE models to the South African medical scheme population / medical scheme members	❒	❒	❒	❒	❒
Availability of centres of excellence for PE	❒	❒	❒	❒	❒
Availability of a legal framework that governs the application of PE in South Africa	❒	❒	❒	❒	❒
Transparency in the PE studies submitted by the manufacturer	❒	❒	❒	❒	❒
Provision of formal training on PE	❒	❒	❒	❒	❒
Availability of a central repository of data for the country	❒	❒	❒	❒	❒

Question 6b. Critical Success Factors for Appropriate Implementation of Pharmaco-Economic submissions

Listed below are the same factors just considered. Please could you rank these from most to least important, by writing a "1" next to the option that you believe is the most important critical success factor, a "2" next to the option that you believe is the second most important critical success factor ... up to "10" being the least important of the ten factors given. Please note that you can only use each number from 1 – 10 once.

	RANKING FROM 1 – 10
Adequate resources and capacity for the review process	
Availability of local epidemiological data for South Africa	
Relevance of PE models to current treatment guidelines	
Relevance of PE models to the South African population	
Relevance of PE models to the South African medical scheme population / medical scheme members	
Availability of centres of excellence for PE	
Availability of a legal framework that governs the application of PE in South Africa	
Transparency in the PE studies submitted by the manufacturer	
Provision of formal training on PE	
Availability of a central repository of data for the country	

Question 7a. Measures Considered in the Review of Pharmaco-Economic Submissions

The following is a list of measures likely to be considered in the review of pharmaco-economic submissions. Please could you indicate, by ticking the appropriate box, how important you feel each measure is. Please note that you can only mark one box per option.

	Extremely Important	Somewhat Important	Not very Important	Irrelevant	Don't Know
Therapeutic effects of the drug or technology assessed	❒	❒	❒	❒	❒
Clinical guidelines as set by professional bodies	❒	❒	❒	❒	❒
Relationship between the treatment effects and costs (cost vs effects) of the drug or medical technology assessed	❒	❒	❒	❒	❒
Relationship between the treatment effects and total economic cost associated with the use of the drug or medical technology under review.	❒	❒	❒	❒	❒
Impact of the cost of medicines or technology (that is under review) on total cost to the scheme (cost vs budget)	❒	❒	❒	❒	❒
Impact of the cost of medicines or technology (that is under review) onto the total healthcare budget for the country	❒	❒	❒	❒	❒
Impact of the cost of medicines or technology (under review) on the patient's out-of-pocket expenses	❒	❒	❒	❒	❒
Cost of the medicine or new technology only	❒	❒	❒	❒	❒
Years of clinical experience with the medicine or new technology	❒	❒	❒	❒	❒
Reputation and credibility of the manufacturer	❒	❒	❒	❒	❒
Standardised threshold levels for chronic conditions	❒	❒	❒	❒	❒
Standardised league tables for selected chronic conditions	❒	❒	❒	❒	❒

Question 7a. Measures Considered in the Review of Pharmaco-Economic Submissions

Listed below are the same factors just considered. Please could you rank these from most to least important, by writing a "1" next to the option that you believe is the most important measure, a "2" next to the option that you believe is the second most important measure ... up to "12" being the least important of the twelve factors given. Please note that you can only use each number from 1 – 12 once.

	RANKING FROM 1 – 12
Therapeutic effects of the drug or technology assessed	
Clinical guidelines as set by professional bodies	
Relationship between the treatment effects and costs (cost vs effects) of the drug or medical technology assessed	
Relationship between the treatment effects and total economic cost associated with the use of the drug or medical technology under review.	
Impact of the cost of medicines or technology (under review) on the total cost to the scheme (cost vs budget)	
Impact of the cost of medicines or technology (under review) on the total healthcare budget for the country	
Impact of the cost of medicines or technology (under review) on the patient's out-of-pocket expenses	
Cost of the medicine or new technology (under review) only	
Years of clinical experience with the medicine or new technology under review	
Reputation and credibility of the manufacturer	
Standardised threshold levels for chronic conditions	
Standardised league tables for selected chronic conditions	

Question 8. Resources Required For Effective Pharmaco-Economic Evaluations

Please indicate, by ticking the appropriate box, the extent to which you agree that each of the following statements applies to your organisation's systems that are in place for the evaluation of pharmaco-economic submissions. Please note that you can only mark one box per statement.

	Strongly Agree	Agree	Disagree	Strongly Disagree	No Opinion
My organisation has relevantly qualified human resources to perform effective PE analysis	❒	❒	❒	❒	❒
My organisation effectively performs PE evaluations	❒	❒	❒	❒	❒
When performing PE evaluations on **new chemical entities**, my company does so by engaging / discussing pertinent issues with the relevant company during the review process	❒	❒	❒	❒	❒
When performing PE evaluations on **generic** medicines, my company does so by engaging / discussing pertinent issues with the relevant company during the review process	❒	❒	❒	❒	❒
My organisation has a documented policy on how to perform PE evaluations	❒	❒	❒	❒	❒
My organisation has agreed upon time frames for completion of PE evaluations	❒	❒	❒	❒	❒
My organisations has a guideline for the communication of PE evaluations results to all relevant parties	❒	❒	❒	❒	❒

Please record any other comments you would like to add about pharmaco-economic evaluations, the assessment thereof and the role that they play in the South African health care arena.

_____

_____

_____

_____

Please sign below if you would like to receive feedback on the findings of this survey:

I hereby provide VBH permission to reveal my name to IMSA as having participated in this survey. I understand that even if I give permission to reveal my name my responses will remain anonymous; the only reason for revealing my name is so that I can get feedback on the findings of this survey.

Signed: _____ Date: _____

Please return your completed questionnaire on or before Friday, 13 April 2007.

You can fax your completed questionnaire to:

VBH Health Care Research Consultancy – 011 467 6569

Alternatively, if completed electronically, please email to: lesley@vbh.co.za.

Please note that on receipt of this completed questionnaire a payment of R300.00 will be made to you. Please indicate below, how you would like this payment made:

I would like my payment made (please circle appropriate code and supply relevant information):

By cheque - x

By direct bank deposit - y

Name to appear on cheque:

……..

Cheque to be posted to:

……..

……..

……..

……..

Code: ……

Banking details:

Bank: ……..

Branch: ……

Account name: ……..

Account number: ……

THANK YOU FOR YOUR TIME AND INPUT

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