Professional Certificate in Health Economics and Market Access

Healthcare Systems and Policy

Access – related terms: availability, equity, barriers. Access refers to the degree to which patients can obtain needed health services, medicines, and technologies. It encompasses geographic, financial, cultural, and systemic dimensions. For example, a rural clinic that offers tele-medicine expands access to specialty care. In market access strategy, improving access may involve patient assistance programs, insurance coverage negotiations, and supply-chain optimisation. Challenges include addressing disparities, navigating payer formularies, and ensuring sustainable financing while maintaining quality of care.

Benefit-Risk Assessment – related terms: safety profile, efficacy, therapeutic window. This systematic evaluation weighs the positive therapeutic effects of a health intervention against its potential adverse outcomes. Regulatory agencies such as the FDA and EMA require a thorough benefit-risk assessment before approval. A practical application is the use of quantitative scoring models to compare a new oncology drug's survival benefit with its toxicity. Challenges arise from limited long-term data, variability in patient preferences, and differing risk tolerances across jurisdictions.

Cost-Effectiveness Analysis (CEA) – related terms: incremental cost, outcome measure, threshold. CEA measures the relative costs and health outcomes of two or more interventions, expressed as cost per unit of health gain (e.g., cost per QALY). Health technology assessment bodies employ CEA to inform reimbursement decisions. For instance, a CEA might compare a biosimilar insulin to the reference product, revealing lower costs for equivalent glycaemic control. Major challenges include selecting appropriate comparators, accounting for real-world adherence, and dealing with uncertainty in model inputs.

Discount Rate – related terms: present value, time preference, inflation. The discount rate is the percentage used to convert future costs and health benefits into present-day values, reflecting the societal preference for immediate benefits over delayed ones. Economic evaluations typically apply a 3-5% annual discount rate to both costs and outcomes. In practice, adjusting the discount rate can substantially alter the perceived value of preventive interventions that generate benefits far in the future. Determining an appropriate rate is contentious, especially when comparing high-discounted interventions across countries with differing fiscal policies.

Evidence-Based Medicine (EBM) – related terms: clinical guidelines, systematic review, hierarchy of evidence. EBM integrates the best available research evidence with clinical expertise and patient values to guide decision-making. In health policy, EBM underpins clinical pathway development and formulary inclusion criteria. For example, a national guideline may recommend statins for secondary prevention based on multiple randomized controlled trials. Challenges include translating evidence into practice, managing conflicting data, and updating policies as new studies emerge.

Formulary – related terms: tiered coverage, preferred drug list, therapeutic class. A formulary is a curated list of medications approved for prescribing within a health system or insurance plan. It often categorises drugs into tiers that determine patient co-pay levels. Market access teams aim to secure placement on preferred

tiers to improve uptake. An example is a payer moving a novel biologic to a “preferred” tier after negotiating a risk-sharing agreement. Challenges involve balancing cost containment with clinical autonomy, managing formulary revisions, and addressing manufacturer push-back.

Global Burden of Disease (GBD) – related terms: DALY, morbidity, mortality. The GBD study quantifies the impact of diseases and injuries worldwide using disability-adjusted life years (DALYs). Policymakers use GBD data to prioritise resource allocation and identify high-impact therapeutic areas. For instance, a health authority may allocate funding to hepatitis C eradication programmes after GBD analysis shows high DALYs in a specific region. Limitations include data quality gaps, differing definitions across countries, and the difficulty of translating macro-level findings into micro-level reimbursement decisions.

Health Technology Assessment (HTA) – related terms: value assessment, appraisal committee, reimbursement. HTA is a multidisciplinary process that evaluates the medical, economic, social, and ethical implications of health technologies. Agencies such as NICE (UK) and CADTH (Canada) conduct HTA to inform coverage policies. A practical application is the assessment of a new gene-therapy, where HTA examines cost, clinical benefit, and budget impact. Challenges include managing proprietary data, aligning HTA outcomes with stakeholder expectations, and coping with rapid innovation cycles that can outpace assessment timelines.

Incremental Cost-Effectiveness Ratio (ICER) – related terms: comparative analysis, willingness-to-pay, threshold. ICER is the ratio of the difference in costs to the difference in effectiveness between two interventions. It is expressed as cost per additional QALY (or other outcome). For example, an ICER of $25,000/QALY for a new antihypertensive may be deemed acceptable in a jurisdiction with a $30,000 threshold. Interpretation challenges include handling uncertainty (probabilistic sensitivity analysis), selecting appropriate comparators, and addressing ethical concerns when thresholds differ across disease areas.

Joint Commission – related terms: accreditation, quality standards, patient safety. The Joint Commission is a US-based accrediting body that sets performance standards for hospitals and health systems. Accreditation often influences payer contracts and reimbursement rates. A hospital achieving Joint Commission certification for stroke care may receive higher reimbursements under bundled payment models. Challenges include the resource intensity of compliance, keeping pace with evolving standards, and aligning accreditation requirements with local regulatory frameworks.

Key Opinion Leader (KOL) – related terms: clinical champion, thought leader, advisory board. KOLs are respected experts who shape clinical practice, guideline development, and prescribing behaviour. Pharmaceutical companies engage KOLs for scientific dissemination, advisory boards, and speaker programs. For instance, a KOL may present real-world evidence on a new immunotherapy at a national conference, influencing peer adoption. Ethical challenges involve maintaining transparency, avoiding conflicts of interest, and ensuring that KOL insights are evidence-based rather than promotional.

Lifecycle Management – related terms: post-marketing surveillance, label expansion, patent strategy. Lifecycle management encompasses activities that extend a product’s market presence, such as new indications, formulations, or combination therapies. A biotech firm may pursue a pediatric indication for an existing adult oncology drug, thereby increasing market size. Practical applications include negotiating

supplemental indications with regulators and updating health-technology assessments accordingly. Challenges include generating robust evidence for each new claim, managing additional regulatory submissions, and aligning pricing with expanded value.

Market Access – related terms: pricing strategy, reimbursement, payer negotiation. Market access is the set of strategies employed to ensure that a health product reaches patients and is reimbursed under favourable terms. It integrates health economics, HTA, and stakeholder engagement. For example, a manufacturer may propose an outcomes-based contract where payment is linked to patient response rates. Challenges include navigating heterogeneous payer landscapes, demonstrating value in diverse populations, and managing the administrative burden of risk-sharing agreements.

Net Present Value (NPV) – related terms: cash flow, investment appraisal, discounting. NPV calculates the present-day value of a series of future cash flows, subtracting initial investment costs. In pharmaceutical portfolio decisions, NPV helps determine whether to advance a candidate through clinical development. A positive NPV suggests that projected revenues, discounted at the cost of capital, exceed development costs. Challenges involve forecasting uncertain future sales, incorporating regulatory delays, and accounting for competitive dynamics that may erode market share.

Orphan Drug – related terms: rare disease, exclusivity, incentive. Orphan drugs target conditions affecting a small patient population, typically fewer than 200,000 individuals in the US. Regulatory incentives include market exclusivity, tax credits, and fee waivers. A practical example is a gene-therapy for spinal muscular atrophy that receives orphan designation, facilitating accelerated approval and premium pricing. Challenges encompass high development costs, limited evidence bases, and payer scrutiny over cost-effectiveness given the small patient pool.

Pharmacoeconomics – related terms: cost-utility analysis, budget impact, health economics. Pharmacoeconomics studies the economic implications of drug therapy, integrating cost data with clinical outcomes. It informs pricing, reimbursement, and formulary decisions. For instance, a cost-utility analysis may compare a novel anticoagulant to warfarin, revealing higher costs but greater QALYs. Limitations include data gaps on long-term adherence, difficulty capturing indirect costs, and the need for country-specific cost inputs.

Quality-Adjusted Life Year (QALY) – related terms: utility weight, health state, outcome measure. A QALY combines length of life with health-related quality of life, assigning a utility value (0-1) to each health state. It is the cornerstone metric in many CEA studies. Example: a treatment that adds 0.5 QALY per patient may be deemed cost-effective if the ICER falls below the jurisdiction's willingness-to-pay threshold. Challenges include measuring utilities accurately, addressing cultural differences in value judgments, and dealing with ethical concerns when allocating resources based on QALYs.

Real-World Evidence (RWE) – related terms: observational data, registries, electronic health records. RWE derives from data collected outside of controlled clinical trials, such as claims databases, patient registries, and EHRs. It provides insight into actual utilization patterns, safety, and effectiveness. For market access, RWE can support post-approval commitments or justify price premiums. A case study: an oncology drug's real-world survival rates may exceed trial results, strengthening reimbursement negotiations. Challenges

include data quality, heterogeneity, privacy regulations, and the need for robust analytical methods to mitigate bias.

Segmentation – related terms: target audience, payer stratification, disease sub-populations. Segmentation divides the market into distinct groups based on clinical, demographic, or economic characteristics. It enables tailored value propositions and pricing strategies. For instance, a manufacturer may segment patients with severe rheumatoid arthritis separately from those with moderate disease, offering differentiated reimbursement pathways. Practical challenges involve obtaining granular data, avoiding over-segmentation that complicates pricing, and ensuring equitable access across segments.

Therapeutic Index – related terms: safety margin, dose-response, toxicity. The therapeutic index is the ratio between a drug's toxic dose and its effective dose, indicating safety. A high therapeutic index suggests a wide safety margin. In policy, drugs with narrow therapeutic indices may require stricter monitoring and may influence formulary placement. Example: warfarin has a narrow therapeutic index, leading to specific prescribing restrictions. Challenges include educating prescribers, implementing monitoring programs, and balancing safety with access.

Utilization Review – related terms: prior authorization, claim audit, appropriateness criteria. Utilization review assesses the necessity, appropriateness, and efficiency of health services. Payers employ prior authorization to control costs and ensure evidence-based prescribing. For example, an insurer may require prior authorization for high-cost biologics, reviewing clinical indicators before approval. Challenges include administrative burden on clinicians, potential delays in care, and the need for transparent criteria that align with clinical guidelines.

Value-Based Pricing – related terms: outcome-based contract, risk-sharing, price-performance. Value-based pricing links the price of a therapy to the health outcomes it delivers. Contracts may stipulate refunds or discounts if predefined endpoints are not met. A practical illustration is a hepatitis C cure priced based on sustained virologic response rates. While attractive for payers, challenges include measuring outcomes reliably, negotiating data sharing agreements, and managing financial risk for manufacturers.

Willingness-to-Pay (WTP) – related terms: threshold, societal value, cost-effectiveness. WTP represents the maximum amount a society or payer is prepared to spend for a unit of health gain (e.g., per QALY). It serves as a benchmark for judging cost-effectiveness. For instance, the UK's NICE commonly uses a £20,000–£30,000 per QALY threshold. Determining WTP is complex; it varies across countries, disease severity, and public preferences. Challenges include political pressure, budget constraints, and ethical debates over placing monetary values on life.

e-Prescribing – related terms: digital health, interoperability, medication safety. Electronic prescribing enables clinicians to send prescription orders directly to pharmacies via secure digital platforms. It reduces errors, improves adherence tracking, and facilitates data collection for pharmacovigilance. In market access, e-prescribing data can inform real-world utilization analyses. Barriers include integration with legacy systems, varying standards across regions, and ensuring patient privacy.

Formulary Management – related terms: drug selection, therapeutic interchange, cost containment.

Formulary management is the ongoing process of reviewing, updating, and enforcing the list of covered medications. It involves multidisciplinary committees that assess clinical efficacy, safety, and economic impact. A health system may implement a step-therapy protocol requiring patients to try a generic before a brand-name drug. Challenges involve balancing clinical autonomy, managing stakeholder expectations, and adapting to emerging therapies that may disrupt existing pathways.

Health Equity – related terms: social determinants, disparity, access. Health equity aims to eliminate avoidable differences in health outcomes across population groups. Policies such as tiered co-pay reductions for low-income patients seek to improve equity. Market access strategies may incorporate equity adjustments, offering price discounts in underserved regions. Obstacles include measuring equity impacts, aligning incentives across payers, and addressing systemic barriers beyond the health sector.

Indication-Specific Pricing – related terms: multi-indication, price differentiation, value assessment. This pricing model assigns different prices to a drug based on the therapeutic area or indication. A cancer drug may be priced higher for a rare indication than for a common one, reflecting varying value. Implementation requires clear tracking of indication at the point of sale and agreements with payers. Challenges include complexity in invoicing, potential for "indication creep," and regulatory scrutiny over price discrimination.

Joint Negotiation – related terms: group purchasing, consortium, price bargaining. Joint negotiation involves multiple payers or health systems pooling purchasing power to negotiate better terms with manufacturers. In Europe, regional health authorities often engage in joint procurement for vaccines. Benefits include lower prices and streamlined contracts. However, coordination among diverse stakeholders, aligning clinical criteria, and maintaining confidentiality of negotiated terms pose significant challenges.

Key Performance Indicators (KPIs) – related terms: metrics, outcome measurement, dashboard. KPIs are quantifiable measures used to evaluate the success of health policies, programs, or market-access initiatives. Examples include formulary adherence rates, time-to-reimbursement, and budget impact variance. Monitoring KPIs enables continuous improvement and accountability. The main difficulty lies in selecting meaningful indicators, ensuring data integrity, and translating metrics into actionable policy changes.

Licensing Agreements – related terms: royalties, exclusivity, technology transfer. Licensing agreements grant rights to produce, market, or distribute a health technology in exchange for compensation such as royalties or upfront fees. They are common in pharmaceuticals when a biotech company partners with a larger firm for commercialization. Effective agreements can accelerate market entry and broaden geographic reach. Negotiating fair terms, protecting intellectual property, and aligning incentives across parties are frequent hurdles.

Managed Care – related terms: capitation, HMO, utilization management. Managed care organisations (MCOs) coordinate health services to control costs and improve quality. They employ tools such as capitation payments, disease-management programs, and provider networks. For market access, engaging with MCOs early can facilitate formulary inclusion and bundled payment arrangements. Challenges include navigating varying network structures, aligning clinical pathways with payer incentives, and addressing patient choice within managed environments.

Network Adequacy – related terms: provider access, geographic coverage, contractual arrangements. Network adequacy assesses whether a health plan's provider network offers sufficient access to needed services within reasonable travel distances and time frames. Regulators often set minimum standards. Insurers must balance network breadth with cost containment, sometimes employing tiered networks. Practical issues include contracting with specialists in underserved areas, monitoring patient access metrics, and adjusting networks in response to demographic shifts.

Outcome Measures – related terms: clinical endpoints, patient-reported outcomes, surrogate markers. Outcome measures quantify the results of health interventions, ranging from hard clinical events (e.g., mortality) to patient-reported quality-of-life scores. They are essential for HTA submissions, value-based contracts, and performance monitoring. For example, a diabetes drug may be evaluated based on HbA1c reduction and incidence of hypoglycaemia. Selecting appropriate measures, ensuring standardisation, and capturing long-term outcomes remain challenging tasks.

Pharmacovigilance – related terms: adverse event reporting, safety monitoring, post-marketing surveillance. Pharmacovigilance monitors the safety of medicines throughout their lifecycle, detecting, assessing, and preventing adverse effects. It relies on spontaneous reporting systems, registries, and electronic health data. Regulatory authorities may require risk-management plans and periodic safety updates. Real-world implementation challenges include under-reporting, data integration across sources, and timely signal detection to mitigate patient risk.

Quality Improvement (QI) – related terms: continuous improvement, Plan-Do-Study-Act, clinical audit. QI initiatives aim to enhance health care processes and outcomes through systematic, data-driven methods. Examples include reducing hospital readmission rates for heart failure via care-transition protocols. In policy, QI metrics can inform reimbursement incentives tied to performance. Barriers include staff engagement, aligning QI goals with organisational priorities, and sustaining improvements over time.

Risk-Sharing Agreements – related terms: outcome-based contracts, financial guarantees, performance clauses. These contracts align payment with the achievement of predefined clinical or economic outcomes. A manufacturer may refund a portion of the price if a drug fails to achieve a target response rate in the real world. They help manage payer uncertainty about high-cost therapies. Complexity arises in defining measurable outcomes, data collection logistics, and negotiating equitable risk allocation between parties.

Supply Chain Resilience – related terms: inventory management, diversification, contingency planning. Resilience refers to the ability of the pharmaceutical supply chain to withstand disruptions such as pandemics, natural disasters, or geopolitical events. Strategies include dual-sourcing, safety-stock inventories, and digital tracking systems. A resilient supply chain ensures continuous patient access, a core market-access objective. Challenges involve higher inventory costs, regulatory compliance across jurisdictions, and coordination among manufacturers, distributors, and health systems.

Technology Diffusion – related terms: adoption curve, diffusion of innovations, market penetration. Technology diffusion describes how new health technologies spread across providers and patients over time. The S-curve model illustrates early adopters, rapid growth, and eventual saturation. Understanding diffusion patterns assists planners in forecasting demand and budgeting. For example, tele-health services

saw accelerated diffusion during the COVID-19 pandemic. Barriers to diffusion include cost, training needs, and resistance to change among clinicians.

Utilization Management (UM) – related terms: prior authorization, step therapy, formulary restrictions. UM is a set of techniques used by payers to control the appropriateness and cost of health services. It includes review processes that determine whether a prescribed therapy meets clinical criteria before approval. An insurer may require a step-therapy protocol that mandates trying a generic before a brand-name biologic. While UM can contain costs, it may also delay access and increase administrative workload for providers. Balancing cost control with patient-centred care is a persistent challenge.

Value Frameworks – related terms: multicriteria decision analysis, scoring system, stakeholder weighting. Value frameworks provide structured approaches to assess the overall worth of a health technology, incorporating clinical, economic, and societal dimensions. Examples include the ASCO Value Framework for oncology therapies and the ESMO-Magnitude of Clinical Benefit Scale. They facilitate transparent decision-making and support payer-provider negotiations. Limitations involve subjectivity in weighting criteria, data availability, and adapting frameworks to different health system contexts.