Professional Certificate in Pharmacoeconomics in Health Care

Pharmacoeconomic Evaluation Methods

Absolute Cost – The total monetary amount incurred for a health-care intervention, without reference to any comparator. total cost, direct cost It includes drug acquisition, administration, monitoring, and adverse-event management. Example: the absolute cost of a 12-month biologic therapy may be $45,000. Practical use: serves as the base for incremental cost-effectiveness ratios. Challenge: capturing all relevant cost components, especially indirect costs, can be difficult.

Adverse Event Cost – The incremental cost associated with managing side-effects of a treatment. AE cost, toxicity cost This may comprise hospital stays, laboratory tests, and physician visits. Example: chemotherapy-induced neutropenia may add $3,500 per episode. Practical application: incorporated into cost-utility models to reflect real-world resource use. Challenge: variability in event severity and treatment patterns across settings.

Aggregate Data – Summarized information pooled across individuals, such as mean costs or average quality-adjusted life years (QALYs). summary statistics, pooled data Used when patient-level data are unavailable. Example: a meta-analysis reports an average incremental cost of $2,000 for a new antihypertensive. Practical use: facilitates rapid health-technology assessment. Challenge: loss of variability and inability to adjust for covariates.

Budget Impact Analysis (BIA) – An economic evaluation that estimates the financial consequences of adopting a new intervention within a specific budget context. budgetary impact, financial projection It projects changes in expenditures over a defined time horizon. Example: a BIA might predict a $5 million increase in a regional health-authority's drug budget after introducing a novel anticoagulant. Practical application: informs formulary decisions and reimbursement negotiations. Challenge: requires accurate uptake forecasts and population estimates.

Cost-Benefit Analysis (CBA) – A method that expresses both costs and benefits in monetary terms, allowing direct comparison. monetary valuation, net benefit Benefits may be valued using willingness-to-pay or productivity gains. Example: a vaccination program yields $10 million in avoided medical costs and $5 million in productivity gains, against $3 million in program costs, resulting in a net benefit of $12 million. Practical use: supports policy decisions where monetary thresholds are appropriate. Challenge: ethical concerns arise when placing a monetary value on health outcomes such as life years.

Cost-Effectiveness Analysis (CEA) – An evaluation comparing the relative costs and outcomes (often in natural units) of two or more interventions. incremental cost-effectiveness ratio, ICER Outcomes may be life-years saved, symptom-free days, or cases averted. Example: a CEA finds that a new insulin formulation costs $8,000 per life-year saved compared with standard insulin. Practical application: helps determine whether an intervention provides good value for money. Challenge: selecting appropriate outcome measures and handling uncertainty.

Cost-Effectiveness Threshold – The maximum amount a decision-maker is willing to pay for a unit of health gain, such as a QALY. WTP threshold, willingness-to-pay Thresholds vary by country (e.g., $50,000/QALY in the United States, £20,000–£30,000/QALY in the United Kingdom). Practical use: guides interpretation of ICERs. Challenge: thresholds may be arbitrary and not reflect actual budget constraints.

Cost-Minimisation Analysis (CMA) – An economic evaluation used when interventions have proven equivalent effectiveness, focusing solely on cost differences. equivalence analysis, cost comparison Example: two generic antihypertensive drugs with identical blood-pressure control are compared; the cheaper drug is preferred. Practical application: simplifies decision-making when clinical outcomes are identical. Challenge: requires robust evidence of equivalence, which is often lacking.

Cost-Utility Analysis (CUA) – A form of CEA that incorporates quality-adjusted life years as the outcome measure, allowing comparison across disease areas. QALY, utility-based analysis Example: a CUA shows that a new oncology drug yields 0.8 additional QALYs at an incremental cost of $120,000, resulting in an ICER of $150,000/QALY. Practical use: widely accepted by health technology assessment agencies. Challenge: deriving reliable utility weights from patient populations.

Direct Cost – Expenses directly attributable to the provision of a health-care service, such as drug acquisition, hospital stays, and physician fees. medical cost, treatment cost Example: the direct cost of a surgical procedure includes operating-room time, implants, and anesthesia. Practical application: forms the core of most pharmacoeconomic models. Challenge: distinguishing direct from indirect costs, especially in complex care pathways.

Discount Rate – The percentage used to convert future costs and benefits into present-value terms, reflecting time preference. present-value factor, discounting Commonly applied rates are 3 % or 5 % per annum. Example: a cost incurred five years from now is multiplied by (1 + 0.03)^-5 to obtain its present value. Practical use: ensures comparability of costs and outcomes occurring at different times. Challenge: choice of rate can substantially affect results, and guidelines differ across jurisdictions.

Economic Evaluation – The systematic comparative analysis of alternative interventions in terms of both costs and consequences. health-economic analysis, value assessment Includes CBA, CEA, CUA, and CMA. Example: an economic evaluation may compare a new vaccine with existing immunisation schedules. Practical application: provides evidence for resource allocation decisions. Challenge: requires high-quality data on both costs and outcomes.

Health-Related Quality of Life (HRQoL) – A multidimensional concept reflecting an individual's perceived physical, mental, and social wellbeing as it relates to health. patient-reported outcome, QoL Measured using instruments such as EQ-5D or SF-36. Example: a chronic disease may reduce HRQoL, translating into lower utility scores. Practical use: utility values derived from HRQoL are essential for QALY calculations. Challenge:

cultural differences and response bias can affect instrument validity.

Incremental Cost-Effectiveness Ratio (ICER) – The ratio of the difference in costs to the difference in effectiveness between two interventions. cost-effectiveness ratio, incremental ratio Formula: (Cost_A − Cost_B) / (Effect_A − Effect_B). Example: an ICER of $25,000 per QALY suggests the new drug provides additional health benefit at that cost. Practical application: central metric for decision-makers. Challenge: interpretation is sensitive to uncertainty and threshold selection.

Incremental Net Benefit (INB) – An alternative to the ICER that expresses value in monetary terms, calculated as (WTP × ΔEffect) − ΔCost. net monetary benefit, NMB Positive INB indicates a cost-effective intervention at the specified willingness-to-pay. Example: with a WTP of $50,000/QALY, an intervention with ΔEffect = 0.6 QALY and ΔCost = $20,000 yields INB = $10,000. Practical use: facilitates statistical testing of cost-effectiveness. Challenge: requires a pre-specified WTP threshold.

Indirect Cost – Costs that arise from loss of productivity, caregiver time, or other non-medical resources associated with illness or treatment. productivity loss, societal cost Example: absenteeism due to chemotherapy side-effects may cost an employer $1,200 per patient. Practical application: included in societal perspective analyses. Challenge: quantifying intangible costs and attributing them correctly.

Markov Model – A mathematical modeling technique that represents health states and transitions over discrete time cycles. state-transition model, stochastic model Commonly used for chronic diseases where patients may move between states such as “stable,” “progressed,” and “dead.” Example: a Markov model for multiple sclerosis simulates yearly transitions among disability levels. Practical use: captures long-term costs and outcomes. Challenge: requires accurate transition probabilities and may oversimplify complex pathways.

Monte Carlo Simulation – A probabilistic technique that repeatedly samples input parameters from defined distributions to assess uncertainty in model outcomes. probabilistic sensitivity analysis, PSA Generates cost-effectiveness acceptability curves. Example: 10,000 iterations estimate the probability that a new drug is cost-effective at a $30,000/QALY threshold. Practical application: informs decision-makers about robustness of results. Challenge: computationally intensive and dependent on appropriate choice of distributions.

Net Monetary Benefit (NMB) – A reformulation of the ICER that expresses net value in monetary terms: (WTP × Effect) − Cost. monetary benefit, benefit-cost Positive NMB indicates cost-effectiveness. Example: with a WTP of $100,000 per QALY, an intervention delivering 0.5 QALY at $30,000 yields NMB = $20,000. Practical use: simplifies statistical comparison across interventions. Challenge: interpretation hinges on the selected WTP.

Net Health Benefit (NHB) – The health equivalent of NMB, calculated as (Effect) − (Cost / WTP). health benefit, cost-adjusted effect Example: an intervention with 1.2 QALYs and cost $40,000 at a WTP of $50,000 yields NHB = 0.8 QALYs. Practical application: provides a single health metric for ranking alternatives. Challenge:

same dependence on WTP as NMB.

Probabilistic Sensitivity Analysis (PSA) – A method that assigns probability distributions to uncertain parameters and propagates this uncertainty through the model. Monte Carlo, uncertainty analysis Results are presented as cost-effectiveness planes or acceptability curves. Example: PSA shows a 70 % probability that a drug is cost-effective at $25,000/QALY. Practical use: aids transparent decision-making. Challenge: requires specification of appropriate distributions for each parameter.

Quality-Adjusted Life Year (QALY) – A composite measure that combines length of life with health-state utility, where 1 QALY equals one year in perfect health. utility-adjusted survival, health-adjusted life year Example: a patient lives 2 years with a utility of 0.7, resulting in 1.4 QALYs. Practical application: standard outcome in CUAs and health-technology assessments. Challenge: utility measurement can be subjective and varies by instrument.

Reference Case – A set of methodological assumptions (perspective, discount rate, time horizon, etc.) recommended by health-technology assessment bodies for consistency across analyses. standard scenario, methodological baseline Example: the NICE reference case uses a 3.5 % discount rate and a health-service perspective. Practical use: ensures comparability of published studies. Challenge: may not reflect local practice or specific stakeholder preferences.

Societal Perspective – An analytical viewpoint that includes all costs and benefits, regardless of who incurs them, encompassing direct medical, direct non-medical, and indirect costs. population perspective, comprehensive viewpoint Example: a societal analysis of a vaccination program adds productivity gains from avoided illness. Practical application: aligns with many global health-economics guidelines. Challenge: data collection is extensive, and some decision-makers prioritize narrower perspectives.

Time Horizon – The period over which costs and outcomes are measured in an economic evaluation. analysis duration, study period Long time horizons capture all relevant downstream effects; short horizons may miss late benefits or costs. Example: a 20-year horizon for a chronic disease therapy versus a 5-year horizon for an acute intervention. Practical use: influences model complexity and data requirements. Challenge: projecting beyond available data introduces uncertainty.

Willingness-to-Pay (WTP) – The maximum amount a society or individual is prepared to spend for a unit of health gain, often expressed per QALY. threshold value, monetary ceiling WTP thresholds guide interpretation of ICERs. Example: a health system may adopt a $45,000/QALY WTP threshold. Practical application: central to cost-effectiveness decision rules. Challenge: thresholds are often debated and may not reflect actual budget constraints.

Adherence-Adjusted Cost-Effectiveness – An analysis that incorporates real-world medication adherence rates, affecting both costs and effectiveness. compliance-adjusted evaluation, adherence impact Example: lower adherence to an oral anticoagulant reduces its effectiveness, raising the ICER. Practical use: improves relevance of model outcomes to clinical practice. Challenge: adherence data are variable and may be difficult to obtain.

Budget-Constrained Optimization – A decision-making approach that seeks the combination of interventions delivering maximal health benefit within a fixed budget. resource allocation, constrained maximization Often solved using linear programming techniques. Example: allocating limited funds among vaccines to achieve the greatest QALYs gained. Practical application: assists policymakers in prioritizing interventions. Challenge: requires accurate estimation of costs and health gains for each option.

Cost-Effectiveness Plane – A graphical representation plotting incremental costs on the vertical axis and incremental effectiveness on the horizontal axis, dividing the plane into four quadrants. ICER plot, outcome space The northeast quadrant indicates higher cost and higher effectiveness; the southeast quadrant denotes lower cost and higher effectiveness (dominant). Example: a point in the southeast quadrant signifies a cost-saving, more effective intervention. Practical use: visual tool for interpreting results. Challenge: interpretation can be ambiguous when points lie near the axes.

Decision Analytic Model – A structured framework (e.g., decision tree, Markov model) that simulates the pathways, costs, and outcomes of health interventions. modeling framework, analytic structure Enables systematic comparison of alternatives. Example: a decision tree for acute myocardial infarction treatment pathways. Practical application: central to pharmacoeconomic evaluations. Challenge: model validity depends on quality of input data and assumptions.

Deterministic Sensitivity Analysis – A technique that varies one parameter at a time to assess its impact on results, often displayed as tornado diagrams. one-way sensitivity, parameter testing Highlights key drivers of cost-effectiveness. Example: varying drug price between $10,000 and $30,000 changes the ICER from $20,000/QALY to $60,000/QALY. Practical use: identifies parameters requiring robust data. Challenge: does not capture interactions between parameters.

Discounted Cost-Effectiveness Ratio – An ICER calculated after applying discount rates to both costs and outcomes, reflecting present-value comparisons. present-value ICER, discounted ratio Ensures that future costs and benefits are appropriately weighted. Example: a discounted ICER of $35,000/QALY may differ from an undiscounted value. Practical application: aligns with guidelines that mandate discounting. Challenge: selection of discount rates can be contentious.

Health Technology Assessment (HTA) – A multidisciplinary process that evaluates the medical, social, economic, and ethical implications of health technologies. technology appraisal, assessment process HTA bodies (e.g., NICE, CADTH) often rely on pharmacoeconomic evidence. Example: an HTA report recommends reimbursement for a new oncology drug based on CUA results. Practical use: informs policy, pricing, and coverage decisions. Challenge: balancing scientific rigor with timeliness and stakeholder interests.

Incremental Net Monetary Benefit (INMB) – The difference in NMB between two interventions, used to assess cost-effectiveness across varying WTP thresholds. incremental NMB, net benefit difference Positive INMB indicates the intervention is cost-effective at the chosen WTP. Example: INMB of $5,000 at a $50,000/

QALY threshold suggests favorable value. Practical application: facilitates statistical testing of cost-effectiveness. Challenge: depends on accurate estimation of both costs and utilities.

Life-Year Gained (LYG) – A measure of survival benefit that counts additional years of life achieved by an intervention, without adjusting for quality. survival gain, additional years Example: a new vaccine prevents premature death, yielding 0.3 LYG per vaccinated individual. Practical use: alternative outcome metric when quality-adjusted measures are unavailable. Challenge: ignores morbidity and patient preferences.

Markov Cohort Model – A specific type of Markov model that follows a defined cohort of patients through health states over time, assuming homogenous transition probabilities. cohort simulation, state-based model Example: a cohort of diabetic patients progresses annually from “no complications” to “microvascular complications.” Practical application: efficient for large populations where individual heterogeneity is minimal. Challenge: may oversimplify patient-level variability.

Micro-Costing – A detailed costing approach that itemizes each resource used, often at the unit level, to estimate total cost. bottom-up costing, detailed costing Example: calculating the cost of a chemotherapy regimen by summing drug dose, infusion time, nursing hours, and consumables. Practical use: provides precise cost estimates for high-technology interventions. Challenge: time-consuming and requires granular data.

Monte Carlo Probabilistic Model – A model that incorporates randomness by repeatedly sampling from probability distributions for all uncertain parameters simultaneously. stochastic simulation, random sampling Generates a distribution of possible outcomes rather than a single point estimate. Example: estimating the probability that a new drug’s ICER falls below $30,000/QALY across 5,000 simulations. Practical application: informs risk-adjusted decision-making. Challenge: requires specification of joint distributions and may be computationally demanding.

Net Cost – The difference between total costs of an intervention and any cost offsets (e.g., avoided hospitalizations). adjusted cost, residual cost Example: a drug costing $15,000 per patient may have $5,000 in avoided hospitalization costs, yielding a net cost of $10,000. Practical use: provides a more realistic estimate of financial impact. Challenge: accurate estimation of offset costs is often uncertain.

Opportunity Cost – The value of the best alternative foregone when resources are allocated to a particular intervention. alternative cost, forgone benefit In health care, spending on a high-cost drug may preclude funding for preventive services. Practical application: underscores the need for efficient resource allocation. Challenge: quantifying opportunity costs is inherently speculative.

Patient-Reported Outcome Measures (PROMs) – Instruments that capture patients’ perspectives on health status, symptoms, and quality of life. self-reported outcomes, health surveys PROMs inform utility estimation for QALY calculations. Example: the EQ-5D questionnaire provides a utility score used in CUAs. Practical use: enhances relevance of economic evaluations to patient experience. Challenge: selection of appropriate PROMs and cultural adaptation.

Pharmacoeconomic Model Validation – The process of checking that a model accurately represents the real-world system it intends to simulate. model verification, credibility assessment Validation may involve internal checks, external comparisons, and expert review. Example: comparing model-predicted survival rates with registry data. Practical application: builds confidence in model outputs for decision-makers. Challenge: limited availability of high-quality external data.

Probabilistic Decision Tree – A decision-analytic structure where branches are assigned probability distributions, allowing uncertainty propagation through the tree. stochastic decision analysis, random branch Example: a tree evaluating different diagnostic pathways for a disease, each with probabilistic outcomes. Practical use: suitable for short-term, discrete events. Challenge: complexity grows rapidly with many branches.

Quality-Adjusted Life Day (QALD) – A finer resolution of the QALY, representing a single day of life adjusted for quality. daily utility, granular QALY Useful when modelling short-term interventions. Example: a 30-day treatment yielding a utility of 0.8 results in 24 QALDs (30 × 0.8). Practical application: improves precision in acute-care models. Challenge: data on daily utilities are rarely collected.

Reference Case Sensitivity – Examination of how results change when key reference-case assumptions (e.g., perspective, discount rate) are varied. scenario analysis, assumption testing Example: altering the discount rate from 3 % to 5 % shifts the ICER by 15 %. Practical use: assesses robustness of conclusions. Challenge: selection of alternative assumptions must be justified.

Scenario Analysis – Evaluation of model outcomes under alternative sets of assumptions or input values representing plausible real-world conditions. what-if analysis, alternative scenario Example: a scenario where drug price is reduced by 20 % due to a negotiated discount. Practical application: informs negotiations and policy planning. Challenge: may produce numerous scenarios, complicating interpretation.

Sensitivity Threshold – The value of a parameter at which the cost-effectiveness conclusion changes (e.g., from cost-effective to not). break-point, critical value Example: a drug becomes cost-effective only if its price falls below $12,000 per patient. Practical use: highlights price negotiation targets. Challenge: thresholds depend on model structure and other interacting variables.

Standardized Cost-Effectiveness Ratio (SCER) – An ICER that has been adjusted to a common currency and price year, facilitating cross-study comparisons. adjusted ICER, normalized ratio Example: converting an ICER from euros 2015 to US dollars 2022 using purchasing power parity and inflation indices. Practical application: enables meta-analysis of economic evaluations. Challenge: accurate conversion requires reliable economic data.

Survival Analysis – Statistical methods (e.g., Kaplan–Meier, Cox proportional hazards) used to estimate time-to-event outcomes, often feeding into economic models. time-to-event, hazard modeling Example: survival curves for a cancer drug inform transition probabilities in a Markov model. Practical use: provides realistic estimates of disease progression. Challenge: censoring and extrapolation beyond observed data can introduce bias.

Utility Weight – A numeric value between 0 (death) and 1 (perfect health) reflecting the desirability of a health state. preference score, health state value Derived from instruments like EQ-5D, SG, or TTO. Example: a utility weight of 0.55 for moderate chronic pain. Practical application: core input for QALY calculations. Challenge: variability across populations and measurement methods.

Value of Information (VOI) Analysis – An approach that quantifies the benefit of obtaining additional data to reduce decision uncertainty. information gain, EVPI Expected Value of Perfect Information (EVPI) estimates the maximum worth of eliminating all uncertainty. Example: an EVPI of $2 million suggests further research could be justified. Practical use: guides research prioritization. Challenge: computationally intensive and relies on robust probabilistic models.

Willingness-to-Pay Curve – A graphical representation showing the probability that an intervention is cost-effective across a range of WTP thresholds. acceptability curve, cost-effectiveness probability Derived from PSA results. Example: at $30,000/QALY, the curve shows a 60 % probability of cost-effectiveness. Practical application: aids stakeholders in interpreting uncertainty. Challenge: interpretation can be ambiguous when curves intersect.

Zero-Cost Incremental Analysis – An evaluation where the incremental cost between two interventions is negligible, focusing solely on differences in effectiveness. cost-neutral comparison, free-difference Example: two formulations of the same drug with identical acquisition costs but differing adherence rates. Practical use: highlights non-monetary benefits. Challenge: true zero cost is rare; hidden costs may exist.