

Economic Evaluation in Healthcare

Adverse Event Cost (related terms: direct cost, indirect cost, health resource utilization) – The monetary value associated with medical complications arising from treatment, including hospital stays, medication, and lost productivity. Example: estimating the cost of chemotherapy-induced neutropenia helps decide if a supportive drug is cost-effective. Challenges include capturing long-term sequelae and variations across health systems.

Aggregate Cost (related terms: total cost, fixed cost, variable cost) – The sum of all costs incurred by a health intervention across a defined population. It is used in budget impact analyses to forecast expenditures for payers. Practical use: aggregating costs of a vaccination program to assess affordability. Difficulty lies in obtaining accurate population-level utilization data.

Annualized Cost (related terms: annualization, discounting, time horizon) – Converting one-time or multi-year costs into an equivalent yearly amount, typically by applying a discount rate. This facilitates comparison of interventions with different durations, such as a surgical implant versus a drug regimen. A key challenge is selecting an appropriate discount rate that reflects societal time preferences.

Benefit-Cost Ratio (BCR) (related terms: net benefit, willingness-to-pay, economic surplus) – A metric that divides total monetary benefits by total costs; a BCR greater than 1 indicates that benefits outweigh costs. Used in public health to justify investments like smoking-cessation programs. Limitations include difficulty assigning monetary values to health outcomes and potential double-counting of benefits.

Budget Impact Analysis (BIA) (related terms: cost-consequence analysis, payer perspective, financial forecasting) – An assessment of the financial consequences of adopting a new health technology within a specific budget context over a short- to medium-term horizon (typically 1-5 years). Example: estimating the annual budget impact of a novel oncology drug for a national health service. Challenges include projecting uptake rates and accounting for price negotiations.

Cost (related terms: direct cost, indirect cost, opportunity cost) – The monetary value of resources consumed in delivering a health intervention, expressed in a common currency. Costs can be categorized as medical (e.g., drugs, procedures) or non-medical (e.g., transportation). Accurate cost measurement is essential for any economic evaluation but is often hampered by fragmented data sources.

Cost-Benefit Analysis (CBA) (related terms: benefit-cost ratio, net monetary benefit, willingness-to-pay) – A method that compares the total monetary value of benefits to the total costs of an intervention. Results are expressed either as a net benefit (benefits minus costs) or as a ratio. Example: evaluating a community-based hypertension program by monetizing reduced stroke incidence. Main difficulty is converting health outcomes into monetary terms without bias.

Cost-Effectiveness Analysis (CEA) (related terms: incremental cost-effectiveness ratio, willingness-to-pay

threshold, health outcomes) – An evaluation that compares the relative costs and health effects of two or more interventions, with outcomes expressed in natural units such as life-years gained. The primary output is the incremental cost-effectiveness ratio (ICER). Practical use: deciding whether a new anticoagulant is worth its higher price compared with warfarin. Challenges include selecting appropriate comparators and dealing with uncertainty in clinical effectiveness.

Cost-Effectiveness Threshold (CET) (related terms: willingness-to-pay, ICER, societal value) – The maximum amount a society is prepared to pay for an additional unit of health benefit (e.g., per quality-adjusted life-year). Thresholds guide reimbursement decisions; for instance, the UK's National Institute for Health and Care Excellence often uses £20 000-£30 000 per QALY. Determining a credible threshold is contentious because it must reflect budget constraints, opportunity costs, and ethical considerations.

Cost-Utility Analysis (CUA) (related terms: QALY, utility, health state valuation) – A form of CEA that incorporates patient preferences by measuring outcomes in quality-adjusted life-years. CUAs allow comparison across disease areas because they standardize benefits. Example: evaluating a gene therapy for a rare disease using QALYs to compare with conventional treatments. Issues include variability in utility measurement methods (e.g., EQ-5D vs. SF-6D) and cultural differences in health state preferences.

Cost-of-Illness (COI) Study (related terms: direct cost, indirect cost, prevalence) – An observational analysis that quantifies the economic burden of a disease, typically separating costs into direct medical, direct non-medical, and indirect components such as productivity loss. COI studies inform priority setting and resource allocation. Limitations are that they do not assess the value of interventions, only the burden of disease.

Discount Rate (related terms: present value, time preference, future cost) – The percentage used to convert future costs and benefits into present-value terms, reflecting the societal preference for current consumption over future consumption. Standard rates range from 3 % to 5 % in many health-economic guidelines. Selecting an inappropriate discount rate can distort the perceived value of long-term interventions like vaccines.

Health Economic Model (related terms: decision-tree, Markov model, simulation) – A structured analytical framework that simulates the costs and outcomes of health interventions over time. Models can be deterministic or probabilistic and are essential when trial data are limited. Example: a Markov model projecting the lifetime costs and QALYs of a chronic disease therapy. Model validation and transparency are major challenges.

Health Outcome (related terms: clinical endpoint, utility, survival) – Any measurable change in health status resulting from an intervention, ranging from clinical events (e.g., myocardial infarction) to patient-reported outcomes (e.g., pain scores). Outcomes are the denominator in economic ratios. Accurate measurement requires validated instruments and consistent follow-up.

Health Technology Assessment (HTA) (related terms: policy evaluation, reimbursement, evidence synthesis) – A multidisciplinary process that evaluates the clinical, economic, ethical, and social implications of a health technology to inform policy decisions. HTA agencies (e.g., NICE, CADTH) use economic evaluations as a core

component. The complexity of integrating multiple evidence streams poses methodological and political challenges.

Incremental Cost-Effectiveness Ratio (ICER) (related terms: cost-effectiveness, incremental cost, incremental effect) – The ratio of the difference in costs to the difference in effectiveness between two interventions. It is the principal metric for judging whether an intervention provides good value for money. Example: an ICER of \$50 000 per QALY for a new biologic compared with standard therapy. Interpretation depends on the chosen willingness-to-pay threshold and the uncertainty around the estimate.

Incremental Net Benefit (INB) (related terms: net monetary benefit, willingness-to-pay, cost-effectiveness) – An alternative to the ICER that translates cost-effectiveness into monetary terms: $INB = (\lambda \times \Delta E) - \Delta C$, where λ is the willingness-to-pay threshold, ΔE is the incremental effect, and ΔC is the incremental cost. Positive INB indicates cost-effectiveness. This approach simplifies statistical analysis and is useful in probabilistic sensitivity analysis.

Life-Year Gained (LYG) (related terms: survival benefit, mortality reduction, effectiveness) – The additional years of life that an intervention provides compared with a comparator. LYGs are often used in CEAs when quality adjustment is not applied. Example: a cardiac stent that adds 0.8 LYG per patient. LYGs ignore quality of life, which can misrepresent the true value of interventions that extend life with poor health states.

Markov Model (related terms: state transition, cycle length, health economic model) – A type of decision-analytic model that represents disease progression as a series of health states with defined transition probabilities over discrete time cycles. Widely used for chronic diseases where patients can move between states such as “stable,” “progressed,” and “dead.” The model’s accuracy depends on reliable transition data and appropriate cycle length.

Net Monetary Benefit (NMB) (related terms: ICER, willingness-to-pay, incremental cost) – A reformulation of the ICER that expresses cost-effectiveness as a single monetary value: $NMB = (\lambda \times \Delta E) - \Delta C$. Positive NMB indicates that the intervention is worth its cost at the given λ . NMB simplifies decision rules in probabilistic analyses and facilitates comparison across multiple interventions.

Opportunity Cost (related terms: resource allocation, alternative use, economic trade-off) – The value of the best alternative foregone when resources are allocated to a particular health intervention. In health economics, opportunity cost reflects the health gains that could have been achieved elsewhere with the same budget. Quantifying opportunity cost is difficult because it requires estimating the marginal productivity of the health system.

Outcome Measure (related terms: endpoint, utility, clinical efficacy) – The specific variable used to assess the effect of an intervention, such as mortality, disease-specific symptom scores, or QALYs. Selecting appropriate outcome measures is crucial for relevance and comparability across studies. Challenges include aligning clinical endpoints with health-economic outcomes and ensuring measurement validity.

Patient-Reported Outcome Measures (PROMs) (related terms: quality of life, utility, health state valuation) – Instruments that capture patients’ perspectives on their health status, symptoms, and functional abilities. PROMs are used to derive utilities for CUA and to enrich CEAs with real-world relevance. Example: the

EQ-5D questionnaire provides health state utilities for QALY calculations. Limitations involve respondent burden and cultural adaptation.

Pharmacoeconomic Evaluation (related terms: cost-effectiveness analysis, budget impact, HTA) – The systematic assessment of the value of pharmaceutical products, integrating costs, clinical benefits, and sometimes broader societal impacts. It guides formulary decisions and pricing negotiations. A major challenge is the rapid emergence of high-cost specialty drugs that strain traditional evaluation frameworks.

Population-Based Cost-Effectiveness (related terms: public health, societal perspective, aggregate cost) – An evaluation that considers the costs and benefits of an intervention for an entire target population rather than individual patients. Useful for interventions like vaccination campaigns where herd immunity creates spill-over benefits. Requires robust epidemiological data and assumptions about coverage rates.

Probabilistic Sensitivity Analysis (PSA) (related terms: Monte Carlo simulation, uncertainty, cost distribution) – A technique that assigns probability distributions to uncertain parameters and repeatedly samples them to generate a distribution of outcomes (e.g., ICERs). PSA quantifies the probability that an intervention is cost-effective at various willingness-to-pay thresholds. Implementation demands computational resources and careful selection of appropriate distributions.

Quality-Adjusted Life-Year (QALY) (related terms: utility, health state, cost-utility analysis) – A composite measure that combines length of life with quality of life, where each year is weighted by a utility value between 0 (death) and 1 (perfect health). QALYs enable comparison across disease areas. Example: a treatment that yields 0.7 QALY per year for five years provides 3.5 QALYs. Limitations include ethical concerns about valuing life differently across ages or disabilities.

Quality-Adjusted Life-Year (QALY) – Disability-Adjusted Life-Year (DALY) Comparison (related terms: global burden, health economics, outcome measure) – While QALY measures health gains, DALY quantifies health loss by combining years of life lost with years lived with disability. Both are used in resource allocation, but DALYs are more common in global health assessments. Understanding the methodological differences is essential when translating findings across contexts.

Reference Case (related terms: guideline, standard assumptions, HTA) – A set of methodological standards (e.g., perspective, discount rate, time horizon) recommended by health-economic agencies for conducting consistent economic evaluations. Following the reference case ensures comparability across submissions to bodies like NICE or CADTH. Deviations must be justified and may affect acceptance.

Resource Utilization (related terms: cost, health services, utilization data) – The quantity of health-care services (e.g., hospital days, physician visits, laboratory tests) consumed by patients during an evaluation. Accurate measurement of resource utilization underpins cost estimation. Data sources include claims databases, electronic health records, and prospective trial collection. Incomplete capture leads to biased cost estimates.

Scenario Analysis (related terms: deterministic sensitivity, alternative assumptions, model testing) – Exploration of how results change under distinct sets of assumptions (e.g., best-case, worst-case, alternative uptake). Scenario analysis complements PSA by highlighting the impact of structural choices such as

treatment duration or price discounts. It helps decision-makers understand the robustness of conclusions under plausible real-world conditions.

Sensitivity Analysis (related terms: deterministic, probabilistic, uncertainty) – A systematic approach to test how variations in input parameters affect the outcomes of an economic evaluation. Types include one-way, multi-way, and threshold analyses. Sensitivity analysis is essential for transparent reporting and for identifying key drivers of cost-effectiveness.

Societal Perspective (related terms: cost-benefit analysis, indirect cost, broader impact) – An analytical viewpoint that includes all costs and benefits regardless of who incurs them, encompassing direct medical costs, non-medical costs, and productivity losses. This perspective aligns with welfare economics but is less commonly required by national HTA agencies, which often adopt a health-system perspective.

Standard of Care (SOC) (related terms: comparator, clinical guideline, usual practice) – The treatment that represents the current best practice against which new interventions are compared in economic evaluations. Defining SOC accurately is critical because it influences the incremental cost and effect calculations. In rapidly evolving therapeutic areas, SOC may change during the assessment period, adding complexity.

State Transition Model (related terms: Markov model, health states, transition probabilities) – A framework where individuals move among defined health states over time according to probabilities, allowing estimation of long-term costs and outcomes. The model can incorporate absorbing states (e.g., death) and recurring events (e.g., hospitalizations). Specification of transition probabilities often relies on clinical trial data or epidemiologic studies.

Time Horizon (related terms: duration, long-term analysis, discounting) – The period over which costs and outcomes are measured in an economic evaluation. A lifetime horizon captures all relevant future effects, while a shorter horizon may be appropriate for budget impact analyses. Selecting an inappropriate horizon can either overstate benefits (by truncating later costs) or understate them (by missing delayed effects).

Utility (related terms: preference weight, QALY, health state valuation) – A numeric representation of the desirability of a health state, anchored at 0 (death) and 1 (perfect health). Utilities are elicited using techniques such as standard gamble, time trade-off, or rating scales. They are fundamental inputs for CUAs. Variability across populations and measurement methods can affect the comparability of QALYs.

Value of Information (VOI) Analysis (related terms: expected value of perfect information, decision uncertainty, research prioritization) – A method that quantifies the monetary benefit of obtaining additional information to reduce decision uncertainty. VOI helps determine whether further research (e.g., a new trial) is worth its cost. Challenges include computational intensity and the need for robust probabilistic models.

Willingness-to-Pay (WTP) Threshold (related terms: cost-effectiveness threshold, societal value, ICER) – The maximum amount a decision-maker is prepared to spend for a unit of health gain (e.g., per QALY). WTP thresholds differ across countries and may be explicit (e.g., \$100 000 per QALY in the US) or implicit (derived from historical reimbursement decisions). Setting an appropriate threshold is politically sensitive and may influence market access strategies.

Yield of Economic Evaluation (related terms: decision impact, cost-effectiveness, policy relevance) – The practical influence an economic evaluation has on health-policy decisions, such as formulary inclusion, pricing negotiations, or guideline updates. A high yield indicates that the study's methods, transparency, and relevance align with stakeholder needs. Barriers to yield include limited stakeholder engagement and poor communication of results.

Zero-Cost Intervention (related terms: cost-saving, negative cost, free program) – An intervention that incurs no additional financial outlay for the health system, often because costs are offset by savings elsewhere (e.g., a preventive program that reduces hospitalizations). While attractive, claims of zero cost must be substantiated with rigorous accounting of all resource flows.

Adoption Curve (related terms: diffusion of innovation, market uptake, scenario analysis) – The pattern describing how quickly a new health technology is embraced by providers and patients over time. Modeling adoption curves helps predict future budget impact and cost-effectiveness, especially for high-cost therapies. Uncertainty in adoption rates is a common source of variability in long-term analyses.

Benefit-Risk Assessment (related terms: clinical efficacy, safety profile, health economics) – The systematic evaluation of an intervention's therapeutic benefits relative to its adverse effects. In health economics, benefit-risk considerations influence the choice of comparators and may affect the weighting of outcomes in a CUA. Integrating quantitative benefit-risk data into economic models remains a methodological challenge.

Comparative Effectiveness Research (CER) (related terms: real-world evidence, head-to-head trials, HTA) – Studies that directly compare the outcomes of two or more interventions in routine clinical practice. CER provides the effectiveness inputs required for CEAs when randomized controlled trial data are unavailable or not generalizable. Data quality, confounding, and selection bias are key concerns.

Cost-Effectiveness Plane (related terms: ICER, quadrants, decision rule) – A graphical representation with four quadrants: incremental cost on the vertical axis and incremental effectiveness on the horizontal axis. The position of an intervention relative to the origin helps interpret whether it is more effective and more costly, less effective and less costly, etc. The plane is useful for visualizing PSA results (e.g., cost-effectiveness acceptability curves).

Discounted Net Benefit (related terms: present value, NMB, time preference) – The net monetary benefit calculated after applying the discount rate to both costs and effects, ensuring that future values are appropriately weighted. This metric is essential when comparing interventions with differing time horizons.

Economic Burden (related terms: cost-of-illness, societal cost, health expenditure) – The total financial impact of a disease on a society, encompassing direct medical costs, direct non-medical costs, and indirect costs such as lost productivity. Understanding economic burden assists policymakers in prioritizing interventions.

Equity-Weighted QALY (related terms: distributional cost-effectiveness analysis, social value, weighting factor) – An adaptation of the standard QALY that applies higher weights to health gains achieved by disadvantaged groups, reflecting societal preferences for reducing health inequities. Incorporating equity

weights can alter the cost-effectiveness ranking of interventions, but selecting appropriate weights is contentious.

External Validity (related terms: generalizability, real-world evidence, applicability) – The extent to which the results of an economic evaluation can be applied to settings beyond the study sample. High external validity enhances the usefulness of HTA submissions across jurisdictions. Threats include differences in clinical practice, cost structures, and patient demographics.

Health-Adjusted Life Expectancy (HALE) (related terms: QALY, population health, life expectancy) – A summary measure that adjusts life expectancy for the average health quality of a population, expressed in years lived in full health. HALE provides a macro-level perspective for public-health planning and can be used as a benchmark for evaluating population-wide interventions.

Incremental Cost-Utility Ratio (ICUR) (related terms: ICER, QALY, cost-utility analysis) – The ratio of incremental cost to incremental QALYs gained, synonymous with the ICER when outcomes are measured in QALYs. The ICUR is the standard metric reported in CUAs.

Input Parameter (related terms: model variable, sensitivity analysis, data source) – Any numerical value used in an economic model, such as cost, utility, transition probability, or discount rate. The credibility of an evaluation hinges on the quality of input parameters.

Markov Cohort Model (related terms: state transition, deterministic, health economic model) – A specific type of Markov model that follows a cohort of patients through health states over multiple cycles, assuming that all individuals in a state experience the same transition probabilities.

Medical Necessity (related terms: coverage decision, payer criteria, clinical indication) – A determination by a payer that a service or product is essential for diagnosis or treatment. Economic evidence often supports the justification of medical necessity in reimbursement submissions.

Model Calibration (related terms: validation, parameter fitting, goodness-of-fit) – The process of adjusting model parameters so that model outputs align with observed real-world data. Calibration improves confidence in model predictions, especially when direct data are scarce.

Model Validation (related terms: internal validation, external validation, credibility) – The assessment of whether a model accurately predicts outcomes, using techniques such as cross-validation, comparison with independent data sets, or expert review.

Net Health Benefit (NHB) (related terms: QALY, utility, incremental effect) – The total health gain from an intervention after accounting for both benefits and harms, often expressed in QALYs.

Patient Access Scheme (PAS) (related terms: risk-sharing, price discount, reimbursement) – Agreements between manufacturers and payers that provide the drug at a reduced price or with performance-based rebates to improve affordability and access. Economic evaluations inform the design of PAS by estimating the value of risk-sharing.

Pharmacoeconomic Guidelines (related terms: reference case, methodological standards, HTA) – Official

documents issued by health authorities that outline the preferred methods for conducting economic evaluations, including perspective, discounting, and outcome measures.

Population-Based Modeling (related terms: epidemiologic model, aggregate cost, public health) – Modeling approaches that estimate the impact of interventions on entire populations, often using demographic and disease prevalence data.

Probabilistic Model (related terms: Monte Carlo simulation, PSA, uncertainty) – A model that incorporates randomness by assigning probability distributions to inputs, allowing the generation of a distribution of outcomes.

Quality-Adjusted Life-Year (QALY) Threshold (related terms: WTP, CET, cost-effectiveness) – The monetary value set by a health system to determine whether an intervention's cost per QALY is acceptable.

Real-World Evidence (RWE) (related terms: observational data, registry, CER) – Data collected outside of randomized controlled trials, such as from electronic health records, claims databases, or patient registries. RWE provides inputs for effectiveness, resource use, and cost in economic evaluations.

Reference Comparator (related terms: standard of care, control arm, baseline) – The established treatment against which a new intervention is compared in an economic analysis.

Scenario Modeling (related terms: deterministic, alternative assumptions, policy analysis) – Building distinct model structures to reflect different plausible futures, such as varying adoption rates or price changes.

Sensitivity Parameter (related terms: input parameter, variation, tornado diagram) – A specific model input that is varied during sensitivity analysis to assess its impact on the results.

Societal Willingness-to-Pay (related terms: threshold, public opinion, health value) – The aggregate amount society is prepared to invest for health improvements, often inferred from surveys or historical spending patterns.

Stakeholder Engagement (related terms: consultation, HTA, decision-making) – Involving relevant parties such as clinicians, patients, payers, and industry in the design and interpretation of economic evaluations.

Step-Down Costing (related terms: allocation, overhead, cost apportionment) – A method for distributing indirect costs (e.g., administrative overhead) to specific services based on usage metrics.

Time-Dependent Transition Probabilities (related terms: Markov model, hazard rate, survival analysis) – Transition probabilities that change over time, reflecting that the risk of moving between health states can vary as patients age or disease progresses.

Utility Elicitation (related terms: standard gamble, time trade-off, preference measurement) – The process of obtaining utility values from patients or the general public using structured techniques.

Value-Based Pricing (related terms: cost-effectiveness, price negotiation, HTA) – Setting a drug's price based on the health benefits it delivers, often linked to its ICER relative to a threshold.

Virtual Twin Model (related terms: digital twin, simulation, personalized medicine) – A computational replica of an individual patient used to predict outcomes under different treatment scenarios, enabling individualized cost-effectiveness analysis.

Weighted Average Cost (related terms: average cost, volume, price mix) – An average that reflects the proportion of each cost component in the overall mix, useful when multiple price tiers exist.

Zero-Order Discounting (related terms: first-order, discount function, present value) – Applying a constant discount rate without adjusting for changes in the rate over time; the standard approach in most health-economic guidelines.