



**PHARMACY
VISION
20/20**

CSHP SEMINAR 20 • OCTOBER 21-25
Disneyland
RESORT

HEALTH ECONOMICS FOR FORMULARY DECISION-MAKING

CYNTHIA L. GONG, PHARMD, PHD
ASSISTANT PROFESSOR OF RESEARCH
FETAL & NEONATAL INSTITUTE, DIVISION OF NEONATOLOGY
CHILDREN'S HOSPITAL LOS ANGELES
DEPARTMENT OF PEDIATRICS, KECK SCHOOL OF MEDICINE
UNIVERSITY OF SOUTHERN CALIFORNIA



DISCLOSURE

Dr. Gong has no conflicts of interest to disclose.

LEARNING OBJECTIVES

- Identify the role of pharmacoeconomics in value-based decision making.
- Differentiate between different economic model types.
- Identify key parameters in an economic model.
- Apply pharmacoeconomics to value-based decision making.

WHAT IS PHARMACOECONOMICS (PE)?

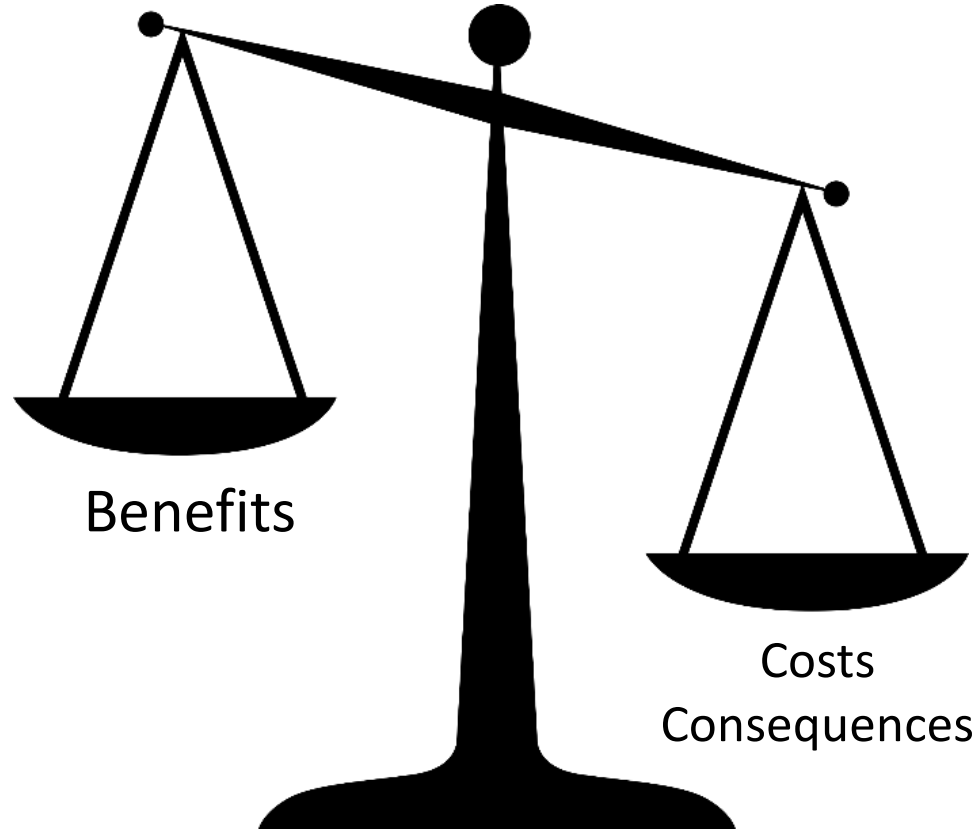


Image source: <https://www.economist.com/news/international/21592655-drug-firms-have-new-medicines-and-patients-are-desperate-them-arguments-over>

[All images licensed under CC BY-SA-NC](#)

TYPES OF PE ANALYSES^{1,2}

Model Type	Description	Example
Cost of Illness (COI)	<ul style="list-style-type: none"> Estimates cost of disease on a population 	<ul style="list-style-type: none"> What is the annual cost of cancer?
Cost Minimization Analysis (CMA)	<ul style="list-style-type: none"> Determines least costly option assuming identical outcomes (e.g. generic equivalent drugs) 	<ul style="list-style-type: none"> How does the cost of drug A compare to drug B, assuming equal efficacy?
Cost Benefit Analysis (CBA)	<ul style="list-style-type: none"> Measures all benefits and consequences in \$\$ 	<ul style="list-style-type: none"> What is the cost benefit of a clinical pharmacy vs. nursing discharge service?

1. Neumann PJ, Sanders GD, Russell LB, et al. *Cost-Effectiveness in Health and Medicine*. 2nd ed. New York, NY: Oxford University Press; 2017.

2. Drummond MF, Sculpher MJ, Claxton K, et al. *Methods for the Economic Evaluation of Health Care Programmes*. 4th ed. Oxford, UK: Oxford Medical Publications; 2015.

TYPES OF PE ANALYSES^{1,2}

Model Type	Description	Example
Cost Effectiveness Analysis (CEA)	<ul style="list-style-type: none"> Measures cost (\$) per clinical outcome Cost-utility analysis: when outcome is QALY 	<ul style="list-style-type: none"> What is the cost-effectiveness of drug therapy A vs. B in terms of \$ / LDL reduction?
Budget Impact Analysis (BIA)	<ul style="list-style-type: none"> Compares alternatives based on cost only Estimates the budget impact to the payer 	<ul style="list-style-type: none"> What is the budget impact of adding a new drug to our formulary?

QALY = quality-adjusted life year; LDL = low-density lipoprotein

1. Neumann PJ, Sanders GD, Russell LB, et al. *Cost-Effectiveness in Health and Medicine*. 2nd ed. New York, NY: Oxford University Press; 2017.

2. Drummond MF, Sculpher MJ, Claxton K, et al. *Methods for the Economic Evaluation of Health Care Programmes*. 4th ed. Oxford, UK: Oxford Medical Publications; 2015.

“ALL MODELS ARE WRONG, BUT SOME ARE USEFUL”

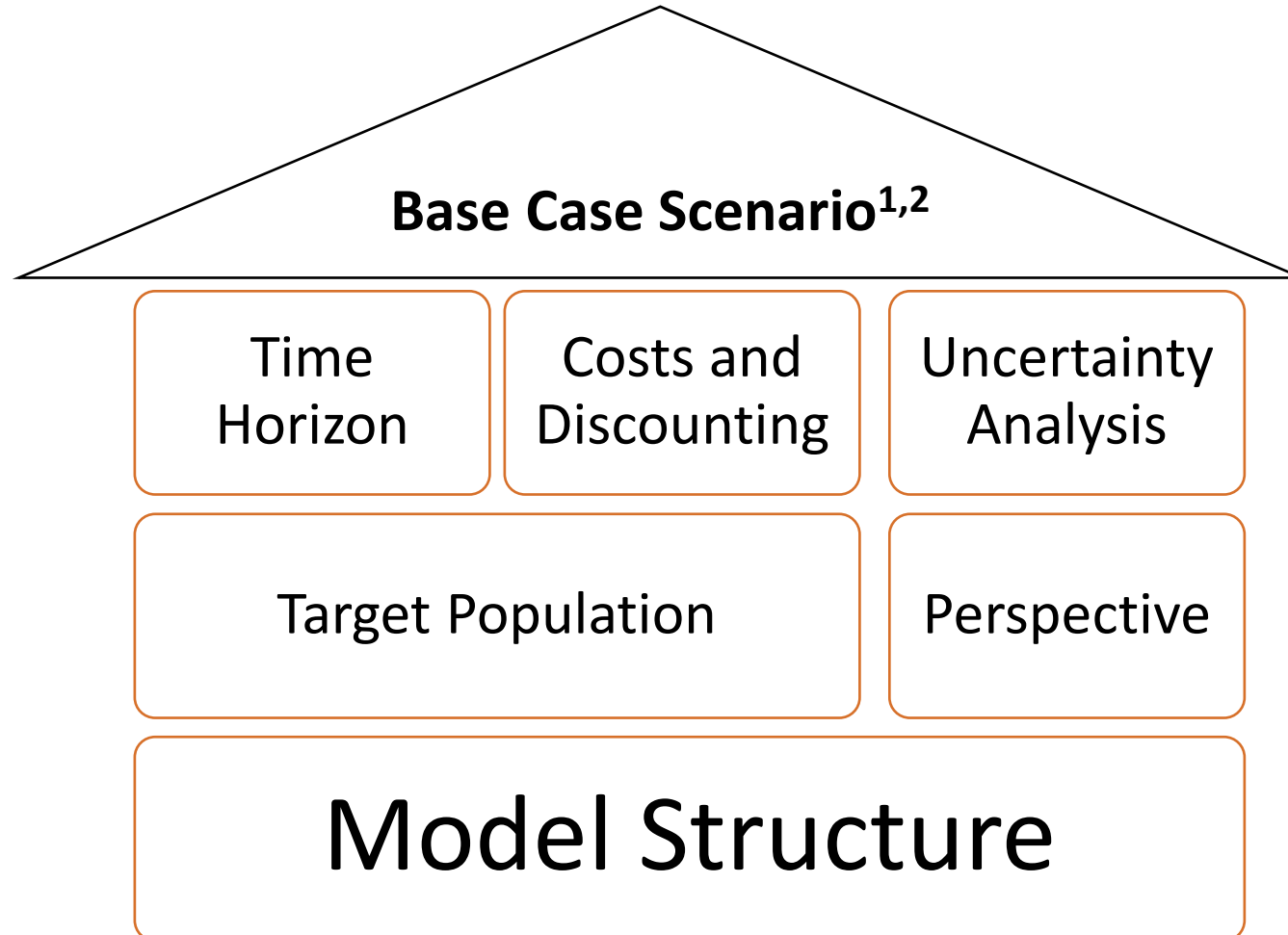
George E.P. Box

Health Economics.... A New Toxicity?

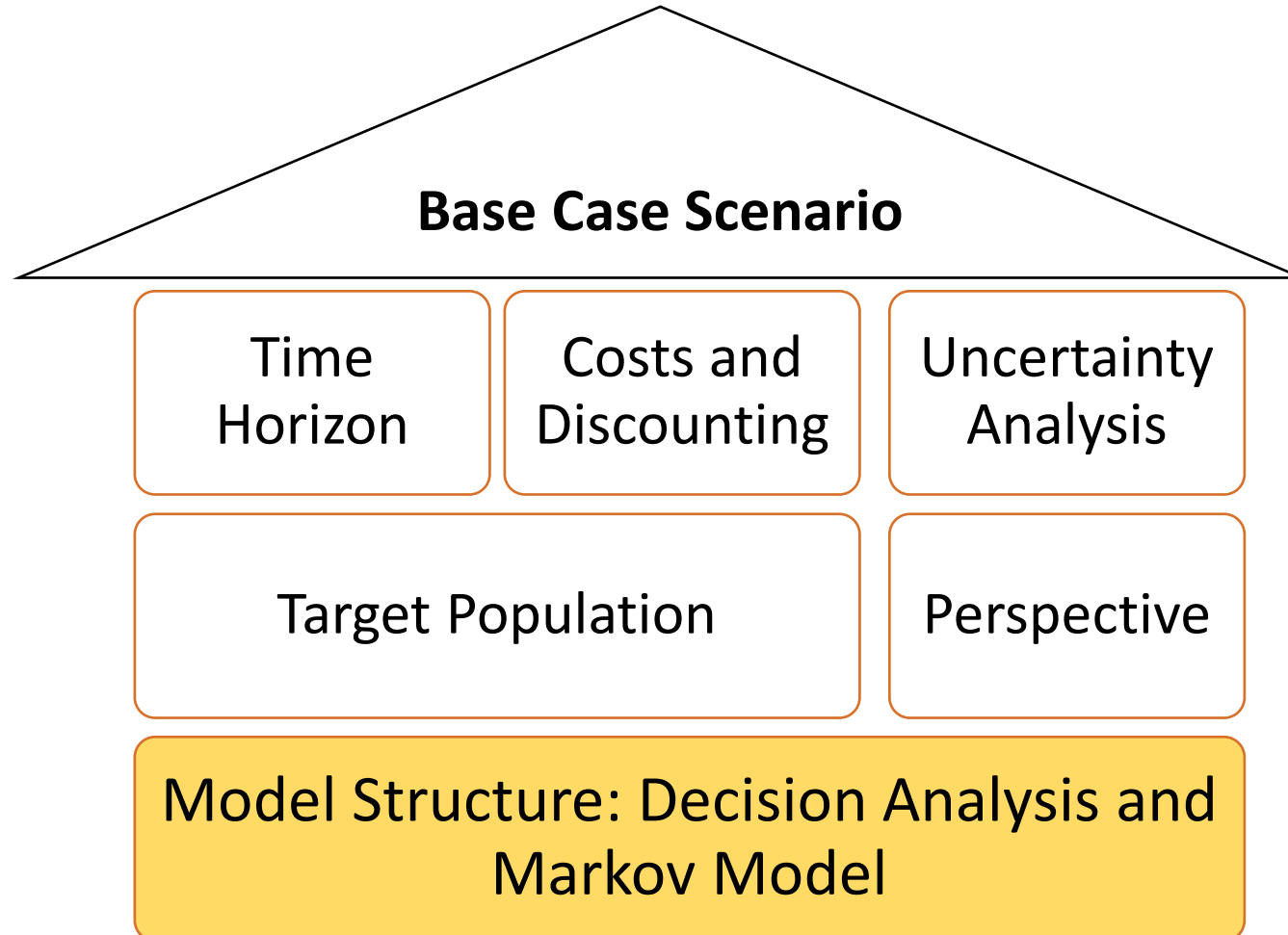


"The drug itself has no side effects - but the number of health economists needed to prove its value may cause dizziness and nausea"

MODELING 101: BASIC BUILDING BLOCKS

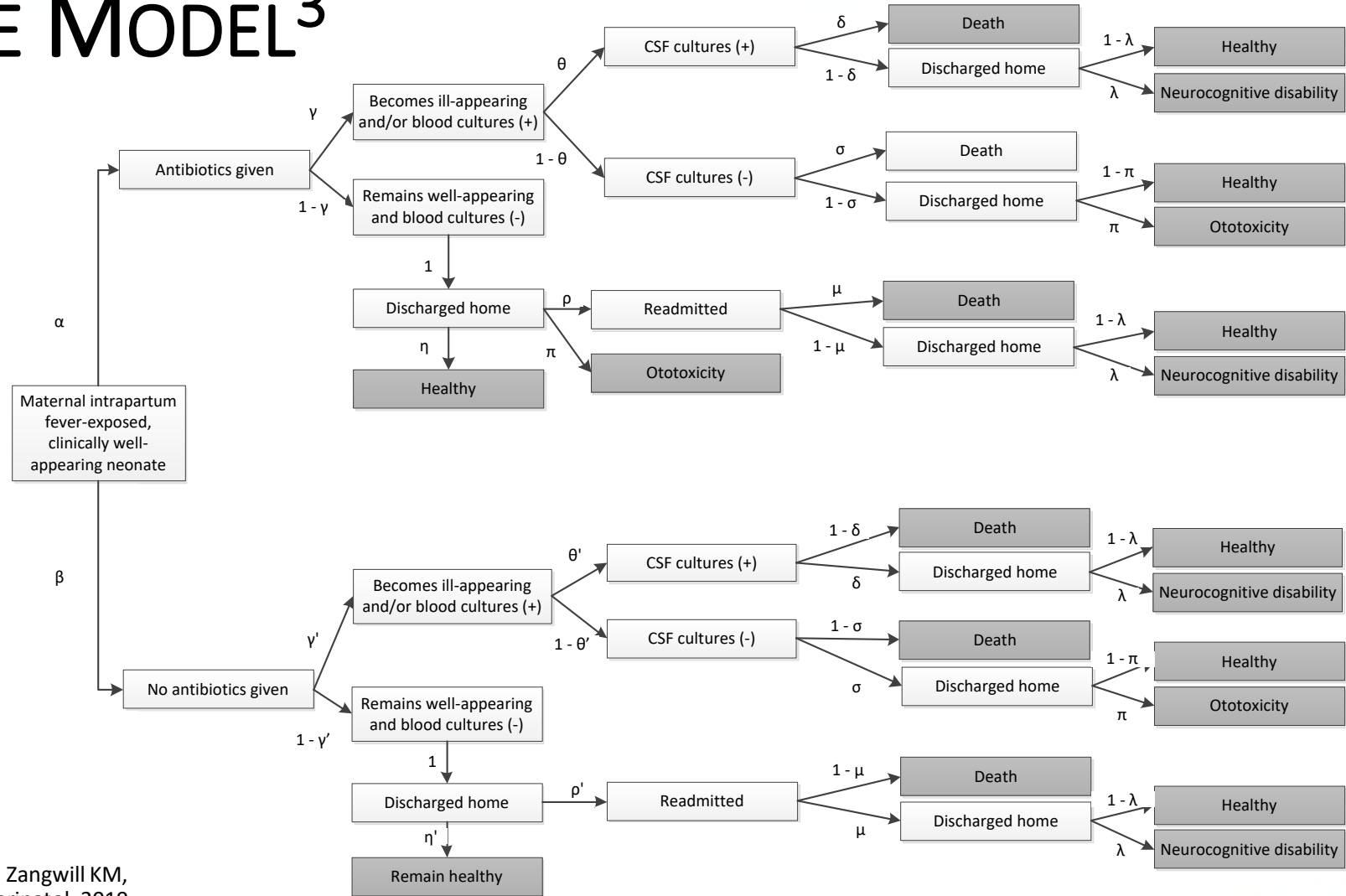


MODELING 101: THE FOUNDATION



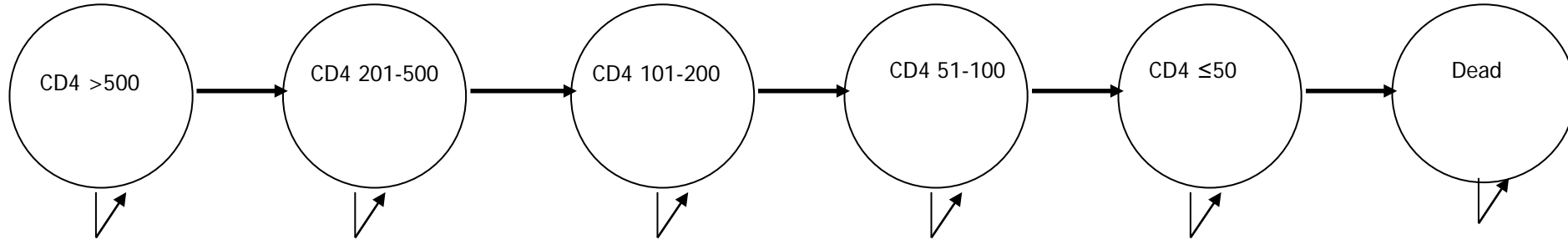
DECISION TREE MODEL³

- Linear progression
- Finite start and end points
- Acute, short-term scenarios

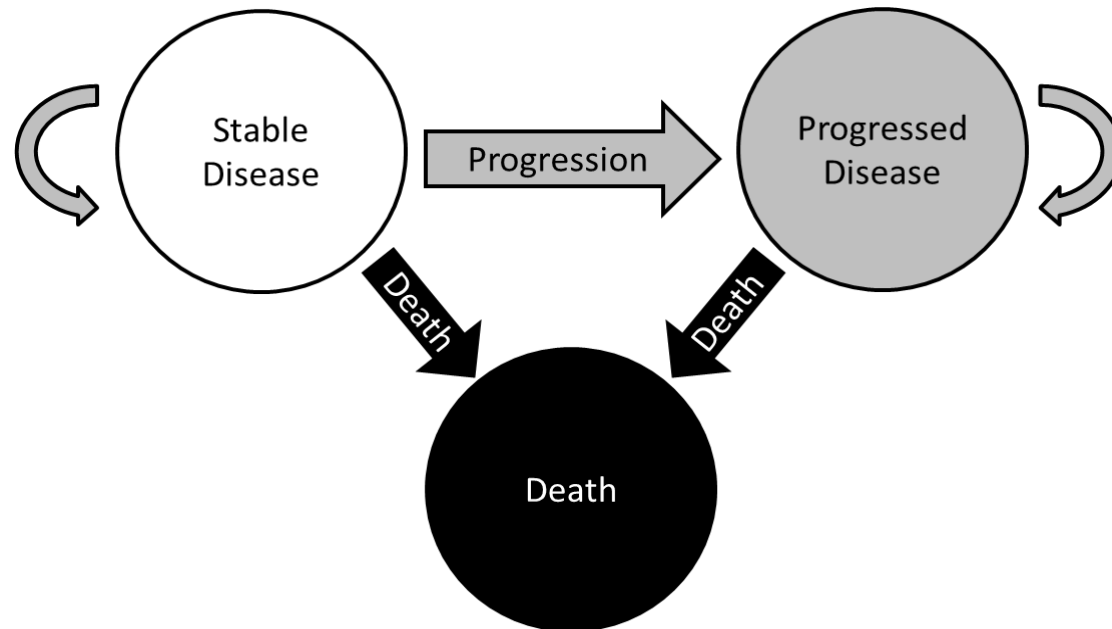


³ Gong CL, Dasgupta S, Zangwill KM, Bolaris M, Hay JW. J Perinatol. 2019 Apr;39(4):571-580.

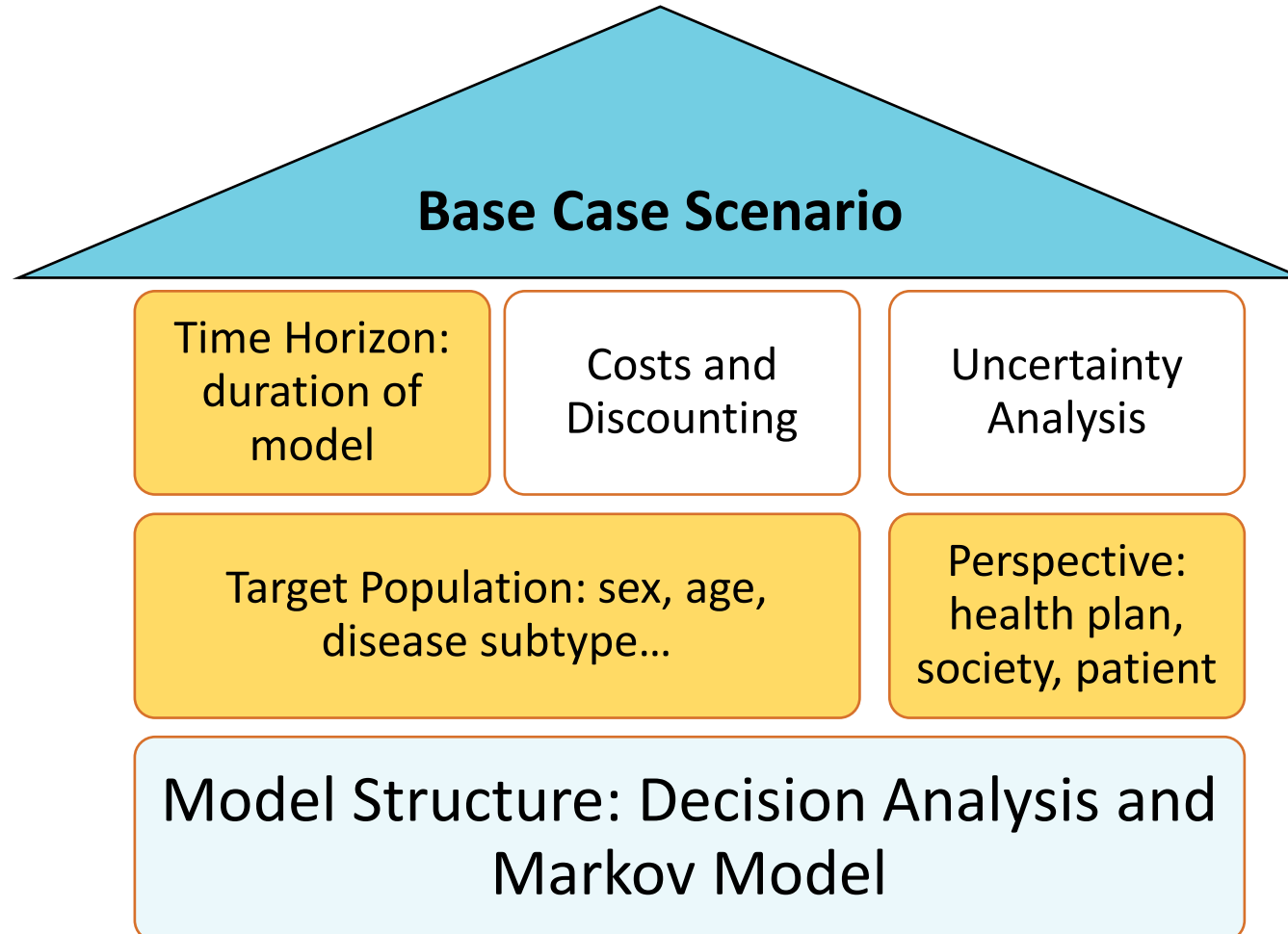
MARKOV MODELS^{4,5}



- Dynamic progression
- Chronic disease states
- Time horizon key factor
- What are some other examples?



MODELING 101: STRUCTURE



BASE CASE ANALYSIS

- Establishes a reference point against which future comparisons will be made (Who pays? Who benefits? Duration?)
- For example, flu vaccination program:
 - Perspective – societal, health plan(?)
 - Population – all eligible individuals (exclusions?)
 - Time horizon – one year (vaccine efficacy)
 - Base case – all eligible individuals
 - Alternate scenario – subgroups?

TEST QUESTION 1

The most commonly used perspective in a CEA model *for the purposes of P&T* is:

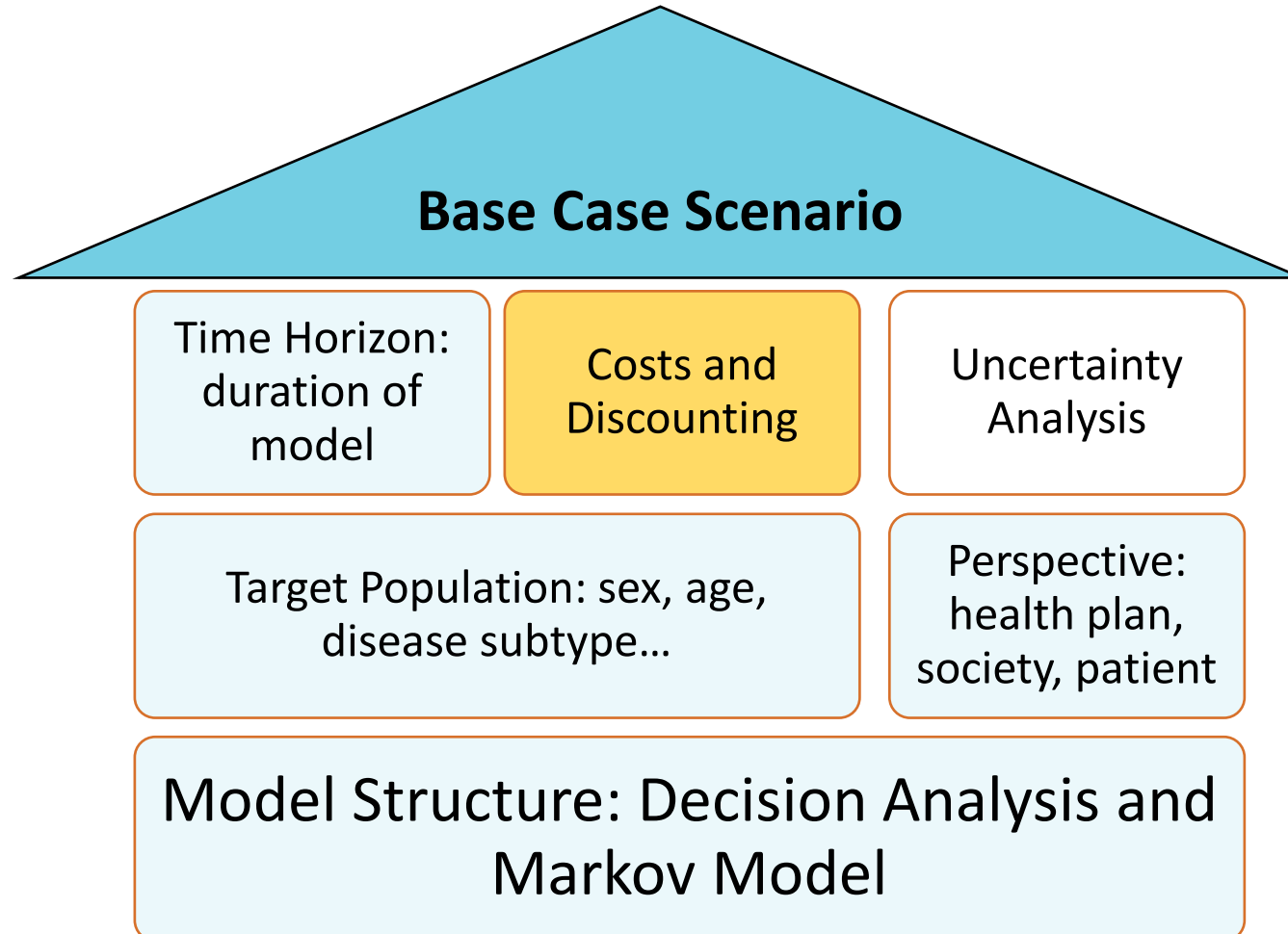
- a) Societal
- b) Patient
- c) Health plan
- d) Physician

TEST QUESTION 1: ANSWER

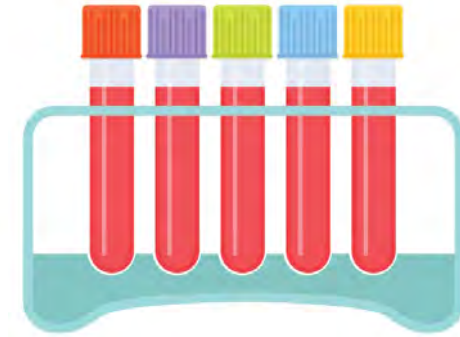
The most commonly used perspective in a CEA model *for the purposes of P&T* is:

- a) Societal
- b) Patient
- c) Health plan**
- d) Physician

MODELING 101: PARAMETER SPECIFICATIONS



COST TYPES



costs should be relevant to the perspective

DISCOUNTING

- Done if analysis spans >1 year; adjusts future costs and benefits using an expected interest value or discount rate (typically 3%)
- For example: new clinical program costs \$1500/year; you want to evaluate it after 4 years

Year	Cost	Present Value
1	\$ 1,500	\$ 1,455
2	\$ 1,500	\$ 1,411
3	\$ 1,500	\$ 1,369
4	\$ 1,500	\$ 1,328
TOTAL	\$ 6,000	\$ 5,563

TEST QUESTION 2

If the cost of Superchemo[®] is \$100,000 today, how much is it worth one year from now, assuming a discount rate of 3%? What about two years from now?

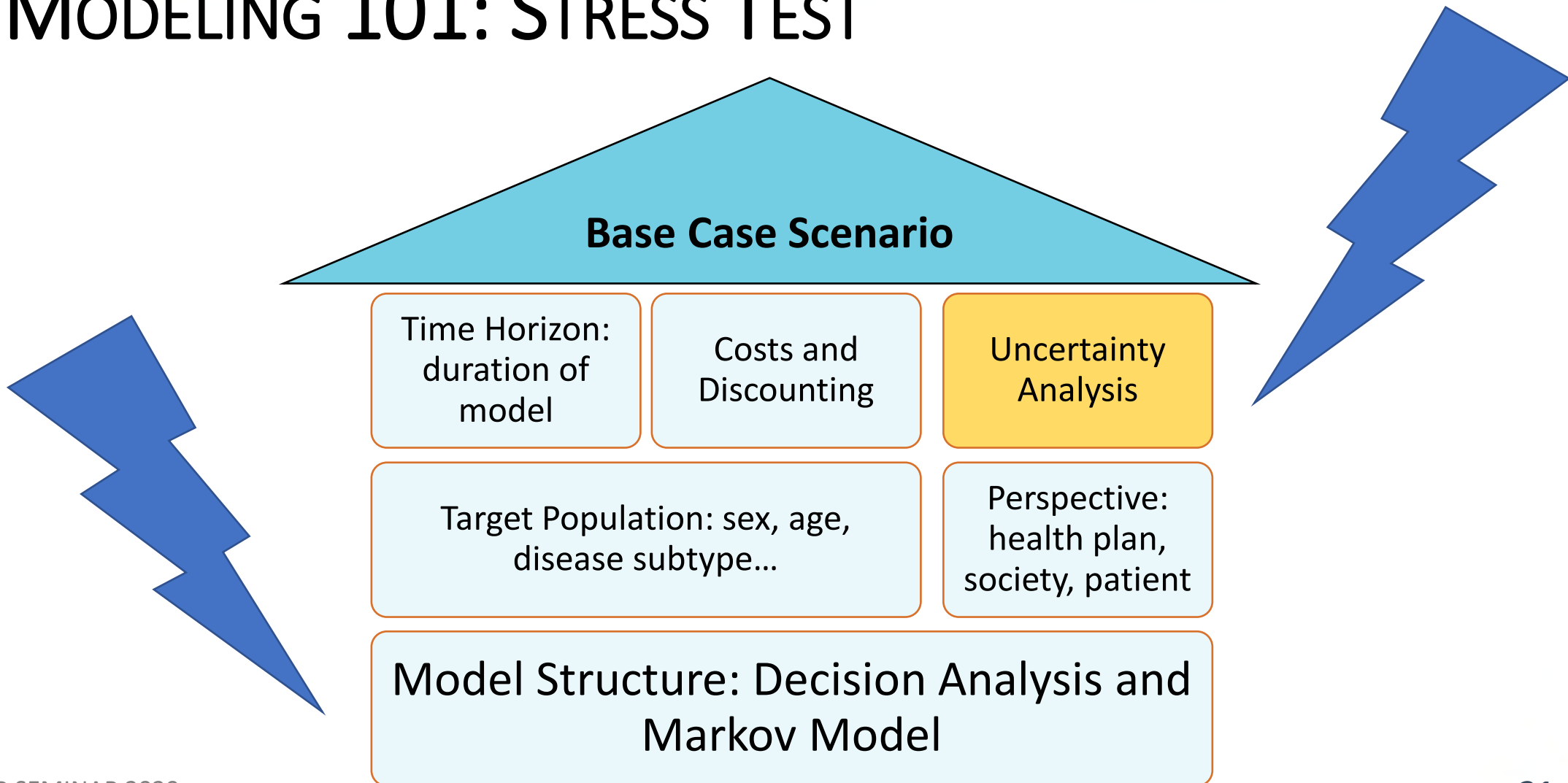
- a) \$99,000 in one year, ~\$98,000 in two years
- b) \$97,000 in one year, ~\$80,000 in two years
- c) \$103,000 in one year, ~\$106,000 in two years
- d) \$97,000 in one year, ~\$94,000 in two years

TEST QUESTION 2: ANSWER

If the cost of Superchemo[®] is \$100,000 today, how much is it worth one year from now, assuming a discount rate of 3%? What about two years from now?

- a) \$99,000 in one year, ~\$98,000 in two years
- b) \$97,000 in one year, ~\$80,000 in two years
- c) \$103,000 in one year, ~\$106,000 in two years
- d) \$97,000 in one year, ~\$94,000 in two years**

MODELING 101: STRESS TEST



SENSITIVITY ANALYSIS^{1,2}

- Are the results robust to realistic changes in various model inputs and parameters?
- One-way, multivariate, and probabilistic
 - Which parameter affects my results the most?
 - What does my model tell me under specific conditions?
 - With repeated simulations, how do the results converge?

1. Neumann PJ, Sanders GD, Russell LB, et al. Oxford University Press; 2017.
2. Drummond MF, Sculpher MJ, Claxton K, et al. Oxford Medical Publications; 2015.

CEAs vs. BIAs

Key differences between the two model types

CEA vs. BIA

COST-EFFECTIVENESS ANALYSIS

- Measures outcomes against costs
- Incremental cost-effectiveness ratio (ICER):

$$\text{ICER} = \frac{\text{Cost}_A (\$) - \text{Cost}_B (\$)}{\text{Effect}_A - \text{Effect}_B}$$

- Cost-UTILITY analysis is a special type of CEA; outcome is quality-adjusted life year (QALY), calculated as **# of life years x utility weight**

BUDGET IMPACT ANALYSIS

- A model to assess the financial impact of adding a new intervention to an organization's budget
- ESSENTIAL in value assessment of treatments or interventions in managed care organizations
- Gives the *comprehensive* financial impact of a new therapy on health plan spending
- Crucial to formulary decision-making

TEST QUESTION 3

If Superchemo[®] costs \$25,000 more than Regularchemo[®] and increases QALYs by 0.25, what is its ICER (\$/QALY)?

- a) \$40,000 / QALY
- b) \$75,000 / QALY
- c) \$100,000 / QALY
- d) \$625,000 / QALY

TEST QUESTION 3: ANSWER

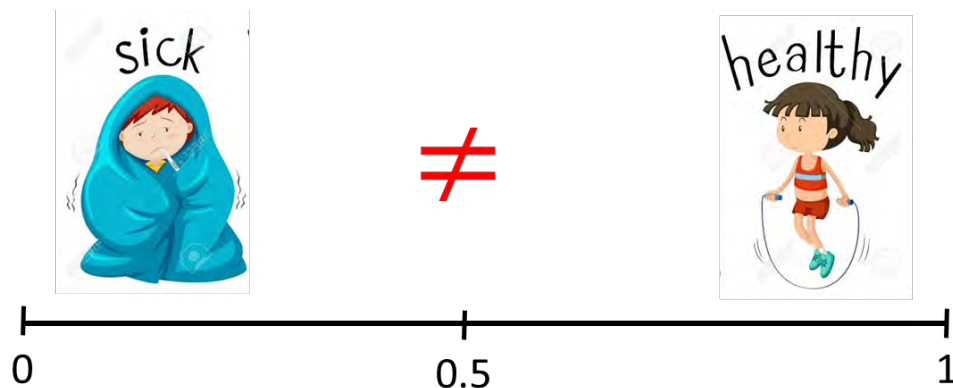
If Superchemo[®] costs \$25,000 more than Regularchemo[®] and increases QALYs by 0.25, what is its ICER (\$/QALY)?

- a) \$40,000 / QALY
- b) \$75,000 / QALY
- c) \$100,000 / QALY**
- d) \$625,000 / QALY

CEA vs. BIA

COST-UTILITY ANALYSIS

- QALY = utility x # of years in health state
- Utility: a weight assigned to a specific health state to adjust the # of years lived according to how good or bad the health state is weighted



BUDGET IMPACT ANALYSIS

- No valuation of clinical efficacy or desirability of alternative treatments
- Overlaps with other model types only in conceptual approach
- Clinical outcomes, QALYs, productivity losses, time loss due to morbidity / mortality are not included in a BIA

TEST QUESTION 4

Assume your survival in health state A is 10 years. The utility weight for health state A is 0.7. How many quality-adjusted life years is health state A worth?

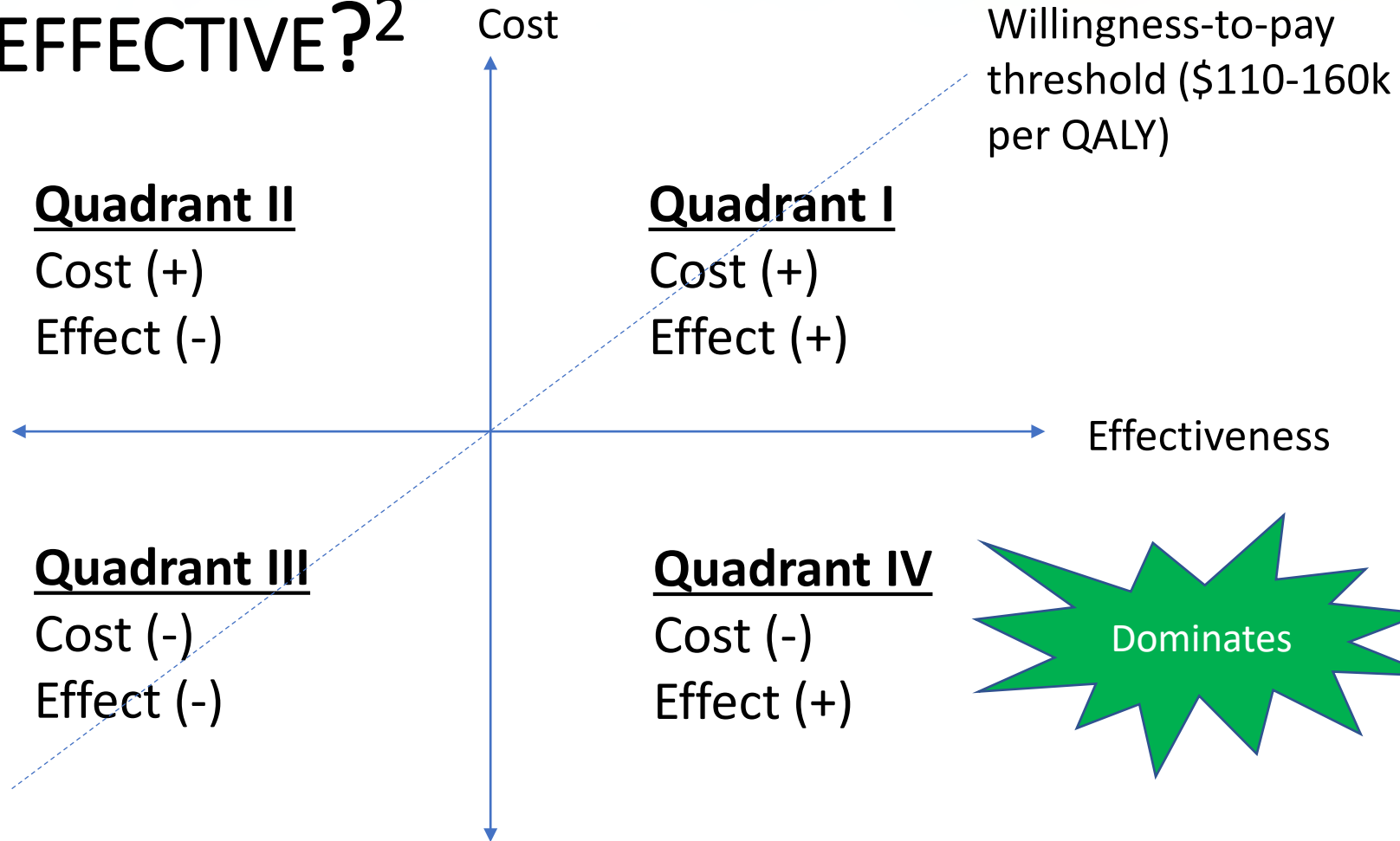
- a) 7 years
- b) 10 years
- c) 9.3 years
- d) 10.7 years

TEST QUESTION 4: ANSWER

Assume your survival in health state A is 10 years. The utility weight for health state A is 0.7. How many quality-adjusted life years is health state A worth?

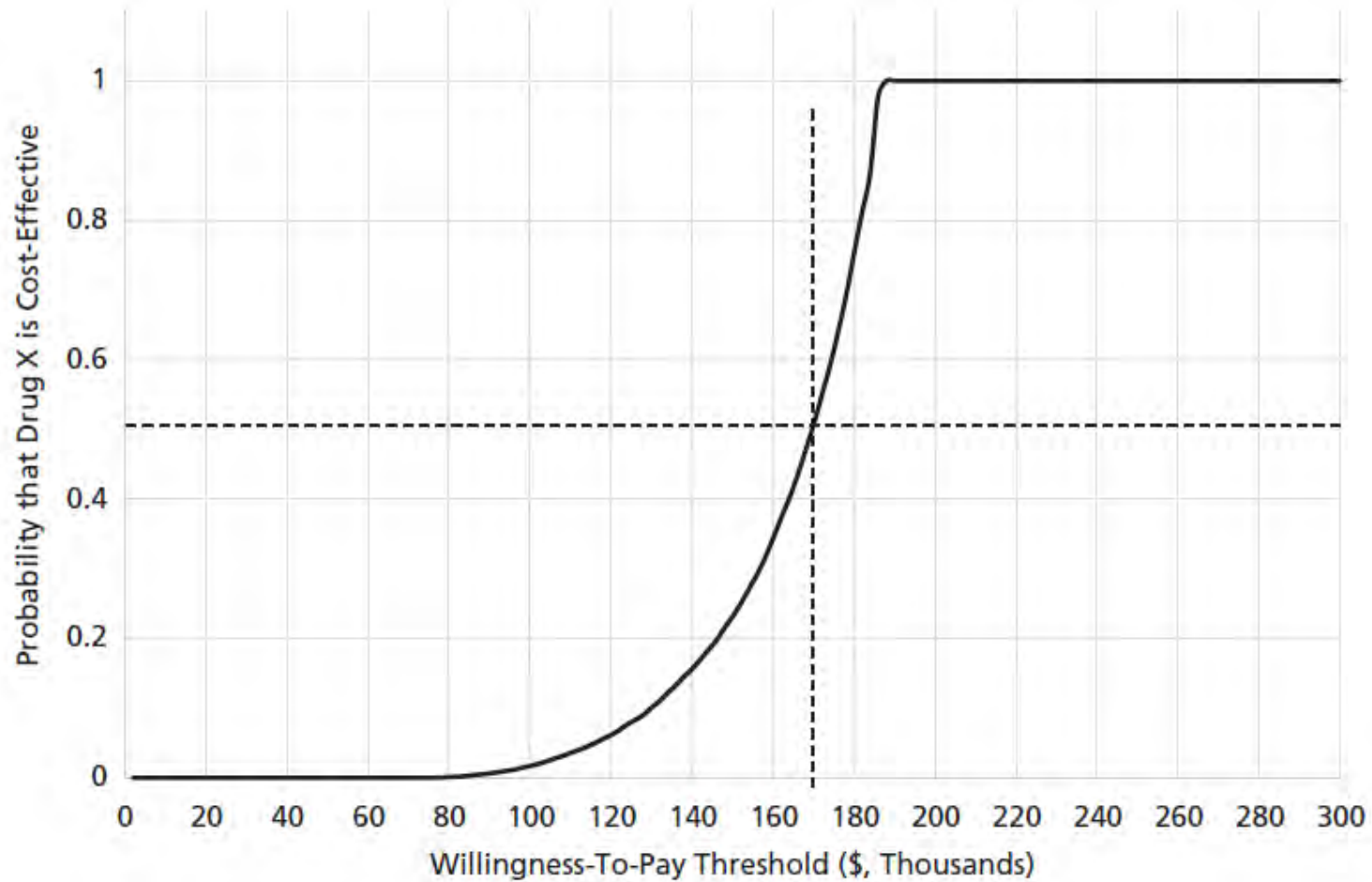
- a) **7 years**
- b) 10 years
- c) 9.3 years
- d) 10.7 years

IS IT COST-EFFECTIVE?²



² Adapted from Drummond MF. Methods for the Economic Evaluation of Health Care Programmes. New York, NY: Oxford University Press; 2005. Copyright 2005 by the Oxford University Press.

IS IT COST-EFFECTIVE?



EXPANDING THE ROLE OF P&T COMMITTEES

- Currently:
 - Clinical efficacy
 - Drug acquisition cost
 - Budget impact analysis
- VALUE: cost per desired outcome?
 - Additional inputs
 - Additional expertise
 - Comprehensive evaluation

FUTURE

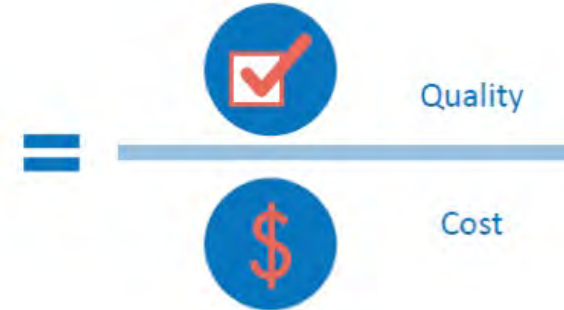


Image reference: <http://www.healthcareitnews.com/sponsored-content/solving-healthcare-value-equation-0>

EXAMPLES OF US VALUE FRAMEWORKS

1. ACC-AHA Cost/Value Methodology
2. ASCO Value of Cancer Care Framework
- 3. ICER Value Assessment Framework**
4. MSKCC Drug Abacus
5. NCCN Evidence Blocks

ACC-AHA: American College of Cardiology – American Heart Association

ASCO: American Society of Clinical Oncologists

ICER: Institute for Comparative Effectiveness Research

MSKCC: Memorial Sloan Kettering Cancer Center

NCCN: National Comprehensive Cancer Network

PHARMACIST'S ROLE

Critically evaluate model methods and translate for clinical and administrative leadership

1. Transparency: assumptions, sensitivity analyses
2. Heterogeneity: adherence, population subtypes
3. Calibration: determining best parameters for base model
4. Validation: internal, external, and cross-validation



ISPOR CHEERS* CHECKLIST⁶

CEA Model Inputs

- Population
- Interventions
- Clinical effects
- Costs (direct, indirect)

Model Structure

- Model design
- Model outputs

Data Sources

- National
- Internal
- Assumptions

⁶ Husereau D et al. ISPOR CHEERS Report. Value in Health, 2013; 231-250

Table 2 – CHEERS checklist—Items to include when reporting economic evaluations of health interventions.

Section/item	Item no.	Recommendation	Reported on page no./line no.
Title and abstract			
Title	1	Identify the study as an economic evaluation, or use more specific terms such as “cost-effectiveness analysis” and describe the interventions compared.	_____
Abstract	2	Provide a structured summary of objectives, perspective, setting, methods (including study design and inputs), results (including base-case and uncertainty analyses), and conclusions.	_____
Introduction			
Background and objectives	3	Provide an explicit statement of the broader context for the study. Present the study question and its relevance for health policy or practice decisions.	_____
Methods			
Target population and subgroups	4	Describe characteristics of the base-case population and subgroups analyzed including why they were chosen.	_____
Setting and location	5	State relevant aspects of the system(s) in which the decision(s) need(s) to be made.	_____
Study perspective	6	Describe the perspective of the study and relate this to the costs being evaluated.	_____
Comparators	7	Describe the interventions or strategies being compared and state why they were chosen.	_____
Time horizon	8	State the time horizon(s) over which costs and consequences are being evaluated and say why appropriate.	_____
Discount rate	9	Report the choice of discount rate(s) used for costs and outcomes and say why appropriate.	_____
Choice of health outcomes	10	Describe what outcomes were used as the measure(s) of benefit in the evaluation and their relevance for the type of analysis performed.	_____
Measurement of effectiveness	11a	Single study-based estimates: Describe fully the design features of the single effectiveness study and why the single study was a sufficient source of clinical effectiveness data.	_____
	11b	Synthesis-based estimates: Describe fully the methods used for the identification of included studies and synthesis of clinical effectiveness data.	_____
Measurement and valuation of preference-based outcomes	12	If applicable, describe the population and methods used to elicit preferences for outcomes.	_____
Estimating resources and costs	13a	Single study-based economic evaluation: Describe approaches used to estimate resource use associated with the alternative interventions. Describe primary or secondary research methods for valuing each resource item in terms of its unit cost. Describe any adjustments made to approximate to opportunity costs.	_____
	13b	Model-based economic evaluation: Describe approaches and data sources used to estimate resource use associated with model health states. Describe primary or secondary research methods for valuing each resource item in terms of its unit cost. Describe any adjustments made to approximate to opportunity costs.	_____
Currency, price date, and conversion	14	Report the dates of the estimated resource quantities and unit costs. Describe methods for adjusting estimated unit costs to the year of reported costs if necessary. Describe methods for converting costs into a common currency base and the exchange rate.	_____
Choice of model	15	Describe and give reasons for the specific type of decision-analytic model used. Providing a figure to show model structure is strongly recommended.	_____
Assumptions	16	Describe all structural or other assumptions underpinning the decision-analytic model.	_____
Analytic methods	17	Describe all analytic methods supporting the evaluation. This could include methods for dealing with skewed, missing, or censored data; extrapolation methods; methods for pooling data; approaches to validate or make adjustments (e.g., half-cycle corrections) to a model; and methods for handling population heterogeneity and uncertainty.	_____

Table 2 - continued

Section/item	Item no.	Recommendation	Reported on page no./line no.
Results			
Study parameters	18	Report the values, ranges, references, and if used, probability distributions for all parameters. Report reasons or sources for distributions used to represent uncertainty where appropriate. Providing a table to show the input values is strongly recommended.	_____
Incremental costs and outcomes	19	For each intervention, report mean values for the main categories of estimated costs and outcomes of interest, as well as mean differences between the comparator groups. If applicable, report incremental cost-effectiveness ratios.	_____
Characterizing uncertainty	20a	<i>Single study-based economic evaluation:</i> Describe the effects of sampling uncertainty for estimated incremental cost, incremental effectiveness, and incremental cost-effectiveness, together with the impact of methodological assumptions (such as discount rate, study perspective).	_____
	20b	<i>Model-based economic evaluation:</i> Describe the effects on the results of uncertainty for all input parameters, and uncertainty related to the structure of the model and assumptions.	_____
Characterizing heterogeneity	21	If applicable, report differences in costs, outcomes, or cost-effectiveness that can be explained by variations between subgroups of patients with different baseline characteristics or other observed variability in effects that are not reducible by more information.	_____
Discussion			
Study findings, limitations, generalizability, and current knowledge	22	Summarize key study findings and describe how they support the conclusions reached. Discuss limitations and the generalizability of the findings and how the findings fit with current knowledge.	_____
Other			
Source of funding	23	Describe how the study was funded and the role of the funder in the identification, design, conduct, and reporting of the analysis. Describe other nonmonetary sources of support.	_____
Conflicts of interest	24	Describe any potential for conflict of interest among study contributors in accordance with journal policy. In the absence of a journal policy, we recommend authors comply with International Committee of Medical Journal Editors' recommendations.	_____

Note. For consistency, the CHEERS statement checklist format is based on the format of the CONSORT statement checklist.

BIA CHECKLIST

BIA Model Inputs

- Population
- Interventions
- Utilization
- Costs

Model Structure

- Model design
- Model outputs

Data Sources

- National
- Internal
- Assumptions

Key Model Inputs	Yes	No
Population size and characteristics		
Are all relevant populations included?		
Does the population mix accurately reflect our population?		
Is the sick population adequate size relative to the total population?		
Does the target population reflect current treatment guidelines?		
Does the treated population reflect treatment patterns in our health system?		
Is the effect in different populations modeled with subgroup analyses?		
Current technology mix		
Does the assumed treatment mix agree with patterns in our health system?		
Does the model allow for changes treatment mix?		
Does it allow for changes by population subgroups?		
Cost of current intervention mix		
Are modeled costs consistent with costs observed in our health system?		
New technology mix		
Is the time frame for adoption of new technology reasonable?		
Does the model allow for changes in the new technology mix?		
Cost of new technology mix		
Are the projected costs of the new technology reasonable?		
Use / cost of condition-related services or outcomes		
Does the model account for effect of new treatment in non-treated populations?		
Is the effect of the new treatment on the treated population captured in the model?		
Is the frequency and treatment of relevant side effects reasonable?		
Do the type and utilization of included health services agree with our trends?		
Are all relevant cost consequences of the existing and new technologies included in the model? (e.g., disease outcomes, tx monitoring, side effects, physician visits, etc.)		
Are modeled costs consistent with costs observed in our health system?		
Model Design		
Does the model design take into account key disease features (disease acuteness, natural course of illness, goal of therapy etc) and reflect them in the model design?		
Is the payer/insurer perspective clearly stated?		
Does the time horizon align with internal budgeting practices?		
Are all key data inputs obvious, transparent, documented, and modifiable?		
Model Outputs		
Is the total and incremental budget impact reported?		
Are the model results easy to identify and understand?		
Is annual resource use listed by contributing factor and reporting change over time?		
Are both aggregated and disaggregated costs reported over time?		
Can alternate assumptions be modeled using sensitivity analysis?		

Data Sources	Population size and characteristics	Current Treatment Mix	Current Treatment Cost	New Treatment Mix	New Treatment Cost	Cost/use of Services/ Outcomes
National						
Internal						

⁵ Hay JW et al. Budget Impact Models in MCO Decision Making: Current Practice, Challenges and Insights .

INITIAL REVIEW

What do the clinical guidelines say?

How is this drug typically used?

What do other institutions do?



Do the model parameters have external validity?

Can the parameters be reliably justified?

Did the model adequately quantify uncertainty?

How does this apply to my institution?

[All images licensed under CC BY-SA-NC](#)

FOLLOW-UP (6 MONTHS, 1 YEAR)

Outcomes Review

- Mortality
- ADRs
- Financial toxicity

Update criteria

- Additional exclusions
- Remove from formulary
- Drive utilization



[All images licensed under CC BY-SA-NC](#)

TEST QUESTION 5

Pharmacoeconomics modeling is a tool to limit treatment with proven benefits to only the cheapest therapies available:

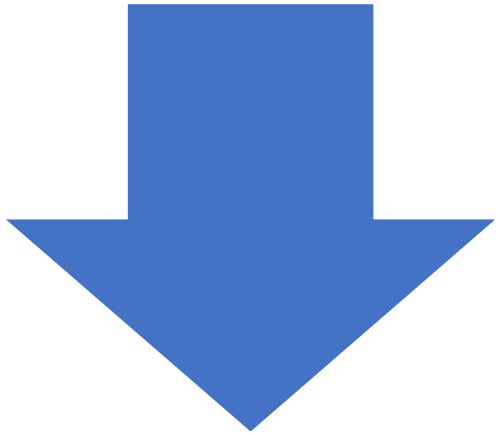
- a) True
- b) False

TEST QUESTION 5: ANSWER

Pharmacoeconomics modeling is a tool to limit treatment with proven benefits to only the cheapest therapies available:

- a) True
- b) False**

ONGOING CHALLENGES



“We’re giving our patients the best treatment possible.”

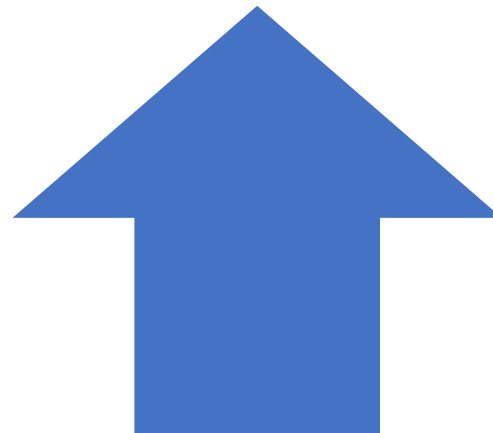
“People come here to get the treatment they need / want.”

“Outpatient administration is revenue-generating.”



“Should insurance dictate how you treat your patients?”

“Insurance will compensate by increasing premiums. Is that good for our patients?”



KEY TAKEAWAYS

As treatment costs continue to rise, formulary management **must consider the costs** associated with clinical outcomes.

Engagement of stakeholders and transparency are essential to the adoption and widespread **use of value-based strategies**.

Pharmacoeconomics can be an effective tool for **determining value and guiding rational use** of high-cost medications within an institution.

REFERENCE LIST

1. Neumann PJ, Sanders GD, Russell LB, et al. *Cost-Effectiveness in Health and Medicine*. 2nd ed. New York, NY: Oxford University Press; 2017.
2. Drummond MF, Sculpher MJ, Claxton K, et al. *Methods for the Economic Evaluation of Health Care Programmes*. 4th ed. Oxford, UK: Oxford Medical Publications; 2015.
3. Gong CL, Dasgupta S, Zangwill KM, Bolaris M, Hay JW. Early onset sepsis calculator-based management of newborns exposed to maternal intrapartum fever: a cost benefit analysis. *J Perinatol*. 2019 Apr;39(4):571-580. doi: 10.1038/s41372-019-0316-y. Epub 2019 Jan 28.
4. Gong CL and Hay JW. Cost-effectiveness analysis of abiraterone and sipuleucel-T in asymptomatic metastatic castration-resistant prostate cancer. *J Natl Compr Canc Netw*. 2014 Oct;12(10):1417-25. PMID: 25313181.
5. Hay JW et al. Budget Impact Models in MCO Decision Making: Current Practice, Challenges and Insights
6. Husereau D, Drummond M, Petrou S, et al. Consolidated Health Economic Evaluation Reporting Standards (CHEERS)— explanation and elaboration: a report of the ISPOR Health Economic Evaluations Publication Guidelines Good Reporting Practices Task Force. *Value Health*. 2013;16:231–250.
7. Sanders GD, Neumann PJ, Basu A, et al. Recommendations for conduct, methodological practices, and reporting of cost-effectiveness analyses: second panel on cost-effectiveness in health and medicine. *JAMA*. 2016;316(10):1093–1103.
8. Studdert AL, Gong CL, Srinivas S, Chin AL, Deresinski S. The Application of Pharmacoeconomics to Formulary Management in a Health-System Setting: A Novel Approach. *American Journal of Health System Pharmacy*. 2019 March 15. 76 (6): 381-6. doi: 10.1093/ajhp/zxy010. Epub 2019 Feb 21.
9. Prasad SA and Gong CL. (2020). Chapter 9: Evaluating Pharmacoeconomic Analyses. In YS Kauffman and DM Witt (Eds.). *The Essential Guide to Pharmacy Residency Research* (pp. XX-XX). Bethesda, Maryland: American Society of Health-System Pharmacists, Inc.

TEST QUESTIONS & ANSWERS	SLIDE #
1. The most commonly used perspective in a CEA model for the purposes of P&T is: Health plan	14-15
2. If the cost of Superchemo [®] is \$100,000 today, how much is it worth one year from now, assuming a discount rate of 3%? What about two years from now? \$97,000 in one year, ~\$94,000 in two years	19-20
3. If Superchemo [®] costs \$25,000 more than Regularchemo [®] and increases QALYs by 0.25, what is its ICER (\$/QALY)? \$100,000 / QALY	25-26
4. Assume your survival in health state A is 10 years. The utility weight for health state A is 0.7. How many quality-adjusted life years is health state A worth? 7 years	28-29
5. Pharmacoeconomics modeling is a tool to limit treatment with proven benefits to only the cheapest therapies available: False	41-42

**SESSION
CODE:**



**PHARMACY
VISION
20/20**

CSHP SEMINAR 20 • OCTOBER 21-25

Disneyland
RESORT