Biostatistics & Epidemiology/Population Health

United States Medical Licensing Examination (USMLE) Step 1

4 to 6% of Step 1 · 7 sections · content outline and specifications

This system covers biostatistics, epidemiology, population health, and how to read medical research. It is about 4% to 6% of Step 1, and its items test practice-based learning and improvement. Many questions give a 2 x 2 table, a results table, or a short study description. You must name the design, find the bias, or calculate a measure such as sensitivity, relative risk, or the number needed to treat. Most calculations are short if you set up the table carefully and check which row or column is the denominator.

You should be able to: After this guide, a learner should be able to name a study design, spot its main bias, and calculate and interpret the common measures from a 2 x 2 table.

Every section: the key point and the trap

1 Measures of disease frequency and health status

Incidence = new cases / population at risk over a period of time. Prevalence = all existing cases / total population at one time.

Trap: Do not let a rising prevalence convince you that more new cases are occurring. Ask whether the duration of disease changed or whether detection improved.

2 Study designs

Case report and case series: describe patients; no comparison group; they generate hypotheses.

Trap: Name the design by how participants were selected, not by when the data were collected. A cohort study can be retrospective if participants are grouped by past exposure.

3 Measures of association and impact

Relative risk (RR) = incidence in exposed / incidence in unexposed (cohort studies and trials). Odds ratio (OR) = ad / bc (case-control studies); it is close to the RR when the disease is rare.

Trap: Do not confuse relative and absolute measures. The relative risk reduction often stays similar when baseline risk changes, but the NNT changes a lot.

4 Testing and screening

Sensitivity = TP / (TP + FN), the share of diseased people who test positive. A very sensitive test, when negative, helps rule a disease out.

Trap: Read the orientation of the table before you calculate. Sensitivity and specificity use the disease columns; predictive values use the test rows.

5 Statistics: distributions, hypothesis testing, and errors

Normal distribution: about 68%, 95%, and 99.7% of values fall within 1, 2, and 3 standard deviations of the mean.

Trap: A p value is not the probability that the null hypothesis is true. It is the probability of results at least this extreme if the null hypothesis were true.

6 Bias, confounding, and validity

Selection bias: the study groups do not represent the target population (for example, Berkson bias, the healthy worker effect, loss to follow-up).

Trap: Match the bias to the design. Recall bias belongs to case-control studies, loss to follow-up to cohorts and trials, and lead-time bias to screening.

7 Prevention, public health, and research ethics

Primary prevention stops disease before it starts (vaccines, quitting smoking). Secondary prevention finds disease early (screening). Tertiary prevention limits harm from established disease (rehabilitation).

Trap: Screening is secondary prevention, even though it is done in people who look healthy. Vaccination is primary prevention.

Key terms

Incidence: The number of new cases in a population at risk over a period of time.

Prevalence: The proportion of a population that has a disease at one time.

Confounder: A third factor linked to both exposure and outcome that distorts their apparent relationship.

Odds ratio: The odds of exposure in cases divided by the odds of exposure in controls.

Likelihood ratio: How much a test result changes the odds of disease.

Power: The probability that a study finds a true difference when one exists.

Confidence interval: A range of values that likely contains the true value.

Intention-to-treat analysis: Analyzing every participant in the group to which they were randomized.

Lead-time bias: Apparent longer survival caused only by earlier diagnosis.

Number needed to treat: How many patients must be treated to prevent one bad outcome.

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