HEOR Value Across the Product Development Timeline

An analytical, actionable report for pharma HEOR & market access professionals (region-agnostic; budget unspecified)

Executive summary

Health Economics and Outcomes Research (HEOR) is the discipline that turns clinical benefit into **decision-grade evidence of value** for regulators, HTA bodies, payers, and health systems—connecting outcomes that matter to patients and clinicians with resource use, costs, and uncertainty. Outcomes research is commonly described as measuring end results of healthcare services beyond clinical effects, explicitly including **economic** and **humanistic** outcomes (functioning, well-being, satisfaction). [1]

Across the lifecycle, HEOR creates value by **reducing decision uncertainty** at every inflection point: early development decisions (what to build and for whom), Phase II–III choices (comparators, endpoints, evidence generation), peri-launch submission success (HTA dossiers, models, indirect comparisons), and post-launch sustainability (managed access, real-world evidence generation, reassessments). NICE explicitly requires companies (in single technology evaluations) to provide a **systematic review** of clinical and cost evidence and an economic evaluation, making early HEOR integration a practical necessity—not a "nice to have." [2]

Best-in-class HEOR programs follow five workstreams end-to-end:
- **Economic modeling** (CEA/CUA) + uncertainty management, aligned with reference cases (e.g., NICE preference for EQ-5D/QALYs; requirements for transparent, executable models). [3]
- **Budget impact analysis (BIA)**, which ISPOR frames as an essential complement to cost-effectiveness and increasingly required for reimbursement decisions. [4]
- **RWD/RWE strategies**, using "fit-for-purpose" data and designs; NICE and HAS provide dedicated methodological frameworks to improve credibility and utility of real-world studies for guidance decisions. [5]
- **Clinical Outcomes Assessment (COA)** including PRO/psychometrics; FDA emphasizes "fit-for-purpose" instruments and that there is no universal validation—evaluation depends on the intended context and claim. [6]
- **Evidence synthesis** (systematic review + meta-analysis/NMA), reported to PRISMA standards and aligned with NICE DSU technical methods for indirect and network comparisons. [7]

Finally, two policy signals raise the strategic stakes: EU HTA Regulation implementation applies from **12 Jan 2025**, and joint clinical assessments begin with oncology medicines and ATMPs—reinforcing the need for early cross-market evidence planning. [8]

Definitions and scope of HEOR

What HEOR covers in pharma development

HEOR typically spans (1) **economic evaluation** (cost-effectiveness/cost-utility), (2) **health-related quality of life and patient experience** (COA/PRO; utilities), and (3) **real-world evidence** (RWE) about outcomes, utilization, and comparative effectiveness in routine practice.

A practical anchor definition comes from outcomes research: studies that identify, measure, and evaluate end results of healthcare services and include clinical, economic, and humanistic outcomes. [1]

How HEOR aligns with HTA bodies and health system decision-making

HTA is a "systematic and multidisciplinary evaluation" of health technologies covering direct and indirect consequences. [9]
Because HTA explicitly integrates clinical and economic dimensions, HEOR is the main "evidence engine" that supports HTA dossiers and payer decisions, including the move toward joint clinical assessments at EU level. [8]

RWD vs RWE (why "fit-for-purpose" matters)

NICE defines real-world evidence as evidence generated from analysis of real-world data, and its RWE framework aims to improve evidence quality used in guidance (without setting minimum acceptability standards). [10]
FDA guidance similarly frames the regulatory RWE program and provides considerations for using RWD/RWE to support regulatory decision making for drugs and biologics. [11]

HEOR across the product development timeline

Comparative mapping table: HEOR activities, stages, and deliverables

The table below is intended as a **website-ready reference** for program planning and resourcing. It maps the core HEOR activity families to typical stage-appropriate deliverables (the "minimum viable" outputs for decision making and submissions).

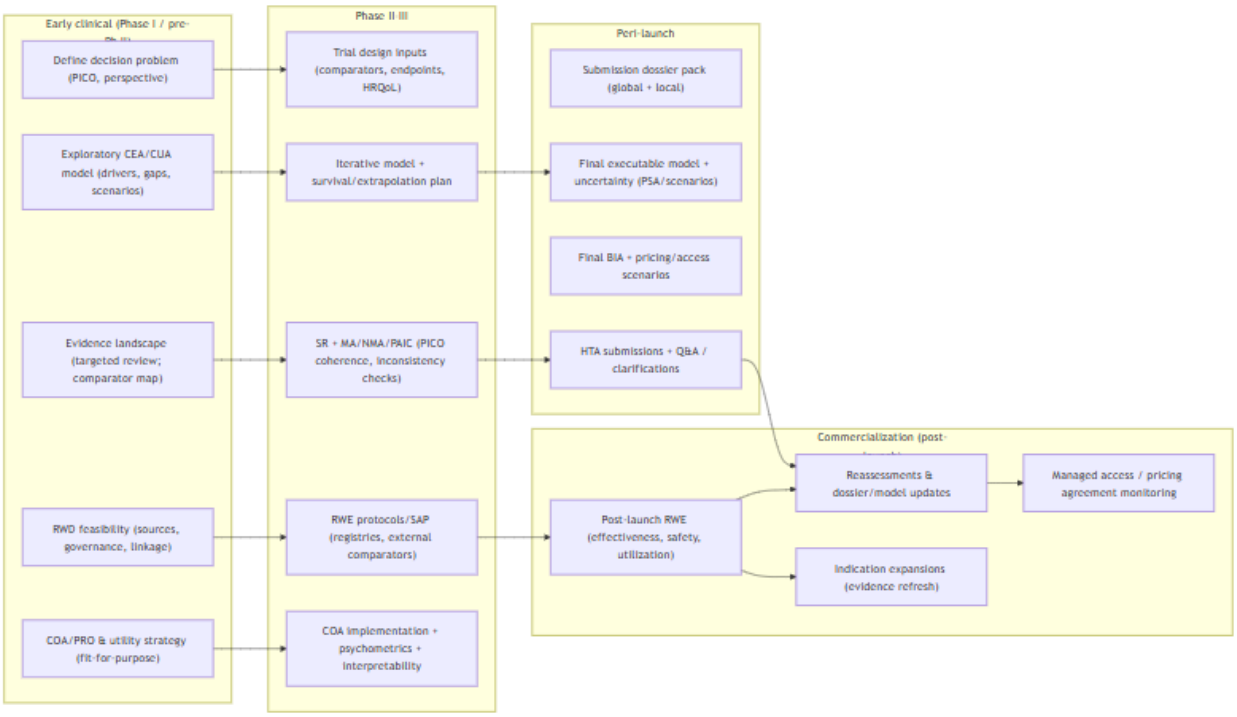
HEOR activity family	Early clinical (pre-Ph II / Phase I)	Phase II–III	Peri-launch (pre-submission → launch)	Commercialization (post-launch)
Value strategy & decision problem (PICO, perspective,	Value hypothesis, PICO draft, target value profile; early	Evidence plan updates (gaps → collection); endpoint & comparator	Integrated value dossier plan (global + local adaptations)	Lifecycle evidence refresh plan (reassessments, extensions, managed access obligations)

HEOR activity family	Early clinical (pre-Ph II / Phase I)	Phase II–III	Peri-launch (pre-submission → launch)	Commercialization (post-launch)
endpoints)	HTA "risk register"	"HTA alignment"		
Economic modeling (CEA/CUA)	Exploratory model: value drivers, data gaps, scenario testing	Iterative model with trial data; survival/extrapolation plan (esp. oncology)	Final executable model + full transparency; sensitivity & PSA; submission-ready documentation	Model recalibration with RWE; updates for new comparators/indications; resubmissions
Budget impact analysis (BIA)	Early eligible population & uptake scenarios; budget-holder perspective outline	Epidemiology refinement; treatment mix and uptake testing	Final BIA aligned to submission/pricing; scenario grid (uptake/price/lines)	Budget tracking vs forecast; renegotiation support; access agreement monitoring
RWD/RWE generation & use	Data feasibility (availability, governance, linkage); protocol concepts	Protocols/SAP; registries; external comparator feasibility; bias/confounding plan	Peri-launch RWE studies (utilization/outcomes); managed access data collection plan	Post-launch effectiveness/safety/utilization; post-listing studies; reassessment packages
COA/PRO & psychometrics (incl. utilities)	COA strategy; instrument selection/development plan; qualitative work for content validity; utility strategy	COA operationalization (ePRO), missing data mitigation; psychometric evidence build	Interpretation package (responder definitions, MIDs); HTA-ready COA evidence	RWE patient experience; mapping/utility updates; instrument refinement
Evidence synthesis (SR/MA/NMA, population-	Landscape review; comparator scan; preliminary	Full SR; NMA/ITC for missing head-to-head; coherence checks	Final SR/NMA integrated into dossier and model; methods	Living reviews & network updates; new evidence integration for new appraisals

HEOR activity family	Early clinical (pre-Ph II / Phase I)	Phase II–III	Peri-launch (pre-submission → launch)	Commercialization (post-launch)
adjusted ITC)	network feasibility		transparency package	

This mapping is consistent with NICE requirements for systematic clinical/cost evidence review and economic evaluation within company submissions, plus the common HTA focus on QALY-based cost-utility analysis and modeling transparency. [12]

Lifecycle timeline flowchart (Mermaid)



Best-practice playbooks by HEOR workstream

Economic modeling (CEA/CUA) and uncertainty management

Objectives
Produce a robust estimate of value (incremental costs vs outcomes) to inform internal decisions (TPP, positioning, price corridor) and HTA submissions that often require cost-utility approaches (QALYs) and explicit handling of uncertainty. NICE states EQ-5D is the preferred HRQoL measure in adults and provides reference-case expectations (e.g., discounting, costing perspective). [13]
HAS economic evaluation guidance similarly focuses on methodological choices to estimate efficiency and emphasizes justification and interpretation of methodological choices. [14]

Methods and best practices
Modeling good practice is strongly shaped by ISPOR-SMDM "Modeling Good Research Practices," which frames essential elements: conceptualization, parameter estimation/uncertainty, and validation/transparency. [15]
Key practices that materially reduce HTA risk include:
- **Decision problem discipline**: define population, comparators, perspective, and time horizon consistent with HTA expectations (and document deviations). [16]
- **Survival extrapolation governance** (common in oncology): NICE DSU TSD14 provides technical guidance for survival analysis and extrapolation when patient-level trial data are available. [17]
- **Validation and transparency by design**: treat the model as a regulated asset—traceable parameters, reproducible runs, and clear alignment between write-up and executable file. ISPOR-SMDM explicitly addresses transparency and validation best practices. [18]
- **Reporting standards**: use CHEERS 2022 to improve interpretability and decision usefulness of health economic evaluations, including stakeholder involvement. [19]

Typical deliverables
A staged "model stack" works best: conceptual model + assumptions log → executable model (Excel/R/Python) → technical report → submission-ready outputs (base case + scenarios + PSA) + audit pack. NICE explicitly requires an **executable electronic copy** with full access to programming code for submissions and coherence between submission content and model content. [20]

Stakeholders
HEOR modelers, market access/pricing, clinical development, biostatistics, RWE/data science, medical affairs, affiliates; external: KOLs, sometimes patients/public (CHEERS 2022 highlights increased role of stakeholder involvement). [21]

Timing
Start exploratory modeling in early clinical to identify value drivers and data gaps; iterate through Phase II–III; finalize peri-launch; maintain post-launch updates (comparators, reassessments, managed access). This aligns with NICE processes that can include managed access and subsequent updates. [22]

Data sources
Trial endpoints (including HRQoL), published utilities, costs (health system tariffs; micro-costing), epidemiology, RWD/claims/EHR for utilization and resource use, expert elicitation (for uncertain long-term parameters). [23]

Common pitfalls
Misaligned comparator or population vs scope; weak or unjustified survival extrapolation; opaque assumptions; mismatch between written submission and model; insufficient uncertainty exploration. These pitfalls are directly targeted by NICE submission requirements and DSU methods guidance. [24]

KPIs / metrics (operational and HTA-facing)
- “Time to stable base case” (cycle time per iteration).
- % parameters with traceable provenance (source + rationale + PSA distribution). (CHEERS-aligned reporting discipline.) [25]
- Number of major HTA critique points attributable to modeling (vs clinical evidence).
- PSA stability: probability cost-effective at threshold(s) under key scenarios (tracked across iterations).

Budget impact analysis (BIA)

Objectives
Quantify financial consequences of adoption for a defined budget holder over a short-to-mid horizon; complement cost-effectiveness with affordability and uptake realities. ISPOR explicitly states BIAs are essential in comprehensive economic assessment and are increasingly required by reimbursement authorities. [4]

Methods and best practices
ISPOR BIA good practice emphasizes system context and market dynamics (eligible population flows, uptake, displacement, treatment mix), with stakeholder relevance and transparency. [26]

Typical deliverables
Budget holder perspective definition; epidemiology and eligibility model; uptake/displacement scenarios; cost offsets (where relevant); final BIA model and summary deck for payer discussions.

Stakeholders
HEOR, pricing/contracting, finance, country market access, epidemiology/RWE.

Timing
Early scenarios for internal price corridor and launch sequencing; final peri-launch; post-launch tracking supports renegotiations and managed access monitoring. [27]

Data sources
Epidemiology, claims/EHR utilization, treatment patterns, internal forecast assumptions; trial resource use for certain items.

Common pitfalls
Over-optimistic uptake; non-transparent population estimation; inconsistent dosing assumptions; double counting offsets; mismatch with submitted/in-market positioning. (These are visible in real HTA critiques—see Spinraza case study below.) [28]

KPIs
Budget forecast error post-launch; % scenarios requested by payers addressed; time-to-update when new utilization data arrives.

RWD/RWE generation and use

Objectives
Address evidence gaps that trials cannot fully cover (generalizability, comparative effectiveness in practice, long-term outcomes, utilization), and support regulatory and HTA decisions. Nice defines RWE as evidence generated from analysis of RWD and provides a framework to improve quality for guidance. [10]
HAS provides a methodological guide to support implementation of real-world studies for evaluation by HAS committees and to optimize evidence level and confidence. [29]
Regulatory use is also formalized: FDA created an RWE program (21st Century Cures Act context) and published guidance on considerations for RWD/RWE in regulatory decision-making. [11]

Methods and best practices
- **Fit-for-purpose framing** (question → estimand → data suitability → design → bias control → transparency). NICE's RWE framework is designed to improve decision usefulness rather than impose strict minimum standards. [30]
- **Protocol-first discipline**: prespecify design and analysis plans (especially for comparative studies) to increase credibility with HTA and regulators. [31]
- **Networked EU capability**: EMA's DARWIN EU is used to support regulatory procedures; the EMA's 2024–2025 report highlights a **median 4-month** duration from protocol approval to final results, enabling timely integration into regulatory timelines. [32]

Typical deliverables
Feasibility assessment (availability, completeness, linkage), protocol + SAP, data dictionary/definitions, confounding plan, results report, and an HTA-facing "credibility pack" (data provenance, validation, sensitivity analyses). In France, post-listing ("post-inscription") studies are systematically evaluated by HAS and published. [33]

Stakeholders
RWE epidemiology, data science, HEOR, medical affairs, pharmacovigilance, legal/privacy, data owners, payer liaisons.

Timing
Feasibility in early clinical; protocols in Phase II; peri-launch studies for uptake/resource/care pathway; post-launch studies for reassessment, managed access or post-listing commitments. NICE describes evidence collection during managed access and subsequent guidance updates. [34]

Data sources
EHR, claims, registries, product support programs, patient registries, national data platforms; EU: DARWIN EU partner network. [35]

Common pitfalls
Confounding by indication, non-comparable populations, poor endpoint observability, changing clinical practice ("channeling"), weak data provenance or missing data

management. NICE and HAS frameworks exist largely to surface and mitigate these issues. [36]

KPIs
Feasibility pass rate (variables captured; completeness); protocol-to-results time (benchmarkable against DARWIN EU median durations where relevant); proportion of HTA uncertainties reduced; number of HTA challenges to RWE credibility. [37]

COA/PRO (including psychometrics and utilities)

Objectives
Select, develop, and operationalize outcome measures (incl. patient-reported outcomes) that can support labeling/claims and HTA value arguments by capturing outcomes meaningful to patients. FDA's PRO guidance explains how FDA reviews PRO instruments used to support labeling claims. [38]
FDA's COA-related guidance materials emphasize evaluation in the context of intended use (trial design, population, desired claim) and that "there is no such thing as instrument validation for all purposes." [39]

Methods and best practices
- **Content validity first**: PRO instrument adequacy must be demonstrated for the concept, population, and context of use (qualitative work, cognitive interviews, item relevance). [38]
- **Psychometrics & measurement properties**: use COSMIN methodology for reviewing measurement properties of PROMs, including Risk of Bias and criteria for good measurement properties. [40]
- **Operational quality controls**: ePRO implementation plans, missing data mitigation, and prespecified scoring and interpretation (responder definitions / MIDs). FDA provides patient-focused outcome measurement roadmaps and guidance on selecting/developing "fit-for-purpose" COAs. [41]
- **Utility strategy**: if your target markets prioritize QALYs, ensure utility capture is planned. NICE prefers EQ-5D for adults and requests justification (including qualitative evidence on content validity) if claiming it is inappropriate. [13]

Typical deliverables
COA strategy (concept model → instrument selection), qualitative evidence package, psychometric validation evidence, COA charter and ePRO operational plan, interpretation dossier (MIDs/responder thresholds), mapping plan (if needed for utilities), submission-ready COA reports.

Stakeholders
Clinical development, HEOR, psychometricians/qualitative researchers, biostats, data management/eCOA vendors, regulatory, patient advocacy.

Timing
Start in early clinical to avoid late, expensive redesigns; implement in Phase II–III; package

peri-launch; extend post-launch to real-world patient experience if required for reassessments. [42]

Data sources
Trial COA data, qualitative patient interviews, published measurement property studies, preference-based valuation studies (for utilities).

Common pitfalls
Selecting instruments without adequate content validity; high missingness; post hoc endpoint changes; unclear interpretation; collecting HRQoL inconsistent with HTA expectations (leading to weak QALY arguments). FDA and NICE guidance explicitly highlight these concerns through their review frameworks. [43]

KPIs
COA completion rate; missingness per visit; number of protocol deviations affecting COA; evidence sufficiency for claims; HTA acceptance of HRQoL/utility inputs.

Evidence synthesis (systematic reviews, meta-analysis, network meta-analysis)

Objectives
Create a transparent evidence base for comparative efficacy/effectiveness, support economic models, and manage missing head-to-head evidence via indirect comparisons and NMA.

Methods and best practices
- **Systematic reviews**: PRISMA 2020 provides the updated checklist and flow diagrams for reporting systematic reviews. [44]
- **Cochrane methods**: the Cochrane Handbook is the core reference for systematic review methodology. [45]
- **NMA/ITC technical approach**: NICE DSU TSD2 provides a generalized linear modeling framework applicable to pair-wise meta-analysis, indirect comparisons, multi-arm trials, and network meta-analysis. [46]
- **Critical appraisal**: NICE DSU TSD7 is a reviewer's checklist intended for pair-wise meta-analysis, indirect comparisons, and NMA (not prescriptive, but structured). [47]
- **Reporting NMA results**: NICE's Appendix K requires NMA reporting meet a modified PRISMA-NMA checklist. [48]
- **PRISMA-NMA**: PRISMA provides an NMA extension for reporting systematic reviews comparing multiple treatments using direct and indirect evidence. [49]

Typical deliverables
Protocol, search strategy, extraction tables, PRISMA flow diagram, risk of bias assessments, meta-analysis outputs, network plots, inconsistency assessments, and a "model integration pack" (effect estimates + uncertainty, consistency assumptions).

Stakeholders
Evidence synthesis leads, HEOR, statisticians (Bayesian/modelling), clinical experts, medical writing, market access.

Timing
Landscape in early clinical; full SR/NMA in Phase II–III to inform trial design and submission; updates post-launch ("living" approach) to support reassessment and new comparators.

Data sources
RCTs, observational studies (when appropriate), registries; data extraction from publications and clinical study reports where available.

Common pitfalls
PICO misalignment with HTA scope, disconnected networks, heterogeneity/inconsistency not addressed, non-transparent methods, and lack of clear linkage into models (effects/uncertainty). NICE DSU checklists are specifically designed to make these risks visible. [50]

KPIs
PRISMA adherence; time-to-final SR/NMA; network connectivity metrics; number of HTA queries related to indirect comparisons; reproducibility checks passed.

HTA / market access submissions (dossiers, models, managed access)

Objectives
Convert the HEOR evidence base into submission packages that satisfy country HTA requirements, minimize technical criticism, and enable timely reimbursement and sustainable access.

Methods and best practices
- **Submission transparency** (NICE): companies must submit an executable economic model with full access to code, and ensure submission content aligns with model content. [20]
- **Evidence standards** (NICE): within health technology evaluations, the company must provide a systematic review of clinical and cost evidence and an economic evaluation; NICE's manual describes methods, processes, and managed access update mechanics. [51]
- **France process signals**: HAS provides submission ("deposit a dossier") routes and highlights that it evaluates efficiency for innovative products with significant expenditure impact. [52]
- **Post-listing evidence** (HAS): results of post-listing ("post-inscription") studies are systematically evaluated and published by HAS. [53]
- **Managed access (NICE)**: evidence is collected until the end date in the managed access agreement; guidance is then updated and the technology may or may not be recommended for routine use depending on cost-effectiveness. [27]

Typical deliverables
Global value dossier + country adaptations; submission dossier (clinical + economic); SR/NMA appendices; executable model + technical report; BIA; RWE strategy and commitments; response packages to HTA questions.

Stakeholders
Global market access, country affiliates, HEOR, medical affairs, regulatory, legal/HEOR governance, external vendors (EAG support, SR/NMA teams).

Timing
Start dossier planning early Phase III; pre-submission internal "red team" to simulate HTA critique; peri-launch submission execution; post-launch updates for managed access and reassessments. [54]

Common pitfalls
Late alignment to HTA scope; model transparency issues; inconsistent narrative vs model; underprepared Q&A responses; lack of credible post-launch plan for remaining uncertainty. NICE requirements make these failure modes explicit. [55]

KPIs
First-cycle submission acceptance rate; number/severity of technical questions; time-to-guidance; managed access conversion rate (managed → routine use); number of post-launch commitments delivered on time. [27]

Case studies showing HEOR impact

Case study summary table (requested)

Case	HEOR lever	Decision impacted	What HEOR changed (practical impact)	Primary source
Spinraza (nusinersen) – France	Economic model + BIA quality	HTA efficiency opinion / affordability debate	HTA critique emphasized lack of QoL integration in interpretation and questioned BIA transparency and sustainability assumptions	HAS efficiency opinion PDF [28]
Kymriah (tisagenlecleucel) – France	Evidence synthesis +	Relative positioning vs comparator	HAS stated role vs Yescarta could not be	HAS (English) product page [56]

Case	HEOR lever	Decision impacted	What HEOR changed (practical impact)	Primary source
	RWD maturity		robustly established due to limitations of indirect comparisons and immature RWD	
Tisagenlecleucel – NICE managed access update (UK)	Managed access + RWE	Update of HTA guidance	NICE guidance review explicitly considers new evidence collected through managed access (trial + NHS treatment data)	NICE TA975 recommendation PDF [57]
Prograf (tacrolimus) – US	RWE for effectiveness	Regulatory label expansion	FDA approved a new use based on real-world evidence for lung transplant rejection prevention	FDA announcement [58]
Jakafi (ruxolitinib) – US	COA/PRO endpoint (symptom scoring)	Label-supported symptom improvement evidence	FDA label documents symptom endpoint using a structured diary instrument and thresholds for symptom score reduction	FDA label PDF [59]
Value of Information (VOI) – NICE/York	Decision modeling to guide research	Research prioritization / trial design strategy	VOI analysis demonstrates and operationalizes how decision-analytic methods identify	University of York report + NICE DSU page [60]

Case	HEOR lever	Decision impacted	What HEOR changed (practical impact)	Primary source
			where additional evidence is most valuable to reduce uncertainty	

What each case teaches (concise takeaways)

Spinraza (HAS, efficiency opinion): "Model credibility is itself a market access asset."
In the HAS efficiency opinion on Spinraza, the document signals major reservations and highlights concerns about budget impact estimation and sustainability at the claimed price level, including issues linked to transparency and assumptions in the BIA. [28]
HEOR lesson: budget impact robustness (population logic, dosing, uptake, transparency) and interpretable outcomes (including QoL when material) are not "nice extras"—they can become central critiques affecting access negotiations.

Kymriah (HAS): "Indirect comparison weakness + immature RWD = unresolved positioning."
HAS explicitly states that the role of Kymriah compared to Yescarta in shared DLBCL indication cannot be robustly established due to limitations of indirect comparisons and immature real-world data. [56]
HEOR lesson: NMA/ITC and RWE strategies are substitutes only when executed to a high standard; otherwise uncertainty persists and constrains pricing/positioning.

NICE TA975 (managed access update): "RWE is part of the HTA contract."
NICE's TA975 PDF notes that the evaluation reviews new evidence collected under the managed access agreement, including a clinical trial and evidence from NHS use in England. [57]
HEOR lesson: plan managed access evidence early—data definitions, collection burden, and analytic credibility—because it directly feeds the reassessment.

FDA Prograf (RWE-based label expansion): "Regulatory-grade RWE is feasible when data and design fit the question."
FDA announced approval of a new indication for Prograf to prevent organ rejection in adult and pediatric lung transplant recipients, explicitly framed as a case of RWE-based regulatory action. [58]
HEOR lesson: invest early in RWD feasibility and protocol discipline—FDA's RWE program and guidance set expectations that can be anticipated. [11]

FDA Jakafi label (symptom endpoint): "COA can be label-visible—and value-critical."
The Jakafi label describes symptom improvement endpoints using a structured symptom assessment approach and specifies responder-style thresholds (e.g., reductions in total

symptom score). [59]
HEOR lesson: COA strategy is not only “patient centric”; it is a measurable differentiator and can become a formal endpoint supporting claims—requiring fit-for-purpose instrument evidence and execution discipline. [61]

VOI (York / NICE DSU): “Use HEOR to design the next study, not just judge the last one.”
The York report aims to demonstrate the benefits of decision-analytic methods and VOI to inform NICE research recommendations and assess feasibility of timely application. [62] NICE DSU hosts the VOI pilot objectives and report as part of methodological development. [63]
HEOR lesson: apply VOI/EVPI/EVSI internally to target the trial features that most reduce reimbursement uncertainty (endpoints, follow-up duration, subgroups, RWE complement).

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