Measurement of outcomes in pharmacoepidemiology

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Definition

- Pharmacoepidemiology evaluates the **effects**, **risks**, **benefits**, and **patterns** of drug use in populations. To assess these factors, **outcomes** must be clearly defined and measured.

What are outcomes?

- In pharmacoepidemiology, **outcomes** are the **health events or conditions** that occur in response to drug exposure. These can be either:
    - **Beneficial outcomes** (e.g., symptom relief, cure)
    - **Adverse outcomes** (e.g., side effects, toxicity, drug interactions)

Types of outcomes measured:

1. **Clinical outcomes** – measurable health status changes (e.g., blood pressure reduction, cancer remission).
2. **Surrogate outcomes** – indirect measures of clinical benefit (e.g., cholesterol levels as a surrogate for heart disease risk).
3. **Patient-reported outcomes** – subjective reports from patients (e.g., quality of life, pain level).
4. **Economic outcomes** – cost-effectiveness or healthcare resource use.
5. **Drug safety outcomes** – adverse drug reactions (ADRs), toxicity, hospitalization due to medication, or mortality.

Why measure outcomes?

- To determine drug **effectiveness** and **safety** in real-world use.
- To assess **risk-benefit** ratios.
- To guide regulatory decisions, clinical guidelines, and public health policy.

Outcome Measures in Pharmacoepidemiology

These are metrics used to **quantify and compare outcomes** in different populations or drug exposures.

Types of Outcomes

1. **Therapeutic outcomes:**
    - ➢ Desired clinical effects (e.g., blood pressure control by antihypertensive drugs).
2. **Adverse drug events (ADEs):**

- Unwanted effects like gastrointestinal bleeding from NSAIDs.

3. Patient-reported outcomes:

- Measured through validated questionnaires (e.g., pain score, quality of life).

4. Surrogate outcomes:

- Indirect markers assumed to reflect clinical benefit (e.g., LDL cholesterol as a surrogate for cardiovascular risk).

Common Outcome Measures

A. Risk (Cumulative Incidence):

- The proportion of individuals developing an outcome during a specified time.
- Risk = (Number of new cases during a time period) / (Number of individuals at risk at start of the period).

B. Incidence Rate (Incidence Density):

- Measures how quickly new cases of an outcome occur in a population.
- Incidence Rate = (Number of new cases) / (Total person-time at risk).

C. Prevalence:

- The proportion of individuals with a condition at a specific point in time or over a period of time.
- Point Prevalence = (Number of existing cases at a specific time) / (Total population at that time).

D. Odds Ratio (OR):

- Used mainly in case-control studies.
- Compares the odds of exposure among cases to odds of exposure among controls.

E. Relative Risk (RR):

- Used in cohort studies.
- Risk of outcome in exposed group divided by risk in unexposed group.

F. Number Needed to Treat (NNT):

- Number of patients who need to be treated to prevent one additional bad outcome.
- NNT = 1 / Absolute Risk Reduction (ARR).

G. Hazard Ratio (HR):

- Used in survival analysis.
- Compares the hazard (instantaneous event rate) between two groups over time.

Sources of Outcome Data

- Medical records
- Administrative claims data
- Disease registries
- Patient self-reporting
- Prescription databases

Challenges in Outcome Measurement

- **Misclassification bias**: Occurs when outcomes are incorrectly categorized.
- **Incomplete data**: Missing or under-reported outcomes reduce reliability.
- **Latency periods**: Some drug effects appear only after prolonged use.

Drug Use Measures in Pharmacoepidemiology

Measuring **how drugs are used** in the population is also central in pharmacoepidemiology.

Common Drug Use Measures

1. **Defined Daily Dose (DDD):**
   - The assumed average maintenance dose per day for a drug used for its main indication in adults.
   - Allows comparison of drug consumption across different populations and settings.
2. **Prescribed Daily Dose (PDD):**
   - The average dose that is actually prescribed.
3. **Medication Possession Ratio (MPR):**
   - A measure of adherence.
   - MPR = (Total days of medication supplied) / (Number of days in the observation period).
4. **Proportion of Days Covered (PDC):**
   - Another adherence measure.
   - PDC = (Number of days with medication on hand) / (Number of days in period).
5. **Drug Utilization Rate:**
   - Describes the proportion of the population that uses a specific drug or drug class over a given time.

Basic Epidemiological Measures

Prevalence

Definition:

- Prevalence refers to the **proportion of a population** that has a specific characteristic (such as drug use or a health condition) at a particular point in time or over a specified period.

Types:

1. **Point prevalence** – proportion at a specific point in time.
2. **Period prevalence** – proportion over a defined time interval.

Formula:

$$\text{Prevalence} = \frac{\text{Number of existing cases}}{\text{Total population}} \times 100$$

Example:

- "In 2024, 8% of adults were using antidepressants."
- If 1,000 people in a city of 10,000 are on antihypertensive drugs as of September 30, the point prevalence is:

$$\frac{1000}{10000} \times 100 = 10\%$$

Uses in Pharmacoepidemiology:

- Quantify **burden of disease or drug use**
- Plan **healthcare services and resource allocation**
- Compare **drug usage trends across populations or regions**

Incidence

Definition:

- Incidence refers to the **number of new cases** (e.g., new drug users or new adverse events) occurring in a population over a defined time period.

Types:

- **Cumulative Incidence**: Total number of new cases over a time frame
- **Incidence Rate (See below)**: Includes person-time denominator

Formula:

$$\text{Incidence} = \frac{\text{Number of new cases during a time period}}{\text{Population at risk during that time}} \times 1000$$

Example:

- "The incidence of first-time use of insulin in diabetics was 25 per 1,000 persons per year."
- If 200 new cases of drug-induced liver injury are reported among 10,000 patients over 1 year:

$$\text{Incidence} = \frac{200}{10000} \times 1000 = 20 \text{ per } 1000 \text{ persons per year}$$

Uses in Pharmacoepidemiology:

- Evaluate **risk of disease** or **adverse drug events**
- Compare **emergence of side effects** between drugs
- Study **time patterns** (e.g., seasonal variation in drug-related events)

Incidence Rate (Incidence Density)

Definition:

- Incidence rate is a more precise measure of incidence that accounts for the **time at risk** (person-time). It tells us how quickly new cases occur in a population.

Formula:

$$\text{Incidence Rate} = \frac{\text{Number of new cases}}{\text{Total person-time at risk}}$$

- **Person-time** = the sum of individual periods during which people were at risk and observed.
- **Units**: cases per person-year (or other time units)

Example:

- "There were 3 cases of liver toxicity per 10,000 person-years of statin use."

Example:

- In a study with 500 new users of a drug, followed for a total of 2,500 person-years, if 25 adverse reactions are recorded:

$$\text{Incidence Rate} = \frac{25}{2500} = 0.01 \text{ per person-year}$$

- 10 adverse events per 1000 person-years

Importance:

- Useful when follow-up periods vary between individuals
- More accurate than simple incidence when dealing with **dynamic populations**

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