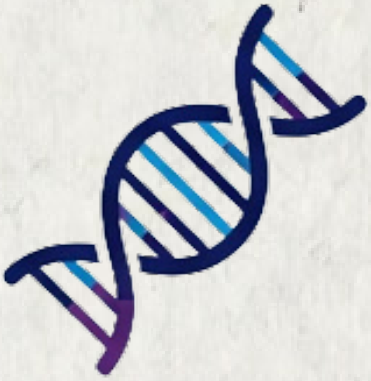

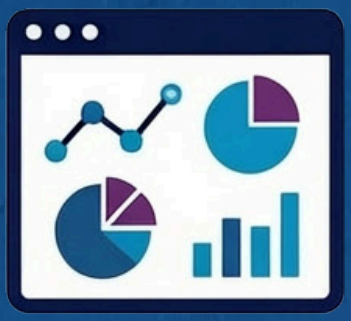

AMCP GLOSSARY OF RWE TERMS

Updated April 2026

AMCP Research Institute™

AMCP Learn

Glossary of Terms

A

Absolute Risk Reduction

The difference in event rates between the two groups.

Accessibility

A patient's ability to obtain and utilize healthcare services and products is determined by their availability and acceptability to the patient, geographic location of healthcare facilities, transportation options, hours of operation, patient preferences, and cost of care.

Adherence

The extent to which a patient takes medications or follows a treatment regimen as prescribed, including the correct dose, route, timing, and duration. Optimal adherence rates may vary by disease state and desired clinical outcome. Common methods for measuring adherence include Medication Possession Ratio (MPR) and Proportion of Days Covered (PDC).

Adherence Bias

Patients who closely follow treatment plans often also practice other health behaviors (e.g., diet, exercise), which may make the treatment seem more effective than it truly is.

Administrative Claims Data

Data obtained from clinical sites of care, such as medical offices and hospitals. These claims are submitted by the site to payers for reimbursement of services rendered or infusions or injectables administered during the on-site visit by the care team.

AMCP RWE Checklist

A structured tool used to apply the AMCP RWE Criteria to individual studies.

AMCP RWE Criteria

A set of evaluative standards used to assess the relevance, rigor, and usefulness of individual RWE studies.

AMCP RWE Framework

A structural roadmap that organizes real-world evidence expectations across the product lifecycle and by dossier type.

AMCP RWE Standards

A set of standardized recommendations developed by AMCP to guide the generation, evaluation, and communication of real-world evidence in support of U.S. payer decision-making.

Approved Product Dossier

A structured, comprehensive document that standardizes clinical and economic evidence, including comparative effectiveness for drugs, biologics, and vaccines.

B

Bias

A factor that affects the accuracy or credibility of study findings.

Blinding

An experimental study in which participants do not know the treatment they are receiving; investigators may also be blind to the specific treatments.

Budget Impact

The financial impact of a new product entering the market.

C

Case-Control Study

A retrospective observational study that compares a group with a particular condition (cases) and a group without it (controls) to determine whether the condition is associated with a specified factor.

Case Report

A report of a medical condition, its treatment, and outcome in a single patient.

Case Series

A collection of information about a medical condition, its treatment, and outcomes from several individuals.

Causation

An association between two characteristics that can be demonstrated to be due to cause and effect (i.e., a change in one causes a change in the other).

Claims Data

Data that comes from requests for reimbursement for care, or claims, that have been adjudicated (resolved/decided) by the insurance company. They can include claims related to prescription, hospital, and medical activities.

Closed Claims Data

Provides a comprehensive view of costs and utilization through both pharmacy and administrative claims. It tracks care across multiple settings (e.g., inpatient, outpatient, pharmacy, and ancillary services.)

Cohort Study

A study used to evaluate the association between the exposure or risk factor and the subsequent development of disease or outcome.

Comparative Effectiveness

Highlights differences in safety and effectiveness outcomes between new and existing treatments in a broader population, giving payers additional data to help guide drug coverage or preferred product decisions.

Confounding Bias

Bias due to an external, uncontrolled factor that misrepresents the association between the exposure or treatment and the outcome.

Correlation

A relationship between two variables (characteristics), such that as one changes, the other changes in a predictable way.

Cost Effectiveness

Cost-effectiveness models use similar RWE inputs as budget impact models and incorporate comparative-effectiveness data to determine if the cost and clinical outcomes of a new therapy provide added value to the existing therapeutic ecosystem.

Cross-Sectional Study

Simultaneous assessment of an exposure and outcome at a specific point in time. Typically conducted in a sample of people defined from the overall population.

D

Data Quality

Refers to assessing the accuracy, completeness, and transparency of data input to identify any potential bias that may impact internal validity.

Data Relevancy

Refers to whether the dataset can appropriately answer the research question in the clinical context while representing the population of interest.

Dossier

Documents that serve as a continuous, evolving source of objective, credible, and relevant information throughout the development and commercial life cycles of products.

Durability of Effect

May enable a payer to extend their approval timeframe if a drug shows long-term benefit. This can reduce administrative burden for patients and prescribers if reauthorizations can be submitted less frequently.

E

Electronic Medical Records (EMR)

Data collected for the documentation, assessment, and provision of clinical care and treatment pathways within healthcare systems, inpatient, or outpatient settings.

External Comparators

A cohort of patients, often generated from real-world data such as electronic health records or registries, serves as a comparison group for a clinical study. May also be referred to as synthetic control data.

External Validity

The extent to which results provide a correct basis for generalizations to other circumstances.

Efficacy

Measures how well a treatment works in ideal, controlled conditions (i.e., clinical trial data.)

Effectiveness

Measures how well a treatment works in real-world populations.

F

FDA Guidance Documents

Describe the FDA's interpretation of or policy on a regulatory issue. In general, FDA's guidance documents do not establish legally enforceable responsibilities and thus are not binding on the FDA or the public.

Fit-for-Purpose Data

Selecting data that is appropriate to specific research objectives. It involves considering quality, relevance, and compliance that will aid in answering the question reliably.

G

Generalizability

The extent to which the effects observed in published studies are likely to achieve similar results when the same intervention is applied to the population of interest under "real-world" conditions.

GLOSSARY

H

Healthcare Economic Information

Any analysis (including clinical data, inputs, clinical or other assumptions, methods, results, and other components underlying or comprising the analysis) that identifies, measures, or describes the economic consequences of the use of a drug.

Healthcare Resource Utilization (HCRU)

The use of health care services for the treatment or prevention of a disease or other health issues.

Health Technology Assessment

The assessment of the relative effects of a health technology to inform decision-making on its use or reimbursement.

Heterogeneous

Differences between studies in key characteristics of the participants, interventions, or outcome measures.

Homogeneous

Similarity of participants, interventions, and measurement of outcomes across a set of studies. In meta-analysis, used specifically to describe the effect estimates from a set of studies where they do not vary more than would be expected by chance.

Hospital-Based Registries

Track patients diagnosed or treated at a specific facility and help monitor the quality of care.

I

Indication Expansion Dossier

A specialized, evidence-based document that manufacturers submit to health care decision makers to support the use of a previously approved drug for a new, often unapproved, or recently approved condition.

Information Bias

See measurement bias.

Internal Control

Patient cohort recruited for a trial to undergo the same protocol and are typically randomized to receive a placebo or treatment.

Internal Validity

The extent that the design and conduct of a study are likely to have prevented bias. More rigorously designed (better quality) trials are more likely to yield results that are closer to the truth.

Intervention Study Design

Participants are assigned to receive an intervention or treatment, and researchers evaluate the effects of the intervention on health-related outcomes.

Medical Benefit

Real-world evidence to guide coverage, dosing, and site-of-care decisions for provider-administered drugs so that treatments are both clinically effective and cost-efficient.

Measurement Bias

Occurs when data is misclassified, or there is an inaccurate measurement of exposures, outcomes, or confounders. Also referred to as information bias.

N

Natural History of Disease

The course of a disease from inception to resolution (recovery or death).

Non-Intervention Study Design

Observational study design of which patient data is obtained and evaluated in routine clinical setting(s).

Non-Randomized Study Design

Participants are not assigned (by chance) to different treatment groups.

Number Needed to Harm

The number of patients that must receive a treatment for one additional patient to experience an adverse drug event.

Number Needed to Treat

The number of patients that need to be treated to prevent one event.

O

Observational Studies

Study that documents or analyzes the relationship between the exposure and the outcome. The exposure or intervention is not assigned by the investigator.

Odds Ratio

Compares the odds of experiencing an event versus not experiencing the event between two groups.

Off-Label Use

Prescribing trends outside the FDA-approved use.

Open Claims Data

Data that is aggregated across multiple payers and sources. Offers a broader market view but may miss details like copay amounts or continuity of care.

Outcome-Based Contracts

Some payers use RWE to track performance metrics tied to payment.

P

Patient Registry Data

Provides epidemiology of disease incidence, prevalence, and trends for disease monitoring. Registries are managed by federal, state, hospital, researchers, or industry/pharmaceutical organizations.

Patient-Reported Outcomes (PROs)

Additional context to the quality of life or disease severity, beyond objective outcomes measures.

Payers

To inform value-based and evidence-informed decision-making.

Pharmacy-Based Claims Data

Data collected by community, mail, and specialty pharmacies for billing or reimbursement purposes via electronic form.

Placebo Effect

The effect on a patient's health of his or her belief that he or she is receiving a given intervention.

Pre-approved Product Dossier

A document that compiles evidence intended to help stakeholders understand the disease, unmet need, and potential value of a therapy prior to launch.

Pre-Approval Information Exchange (PIE) Act

Allows pharmaceutical manufacturers to share economic and scientific data on drugs with health payers before FDA approval.

Population-Based Registries

Capture all cases in a defined geographic area, which are used for public health and epidemiology.

Post-Approval (Updated Approved Product Dossier)

A standardized document that incorporates mature real-world evidence that builds on earlier data. It reassesses value, safety, effectiveness, and utilization based on real-world experience.

Pragmatic Trial

A controlled clinical trial designed to measure the benefit of an intervention in normal practice (effectiveness) to help guide decisions between options for care.

Prospective Observational Studies

A prospective study follows subject(s) over a period of time to determine how specific factors lead to outcomes. Includes non-interventional cohort studies using primary data and registry studies.

Primary Real-World Data

Any data collected specifically for research purposes, (e.g., prospective studies, patient record forms, surveys.)

Q

Quality Metrics

Health plan performance is assessed through quality measures from various organizations, such as the National Committee for Quality Assurance (NCQA) and the Centers for Medicare and Medicaid (CMS). Many of these metrics involve drug utilization rates such as adherence, polypharmacy, and guideline-concordant treatment for chronic disease management.

R

Randomized Controlled Trials (RCTs)

An experimental study (controlled trial) in which participants are randomly assigned to treatment groups (experimental and control groups).

Real-World Data (RWD)

Data relating to patient health status and/or the delivery of health care are routinely collected from EHRs, registries, claims, patient-generated data, mobile health apps, and wearable devices.

Real-World Evidence (RWE)

The clinical evidence about the usage and potential benefits or risks of a medical product is derived from analysis of real-world data.

Regulators

To inform decisions on new indications, post-market studies (e.g., of safety), and supplement traditional clinical evidence.

Relative Risk

Compares the probability of an event between two groups.

Relative Risk Reduction

How much the treatment reduced the risk of an outcome occurring compared to the control.

Relevance

Data that is fit for purpose and includes an assessment of whether the data captures relevant data on exposure, outcomes, and covariates.

Reliability

The extent to which an instrument, scale, or other type of measurement or procedure yields consistent and reproducible results.

Retrospective Chart Abstractions

Includes studies based on a manual pull of data from patient charts.

Retrospective Study

A study that looks back at factors as they relate to an outcome that is established at the start of the study.

Risk Assessment

The process of evaluating the probability of an adverse event occurring. The estimation can be either qualitative or quantitative.

S

Safety Monitoring

Reveals a new drug-drug interaction or adverse drug signals that were underreported or never identified in clinical trials.

Selection Bias

Occurs when physicians prescribe certain treatments to patients they believe will respond better to the treatment. The outcomes may appear improved due to patient selection, not the treatment itself.

Secondary Real-World Data

Any data collected for other purposes than specific research objectives, (e.g., EMR, population-wide registries, administrative claims data, pharmacy claims data.)

Specificity

The proportion of time for a diagnostic test is negative in individuals who do not have the disease or condition. A specific test has a low false-positive rate. Calculation: Specificity = TN / (TN + FP), where TN = true negative and FP = false positive.

Survivorship Bias

Occurs when data from only live patients are captured, and data from deceased patients are not.

T

Target Trial Emulation

The development of a protocol for a hypothetical randomized control trial and applying that framework to real-world data.

Tokens

Enables current or future linkage of RWD to other RWD or clinical trial data without having to regather PII & reconsent; tokenization can facilitate linking datasets having the same tokens.

Transparency

Report limitations and subgroup differences to prevent overgeneralization and inequitable decision-making.

U

Utilization Management

RWE informs prior authorization, step therapy, and eligibility criteria, especially when trial populations differ from practice.

V

Validation

The process of testing and accumulating evidence that supports the valid use or interpretation of results from a measure or study.

Value Assessment

A method to measure the outcomes or benefits of a treatment or intervention relative to its cost.

Value-Based Contracts (VBC)

Performance based and involves reimbursement tied to a health outcome over a set timeframe.

Value Reassessment

Payers use RWE to reassess outcomes, durability, and costs over time, particularly for high-cost therapies.

GLOSSARY

Common Abbreviations

Abbreviation	Full Term
ARR	Absolute risk reduction
EHR	Electronic health record
HCDM	Health care decision maker
HCEI	Health care economic information
HTA	Health technology assessment
NNH	Number needed to harm
NNT	Number needed to treat
OR	Odds ratio
PIE	Pre-approval information exchange
PRO	Patient-reported outcomes
RCTs	Randomized controlled trials
RR	Relative risk
RRR	Relative risk reduction
RWD	Real-world data
RWE	Real-world evidence
TTE	Target trial emulation
UM	Utilization management
VBC	Value-based contracts

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