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Item 8 of 40 Question Id: 19391

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As part of the Food and Drug Administration drug approval process, a study is conducted to assess the clinical benefit and toxicity of a new drug that is intended to be used in combination with current standard chemotherapy for patients with recurrent glioblastoma. Fifty patients with recurrent glioblastoma enroll in the trial and are randomized to receive standard chemotherapy plus either placebo or 1 of 3 possible doses of the new drug. Study results show a dose-dependent reduction in tumor size with all 3 doses of the new drug, along with a significant increase in adverse drug effects, including hypertension, muscle weakness, lymphopenia, and hypophosphatemia. The researchers conclude that the middle dose of the new drug offers the greatest ratio of benefit to toxicity. Which of the following best describes this type of study?

- A. Phase I clinical trial (12%)
- B. Phase II clinical trial (48%)
- C. Phase III clinical trial (31%)
- D. Phase IV clinical trial (6%)
- E. Preclinical study (0%)

Omitted

Correct answer

B



48%

Answered correctly



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Time Spent



2023
Version

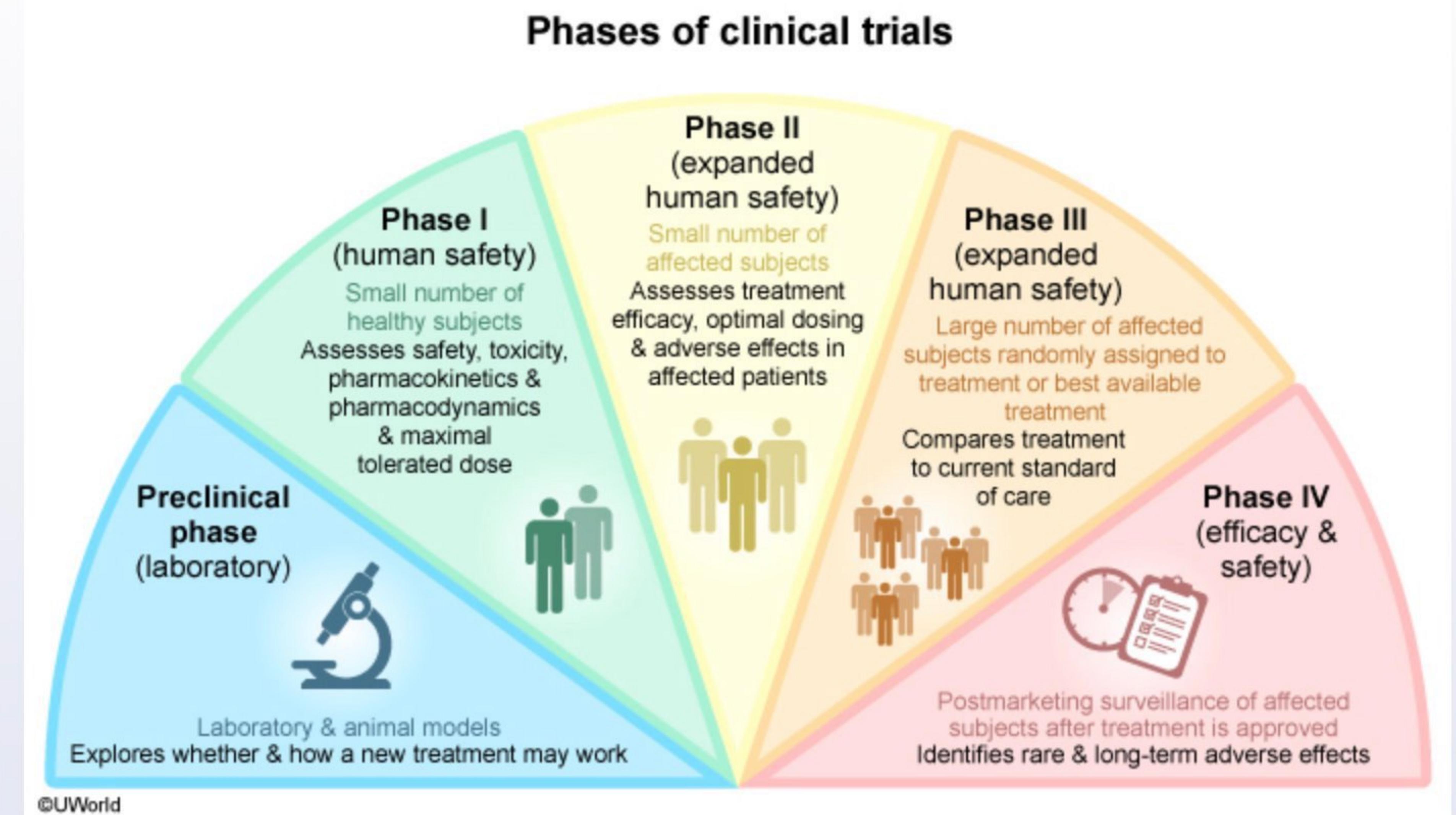
Explanation

Phases of clinical trials

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Efficacy and safety of new drugs are established during the **clinical trials process**. New drugs are approved by a regulatory body (eg, Food and Drug Administration) following review of phase I to III trials. Phases differ in size, objective, and participant selection.

- **Phase I:** small trials usually conducted with **healthy participants** to assess safety and pharmacokinetics, often performed in a strictly controlled environment with extensive biochemical and physiologic monitoring. Although some phase I chemotherapy trials may involve subjects with cancer due to the inherent toxicity of treatment, drug efficacy (eg, tumor size reduction) is not assessed during a phase I trial (**Choice A**).
- **Phase II:** small- to medium-sized trials conducted with participants having the condition of interest to

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- **Phase I:** small trials usually conducted with **healthy participants** to assess safety and pharmacokinetics, often performed in a strictly controlled environment with extensive biochemical and physiologic monitoring. Although some phase I chemotherapy trials may involve subjects with cancer due to the inherent toxicity of treatment, drug efficacy (eg, tumor size reduction) is not assessed during a phase I trial (**Choice A**).
- **Phase II:** small- to medium-sized trials conducted with participants having the condition of interest to assess **treatment efficacy**, toxicity, **adverse effects**, and **optimal dosing** strategies. These studies may or may not have a control group and can be called pilot studies.
- **Phase III:** **large trials** (typically >300 patients) that are adequately powered to fully assess treatment response and safety, often including analysis of treatment effects in selected subsets of the target patient population. These trials must show adequate effectiveness and safety compared to standard treatment for the drug to obtain **regulatory approval**.

(Choice C) This trial enrolled a small number of participants with recurrent glioblastoma and compared multiple drug doses with respect to treatment efficacy and toxicity. The results were reported to advise optimal drug dosing (ie, dose-finding); although drug dose correlated with a decrease in tumor size, effectiveness and safety outcomes were not compared with the standard treatment (eg, absolute/relative risks). Therefore, this study is best characterized as a phase II trial.

(Choice D) Phase IV trials are performed after a new drug has obtained regulatory approval for clinical use. These trials are typically performed to assess long-term benefits and risks or identify uncommon adverse effects that were not fully characterized in phase III studies.

(Choice E) In contrast to phase I, II, III, and IV trials, which include human subjects, preclinical studies do not involve human subjects.

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- **Phase III:** large trials (typically >300 patients) that are adequately powered to fully assess treatment response and safety, often including analysis of treatment effects in selected subsets of the target patient population. These trials must show adequate effectiveness and safety compared to standard treatment for the drug to obtain **regulatory approval**.

(Choice C) This trial enrolled a small number of participants with recurrent glioblastoma and compared multiple drug doses with respect to treatment efficacy and toxicity. The results were reported to advise optimal drug dosing (ie, dose-finding); although drug dose correlated with a decrease in tumor size, effectiveness and safety outcomes were not compared with the standard treatment (eg, absolute/relative risks). Therefore, this study is best characterized as a phase II trial.

(Choice D) Phase IV trials are performed after a new drug has obtained regulatory approval for clinical use. These trials are typically performed to assess long-term benefits and risks or identify uncommon adverse effects that were not fully characterized in phase III studies.

(Choice E) In contrast to phase I, II, III, and IV trials, which include human subjects, preclinical studies do not involve human subjects.

Educational objective:

Phase II studies are small- to medium-sized trials conducted with participants having the condition of interest to assess treatment efficacy, toxicity, adverse effects, and optimal dosing strategies; they are sometimes called pilot studies.

References

- Key concepts of clinical trials: a narrative review.

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Item 9 of 40 Question Id: 1302

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A researcher studying physician behavior is interested in how often primary care physicians take the sexual histories of patients during clinic visits. As part of the study, patients who attend a primary care clinic are asked to fill out a questionnaire immediately following a visit with their physician. Once the physicians become aware that their own behavior is being studied, which of the following is most likely to be a potential problem?

- A. Berkson's bias (9%)
- B. Hawthorne effect (69%)
- C. Lead-time bias (2%)
- D. Pygmalion effect (15%)
- E. Recall bias (2%)

Omitted
Correct answer
B

69%
Answered correctly

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Time Spent

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Explanation

The **Hawthorne effect (observer effect)** is the tendency of study subjects to change their behavior as a result of their awareness that they are being studied. This can impact the observed outcomes, thereby seriously affecting the validity of the study. The Hawthorne effect is commonly seen in studies concerning behavioral outcomes or outcomes that can be influenced by behavioral changes. In this example, physicians (not patients) are the subjects of the study; those physicians who are aware that they are being studied may modify their behavior and

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Question Id: 1302

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their awareness that they are being studied. This can impact the observed outcomes, thereby seriously affecting the validity of the study. The Hawthorne effect is commonly seen in studies concerning behavioral outcomes or outcomes that can be influenced by behavioral changes. In this example, physicians (not patients) are the subjects of the study; those physicians who are aware that they are being studied may modify their behavior and start taking sexual histories. To minimize the Hawthorne effect, study subjects can be kept unaware that they are being studied, but this can occasionally pose ethical problems.

(Choice A) Berkson's bias refers to selection bias created by choosing hospitalized patients as the control group.

(Choice C) Lead-time bias refers to the apparent prolongation of survival after applying a screening test that detects a disease earlier than it would have been otherwise detected but without any real effect on prognosis.

(Choice D) Pygmalion effect describes the fact that a researcher's beliefs in the efficacy of treatment can potentially affect the outcome. In the classic classroom experiment that first described the Pygmalion effect, a group of students were randomly assigned high intelligence quotient (IQ) scores; their teachers were then told of these artificial results and had higher expectations of this group as a result. The students with the randomly assigned high IQ scores actually performed better, likely because their teachers unconsciously behaved in a manner that would facilitate their success.

(Choice E) Recall bias results from inaccurate recall of past exposures by patients. Although recall bias is possible whenever questionnaires are administered, it is unlikely in this case as the patients fill out a form immediately upon leaving the doctor's office.

Educational objective:

The Hawthorne effect (observer effect) is the tendency of study subjects to change their behavior as a result of their awareness that they are being studied.

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Item 10 of 40 Question Id: 19742

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A group of sports physicians plans to conduct a case-control study to investigate a possible association between adolescent idiopathic scoliosis (AIS) and sacroiliac joint (SIJ) dysfunction in young athletes. The case group will consist of young athletes who were diagnosed with AIS during a regular checkup by a sports physician. Which of the following is the most appropriate control group for this study?

- A. Young athletes with a diagnosis of AIS and SIJ (3%)
- B. Young athletes with a diagnosis of AIS but not of SIJ (6%)
- C. Young athletes with a diagnosis of AIS irrespective of SIJ status (4%)
- D. Young athletes with no diagnosis of AIS irrespective of SIJ status (57%)
- E. Young athletes with no diagnosis of AIS or SIJ (22%)
- F. Young nonathletes with a diagnosis of AIS but not of SIJ (2%)
- G. Young nonathletes with a diagnosis of AIS irrespective of SIJ status (2%)

Omitted
Correct answer
D



57%
Answered correctly



02 secs
Time Spent



2023
Version

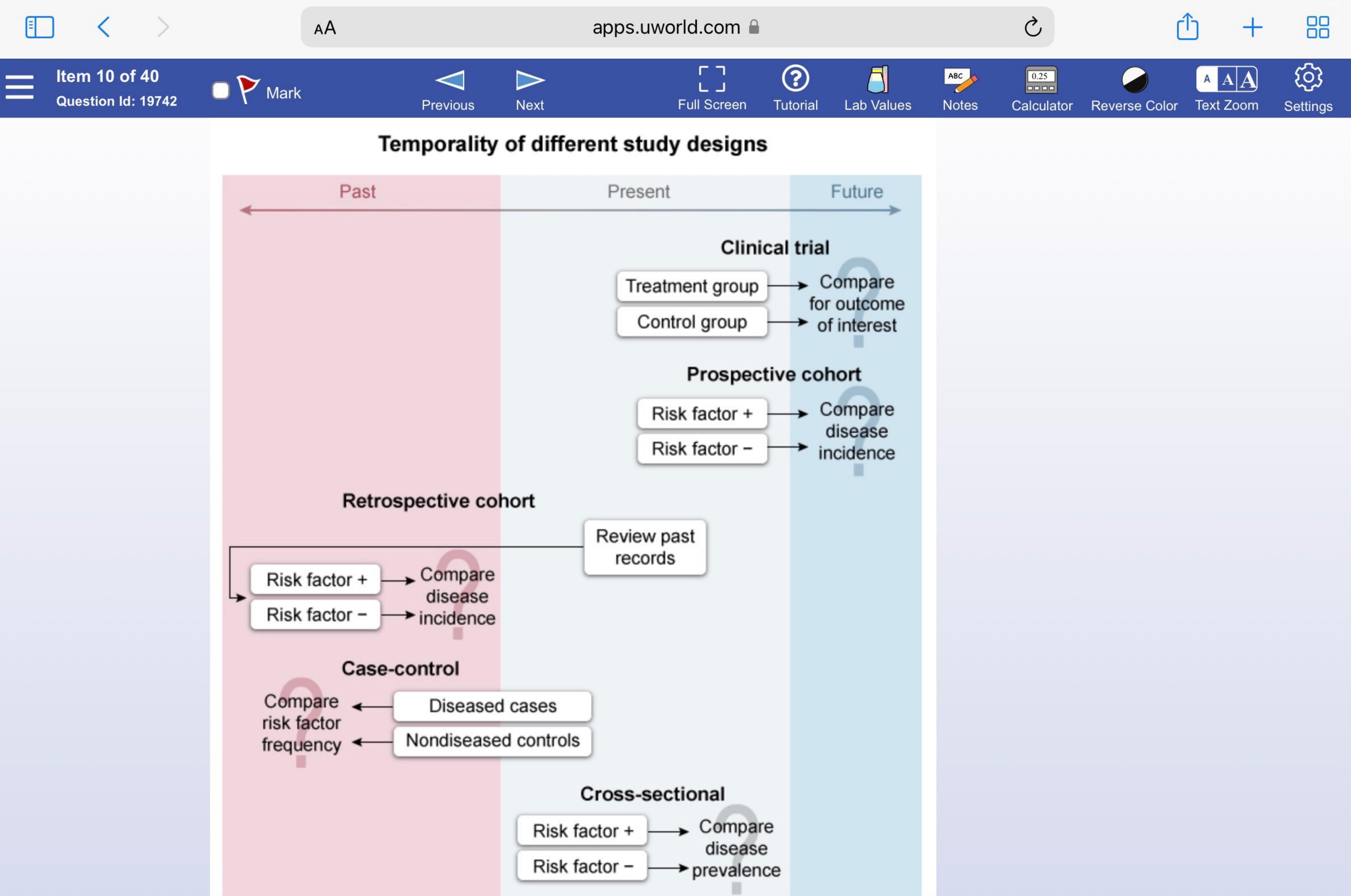
Explanation

Temporality of different study designs

Past

Present

Future



The screenshot shows a mobile application interface for a medical study. At the top, there is a navigation bar with icons for back, forward, and search. The URL 'apps.uworld.com' is displayed in the address bar. Below the address bar, there is a toolbar with various icons: 'Item 10 of 40', 'Mark' (with a red flag icon), 'Previous' and 'Next' arrows, 'Full Screen', 'Tutorial', 'Lab Values', 'Notes' (with a pencil icon), 'Calculator' (with a '0.25' icon), 'Reverse Color', 'Text Zoom' (with a 'A A A' icon), and 'Settings' (with a gear icon). The main content area contains a text block describing a case-control study design.

A **case-control** study is an observational design in which potential participants are initially identified as **cases** or **controls** according to the **dependent variable or outcome** (eg, disease of interest). Once cases and controls are identified, the presence of past exposure to ≥ 1 **risk factors** of interest is determined in each group. Finally, the **frequency** of exposure to the risk factors is compared between cases and controls to estimate the **association** between the risk factors and the outcomes. If there is a statistically significant difference in the frequency of exposure to the risk factor between the 2 groups, it is likely that the risk factor in question is associated with the disease.

In this example:

- The population of interest is young athletes. Therefore, both the cases and control groups must consist of young athletes (**Choices F and G**).
- The disease of interest (ie, what defines a case) is adolescent idiopathic scoliosis (AIS). Therefore, the cases must have AIS and controls must not have AIS (**Choices A, B, and C**).
- The risk factor of interest is sacroiliac joint (SIJ) dysfunction. Cases and controls must be selected irrespective of SIJ status because the presence of SIJ is what is compared between cases and controls (**Choice E**).

Therefore, the cases are young athletes with a diagnosis of AIS irrespective of SIJ status; the **controls** are **young athletes with no diagnosis of AIS irrespective of SIJ status** because what determines whether the disease (ie, AIS) is associated with the risk factor (ie, SIJ) is the difference in the frequency of the risk factor between cases and controls.

Educational objective:

A case-control study is an observational study design; it begins with individuals who have the outcome ("cases")

The screenshot shows a mobile application interface for a case-control study analysis. At the top, there is a navigation bar with icons for back, forward, and search. The URL 'apps.uworld.com' is displayed in the address bar. Below the address bar, there is a toolbar with various icons: 'Item 10 of 40', 'Mark' (with a red flag icon), 'Previous' and 'Next' arrows, 'Full Screen', 'Tutorial', 'Lab Values', 'Notes', 'Calculator', 'Reverse Color', 'Text Zoom', and 'Settings'. The main content area contains text about case-control studies, mentioning risk factors, frequency, association, and statistical significance. It also includes an example involving young athletes and adolescent idiopathic scoliosis (AIS). The bottom of the screen shows a blue footer bar with the text 'Test Id: 302978480' and 'Scanned with CamScanner'.

are identified, the presence of past exposure to ≥ 1 **risk factors** of interest is determined in each group. Finally, the **frequency** of exposure to the risk factors is compared between cases and controls to estimate the **association** between the risk factors and the outcomes. If there is a statistically significant difference in the frequency of exposure to the risk factor between the 2 groups, it is likely that the risk factor in question is associated with the disease.

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- The risk factor of interest is sacroiliac joint (SIJ) dysfunction. Cases and controls must be selected irrespective of SIJ status because the presence of SIJ is what is compared between cases and controls (**Choice E**).

Therefore, the cases are young athletes with a diagnosis of AIS irrespective of SIJ status; the **controls** are **young athletes with no diagnosis of AIS irrespective of SIJ status** because what determines whether the disease (ie, AIS) is associated with the risk factor (ie, SIJ) is the difference in the frequency of the risk factor between cases and controls.

Educational objective:

A case-control study is an observational study design; it begins with individuals who have the outcome ("cases") and compares them with individuals who do not have the outcome ("controls") according to history of exposure to ≥ 1 risk factors.



AA

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Question Id: 108159

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A clinical study compares several cut points for a new biomarker for bacterial bloodstream infection among adult patients in the intensive care unit (ICU) against blood culture results. Analysis shows that a biomarker value $\geq 2.6 \text{ ng/mL}$ has a sensitivity of 32% and a specificity of 96% for predicting bloodstream infections. Which of the following conclusions about the study results is correct?

- A. Based on a cut point of $\geq 2.6 \text{ ng/mL}$, 4% of adult patients in the ICU with bacterial bloodstream infections will be correctly identified.
- B. Based on a cut point of $\geq 2.6 \text{ ng/mL}$, 4% of adult patients in the ICU without bacterial bloodstream infections will be incorrectly identified. (47%)
- C. Based on a cut point of $\geq 2.6 \text{ ng/mL}$, 96% of adult patients in the ICU with bacterial bloodstream infections will be correctly identified. (30%)
- D. Based on a cut point of $\geq 2.6 \text{ ng/mL}$, 96% of adult patients in the ICU without bacterial bloodstream infections will be incorrectly identified. (5%)
- E. Of all adult patients in the ICU with bacterial bloodstream infections, 4% will have a biomarker value $< 2.6 \text{ ng/mL}$. (7%)
- F. Of all adult patients in the ICU with bacterial bloodstream infections, 96% will have a biomarker value $< 2.6 \text{ ng/mL}$. (4%)

Omitted

Correct answer

B



47%

Answered correctly



02 secs

Time Spent



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	Positive condition	Negative condition	
Positive test result	TP	FP	$PPV = \frac{TP}{TP + FP}$
Negative test result	FN	TN	$NPV = \frac{TN}{TN + FN}$
	Sensitivity = $TP / (TP + FN)$	Specificity = $TN / (TN + FP)$	

FN = false negative; **FP** = false positive; **NPV** = negative predictive value; **PPV** = positive predictive value; **TN** = true negative; **TP** = true positive.

Compared to blood culture (the reference standard for diagnosing bacterial bloodstream infection), a biomarker value cut point of ≥ 2.6 ng/mL has a sensitivity of 32% and a **specificity of 96%**.

- The **specificity** of the test is the percentage of **healthy individuals** (eg, adult patients in the intensive care unit [ICU] without bacterial bloodstream infection) who are **correctly identified** (ie, negative test result) by the diagnostic test. Its complement ($1 - \text{specificity}$) is the false-positive rate (eg, percentage of adult patients in the ICU without bacterial bloodstream infection who test positive).
- The sensitivity of the test is the percentage of diseased individuals (eg, ICU patients with bacterial bloodstream infection) who are correctly identified (ie, positive test result) by the diagnostic test. Its complement ($1 - \text{sensitivity}$) is the false-negative rate (eg, percentage of adult patients in the ICU with bacterial bloodstream infection who test negative).

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The top navigation bar includes icons for back/forward, search, and other functions. Below it, a blue header bar contains the text "Item 11 of 40" and "Question Id: 108159". To the right are icons for marking the question, previous/next, full screen, tutorial, lab values, notes, calculator, reverse color, text zoom, and settings.

unit [ICU] without bacterial bloodstream infection) who are **correctly identified** (ie, negative test result) by the diagnostic test. Its complement ($1 - \text{specificity}$) is the false-positive rate (eg, percentage of adult patients in the ICU without bacterial bloodstream infection who test positive).

- The sensitivity of the test is the percentage of diseased individuals (eg, ICU patients with bacterial bloodstream infection) who are correctly identified (ie, positive test result) by the diagnostic test. Its complement ($1 - \text{sensitivity}$) is the false-negative rate (eg, percentage of adult patients in the ICU with bacterial bloodstream infection who test negative).

This test's specificity indicates that 96% of adult patients in the ICU without bacterial bloodstream infection will be correctly identified (ie, test negative) based on the biomarker cut point of $\geq 2.6 \text{ ng/mL}$ (ie, test result $< 2.6 \text{ ng/mL}$) (**Choice D**). Conversely, $100\% - 96\% = 4\%$ of adult patients in the ICU **without** bacterial bloodstream infection will be **incorrectly identified**.

(Choices A, C, E, and F) This test's sensitivity indicates that 32% of adult patients in the ICU with bacterial bloodstream infection will be correctly diagnosed (ie, test positive) based on the biomarker cut point of $\geq 2.6 \text{ ng/mL}$. Conversely, $100\% - 32\% = 68\%$ of adult patients in the ICU with bacterial bloodstream infection will be missed (ie, test negative with a result $< 2.6 \text{ ng/mL}$).

Educational objective:

Sensitivity represents the probability that an individual with disease will have a positive test result. Specificity represents the probability that an individual without disease will have a negative test result.

Biostatistics

Biostatistics & Epidemiology

Subject

System

Sensitivity and specificity

Topic

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Item 12 of 40 Question Id: 14862

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Researchers want to estimate the association between environmental lead exposure and cognitive deficits in children. Among all children who received preventive care at 5 local pediatric clinics over the last 5 years, they identify 20 children diagnosed with cognitive deficits and 40 without the diagnosis. The researchers then review the patients' medical records and calculate the distance between the patients' residence and known lead-contaminated geographic areas. Which of the following best describes the study design used by the researchers?

- A. Case-control study (68%)
- B. Case series study (10%)
- C. Cross-sectional study (16%)
- D. Prospective cohort study (4%)
- E. Randomized control trial (0%)

Omitted
Correct answer
A

68%
Answered correctly

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Time Spent

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Version

Explanation

Temporality of different study designs

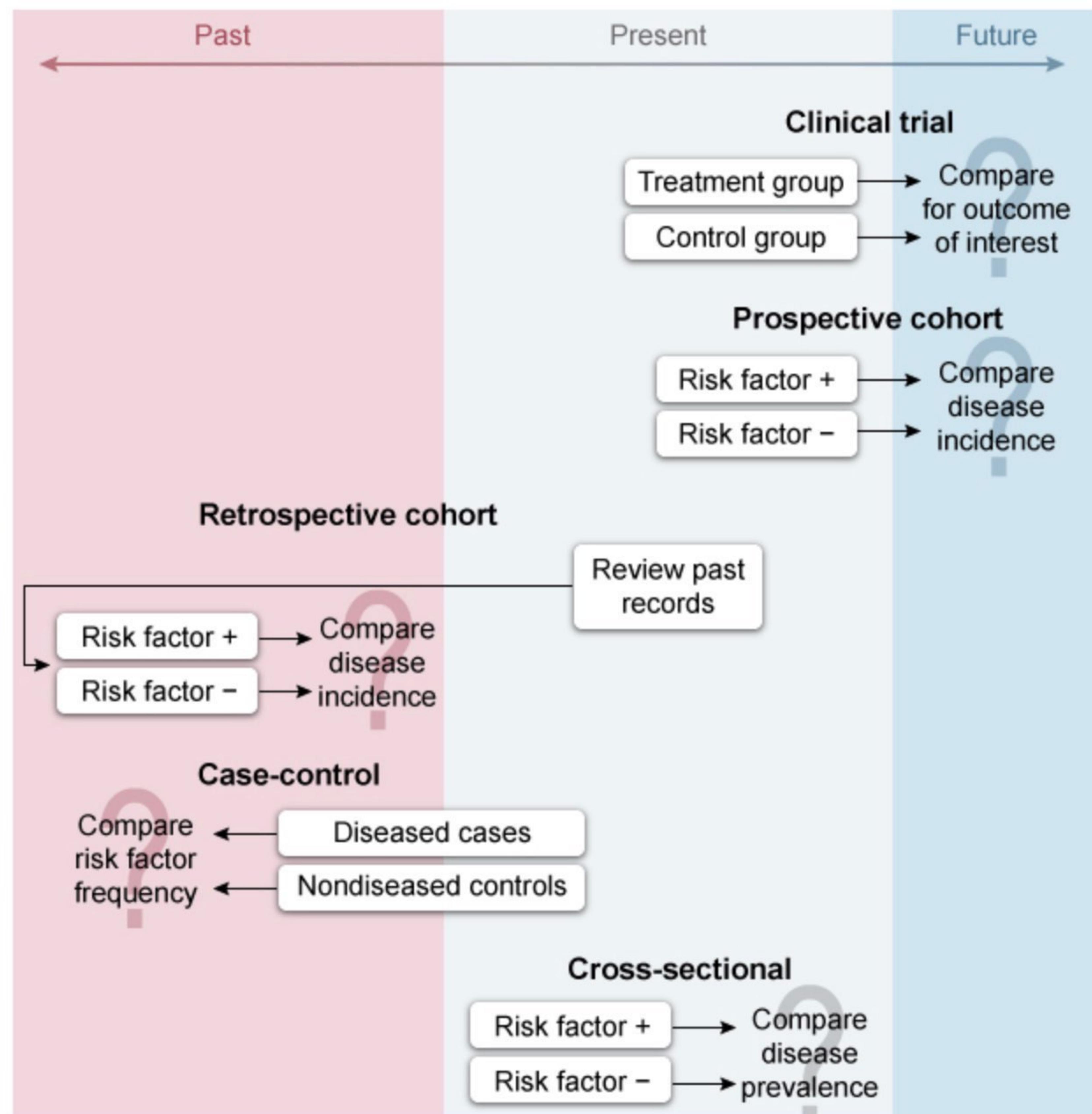


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Temporality of different study designs



The screenshot shows a mobile application interface for a medical study. At the top, there is a navigation bar with icons for back, forward, and search. The URL 'apps.uworld.com' is displayed in the address bar. Below the address bar, there is a toolbar with various icons: 'Item 12 of 40', 'Mark' (with a red flag icon), 'Previous' and 'Next' arrows, 'Full Screen', 'Tutorial', 'Lab Values', 'Notes' (with a pencil icon), 'Calculator' (with a '0.25' icon), 'Reverse Color', 'Text Zoom' (with a 'A A A' icon), and 'Settings' (with a gear icon). The main content area contains text about observational studies.

Observational studies (eg, case series, case-control studies, cohort studies, cross-sectional studies) differ from experimental studies (eg, clinical trials) in that the researchers passively observe the study participants rather than monitor the result of a specific intervention. Observational studies can be subdivided as follows:

- Descriptive studies collect data to characterize a specific health problem (eg, disease prevalence, incidence rates).
- Analytical studies test hypotheses to evaluate associations between risk factors and disease.

A **case-control** study is an analytical observational design in which potential participants are initially identified as "**cases**" or "**controls**" according to an **outcome status** (ie, disease of interest). In this example, children diagnosed with cognitive deficits are cases and children without cognitive deficits are controls. Once identified, cases and controls are then **assessed retrospectively** for exposure to **risk factors** of interest. In this case, the risk factor of interest is environmental lead exposure, as measured by the distance between the patients' residence and known lead-contaminated areas. Finally, the prevalence of exposure to the risk factors (ie, frequency of exposure) is compared between cases and controls to estimate the **association** between the risk factors and the outcomes.

(Choice B) A case series study is a descriptive study that tracks patients with a known condition (eg, exposure, risk factor, disease) to document the natural history or response to treatment. Unlike a case-control study, a case series is purely descriptive and cannot establish associations.

(Choice C) Cross-sectional studies may evaluate associations between risk factors and outcomes of interest. However, unlike in case-control studies (participants selected based on the outcome status), the participants in a cross-sectional study are selected based on the inclusion and exclusion criteria set for the study.

(Choice D) In cohort studies, 2 groups of individuals (ie, cohorts) are initially identified based on their exposure status to a specific risk factor (eg, environmental lead exposure) rather than based on their outcome status (eg,

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Mark

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frequency of exposure) is compared between cases and controls to estimate the **association** between the risk factors and the outcomes.

(Choice B) A case series study is a descriptive study that tracks patients with a known condition (eg, exposure, risk factor, disease) to document the natural history or response to treatment. Unlike a case-control study, a case series is purely descriptive and cannot establish associations.

(Choice C) Cross-sectional studies may evaluate associations between risk factors and outcomes of interest. However, unlike in case-control studies (participants selected based on the outcome status), the participants in a cross-sectional study are selected based on the inclusion and exclusion criteria set for the study.

(Choice D) In cohort studies, 2 groups of individuals (ie, cohorts) are initially identified based on their exposure status to a specific risk factor (eg, environmental lead exposure) rather than based on their outcome status (eg, cognitive deficits), as seen in this example. The cohorts are then followed over time for development of the outcome.

(Choice E) A randomized clinical trial would follow individuals who have been randomized to either a treatment arm or a control arm and compare the effect of the intervention.

Educational objective:

A case-control study is an observational study design; it begins with selecting individuals who have the outcome ("cases") and individuals who do not have the outcome ("controls") and then retrospectively comparing their history of exposure to risk factors.

Biostatistics

Subject

Biostatistics & Epidemiology

System

Study designs

Topic

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Item 13 of 40 Question Id: 1176

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A study is designed to evaluate the efficacy of a new drug, KM28. The study will compare KM28 plus standard care versus standard care alone with regard to decreasing the incidence of recurrent breast cancer. The Food and Drug Administration (FDA) will approve the new drug if KM28 plus standard care decreases the rate of breast cancer recurrence by at least 40% compared to standard therapy alone. The recurrence rate on standard therapy is found to be 8%. In order for the FDA to approve KM28, what is the maximal incidence of recurrent disease acceptable for women treated with KM28 plus standard therapy?

- A. 2.8% (2%)
- B. 3.2% (23%)
- C. 3.6% (7%)
- D. 4.8% (62%)
- E. 5.2% (3%)

Omitted
Correct answer
D

62%
Answered correctly

02 secs
Time Spent

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Version

Explanation

The new drug, KM28, will be approved if its associated recurrence rate is decreased by at least 40% compared to the recurrence rate on standard therapy alone, which is given as 8%. As $40\% \text{ of } 8\% = 0.40 \times 8\% = 3.2\%$, the maximum acceptable recurrence rate is $8\% - 3.2\% = 4.8\%$. Another quick solution would be to state that the

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The new drug, KM28, will be approved if its associated recurrence rate is decreased by at least 40% compared to the recurrence rate on standard therapy alone, which is given as 8%. As 40% of 8% is $0.40 \times 8\% = 3.2\%$, the maximum acceptable recurrence rate is $8\% - 3.2\% = 4.8\%$. Another quick solution would be to state that the maximum acceptable recurrence rate is 60% of 8%, which is $0.60 \times 8\% = 4.8\%$.

An alternate solution involves using relative and absolute risk calculations. The 40% (ie, 0.4) mentioned in the prompt refers to **relative risk reduction (RRR)**, defined as the percent reduction in absolute risk (AR) between the treatment group (eg, KM28 + standard therapy) and the control group (eg, standard therapy). The formula for RRR is:

$$\text{RRR} = (\text{AR}_{\text{control}} - \text{AR}_{\text{treatment}}) / \text{AR}_{\text{control}}$$

In this example, $\text{AR}_{\text{control}}$, which represents the risk of recurrence with standard therapy, is given as 8% or 0.08.

The formula can be rearranged to calculate $\text{AR}_{\text{treatment}}$:

$$\text{AR}_{\text{treatment}} = \text{AR}_{\text{control}} - (\text{RRR} \times \text{AR}_{\text{control}})$$

Plugging in the values for RRR and $\text{AR}_{\text{control}}$ gives:

$$\text{AR}_{\text{treatment}} = 0.08 - (0.4 \times 0.08) = 0.08 - 0.032 = 0.048 \text{ (ie, } 4.8\%)$$

RRR may overstate the effectiveness of an intervention. For example, a RRR of 50% occurs whether a drug decreases the incidence of a disease from 2% to 1% or from 50% to 25%. Clearly, the latter is of greater clinical significance.

Educational objective:

$$\text{Relative risk reduction} = (\text{absolute risk}_{\text{control}} - \text{absolute risk}_{\text{treatment}}) / \text{absolute risk}_{\text{control}}$$

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maximum acceptable recurrence rate is 60% of 8%, which is $0.60 \times 8\% = 4.8\%$.

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Plugging in the values for RRR and $\text{AR}_{\text{control}}$ gives:

$$\text{AR}_{\text{treatment}} = 0.08 - (0.4 \times 0.08) = 0.08 - 0.032 = 0.048 \text{ (ie, } 4.8\%)$$

RRR may overstate the effectiveness of an intervention. For example, a RRR of 50% occurs whether a drug decreases the incidence of a disease from 2% to 1% or from 50% to 25%. Clearly, the latter is of greater clinical significance.

Educational objective:

Relative risk reduction = $(\text{absolute risk}_{\text{control}} - \text{absolute risk}_{\text{treatment}}) / \text{absolute risk}_{\text{control}}$

Biostatistics

Biostatistics & Epidemiology

Risk

Subject

System

Topic

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A prospective cohort study was conducted to assess the role of daily alcohol consumption in the occurrence of breast carcinoma. The investigators reported a 5-year relative risk of 1.4 for people who consume alcohol daily compared to those who do not. The 95% confidence interval was 1.02-1.85. Which of the following p-values is most consistent with the results described above?

- A. 0.03 (68%)
- B. 0.06 (15%)
- C. 0.09 (6%)
- D. 0.11 (3%)
- E. 0.20 (5%)

Omitted
Correct answer
A

68%
Answered correctly

03 secs
Time Spent

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Version

Explanation

Relative risk (RR) is used in cohort studies to determine how strongly a risk factor (ie, exposure) is associated with an outcome. RR is the risk of an outcome (eg, breast cancer) in the exposed group (eg, individuals who consume alcohol daily) divided by the risk of that outcome in the unexposed group (eg, individuals who do not consume alcohol daily). If the RR = 1.0 (null value), then there is no association between the exposure and the disease. An RR >1.0 indicates that the exposure is associated with increased risk of disease. An RR <1.0

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consume alcohol daily) divided by the risk of that outcome in the unexposed group (eg, individuals who do not consume alcohol daily). If the RR = 1.0 (null value), then there is no association between the exposure and the disease. An RR >1.0 indicates that the exposure is associated with increased risk of disease. An RR <1.0 means that the exposure is associated with decreased risk of disease.

The RR by itself does not account for the possibility that chance alone is responsible for the results. The 95% confidence interval (CI) and p-value are 2 measures of **statistical significance** that can help strengthen the findings of a study using RR. For a result to be considered statistically significant, its corresponding CI must NOT contain the null value. When the **95% CI** does **not** include the null value, this gives a corresponding **p-value <0.05** and the association between exposure and outcome is considered statistically significant. A p-value <0.05 reflects that there is a very low probability that the result was due to chance alone; formally, the p-value is the probability of observing a given (or more extreme) result due to chance alone assuming that the null hypothesis is true.

In this example, the RR is 1.4 with a 95% CI of 1.02-1.85. It can be concluded that daily alcohol consumption is associated with an increased risk of breast carcinoma (RR >1) and that the findings are statistically significant (95% CI does not include the null value of 1.0). Therefore, the expected p-value would be <0.05.

(Choices B, C, D, and E) These options contain p-values >0.05, so the results would not be statistically significant. Note [the relationship between CI and p-value](#): a statistically significant 95% CI corresponds to a p-value <0.05; a statistically significant 99% CI (would also not include the null value and likely be wider than a 95% CI) corresponds to a p-value <0.01.

Educational objective:

A result is considered statistically significant if the 95% confidence interval does not cross the null value, which corresponds to a p-value <0.05.

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Item 15 of 40 Question Id: 19247

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A study is designed to describe the manifestations of coronavirus disease 2019 (COVID-19) on imaging studies, particularly on computerized tomography (CT) scans. Eleven patients with COVID-19 are recruited for the study, and their CT findings are studied and characterized. Which of the following best describes this study design?

- A. Case-control (4%)
- B. Case series (70%)
- C. Clinical trial (2%)
- D. Cohort (5%)
- E. Cross-sectional (16%)

Omitted
Correct answer
B

70%
Answered correctly

02 secs
Time Spent

2023
Version

Explanation

Research studies are broadly classified as having an observational design or an experimental design depending on the control the researchers have over the independent variables.

- In **observational studies** (eg, case series, case-control studies, cohort studies, cross-sectional studies), the researchers have **no control** over the independent variables (eg, exposure to risk factors, treatments).
- In experimental studies (eg, crossover design, randomized controlled trials), the researchers control and **randomly assign the independent variables** (eg, exposure to risk factors, treatments).

The screenshot shows a mobile application interface for a medical exam question. At the top, there's a header with the URL "apps.uworld.com". Below the header is a toolbar with various icons: a left arrow, a right arrow, a double arrow, a magnifying glass, a question mark, a calculator, a reverse color button, a text zoom button, and a settings gear icon. On the far left, there's a vertical menu icon and the text "Item 15 of 40" and "Question Id: 19247". To the right of the toolbar are buttons for "Previous" and "Next", "Full Screen", "Tutorial", "Lab Values", "Notes", and "Calculator".

- In experimental studies (eg, crossover design, randomized controlled trials), the researchers control and randomly assign the independent variables (eg, exposure to risk factors, treatments).

Case series is a descriptive observational study design in which a (generally small) group of patients with a similar diagnosis or treatment is described at a point in time or followed over a certain time period. Contrary to other observational designs (eg, cohort, case-control), a case series has **no comparison group (Choices A and D)**. For this reason, a case series cannot establish associations between risk factors (eg, treatments) and outcomes (eg, diseases).

In this example, the CT scan findings of 11 patients with coronavirus disease 2019 (COVID-19) are being studied. The study has no control group; therefore, it can only describe COVID-19 manifestations on imaging studies.

(Choice C) A clinical trial is an experimental study in which patients are prospectively assigned to ≥2 interventions (often including a placebo or control treatment) to evaluate the effects of those interventions on outcomes of interest.

(Choice E) A cross-sectional study is an observational study that assesses a population of interest (eg, all women of reproductive age) at a single point in time; it can be used to estimate disease prevalence and association with risk factors of interest. However, it is not used to characterize disease findings because subjects are not selected based on the presence of disease.

Educational objective:

A case series is a descriptive observational study design in which a group of patients with a similar diagnosis or treatment is described at a point in time or followed over a certain period. This study design has no comparison group; therefore, it cannot establish associations between risk factors (eg, treatments) and outcomes (eg, diseases).

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Item 16 of 40 Question Id: 1275

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A hospital wants to estimate the prevalence of diabetic nephropathy in the surrounding population of adults with type 2 diabetes. Kidney biopsy samples are obtained from 500 adult patients with diabetes who receive care at the hospital. The samples are then interpreted by 10 different pathologists, 5 of whom work at the hospital and 5 of whom work at nearby institutions. A preliminary analysis shows that the pathologists who work for the hospital are 3 times more likely to interpret the biopsy samples as diabetic nephropathy compared to those who do not work for the hospital. Which of the following most likely explains this difference in interpretation?

- A. Confounding (6%)
- B. Lead-time bias (0%)
- C. Observer bias (78%)
- D. Recall bias (3%)
- E. Selection bias (11%)

Omitted
Correct answer
C

78%
Answered correctly

02 secs
Time Spent

2023
Version

Explanation

Observer bias (also known as expectancy bias) occurs when investigators **misclassify data** due to preconceived expectations or **prior knowledge** concerning the study or its participants. This type of bias is particularly important when outcomes are **subjective** (ie, involve personal interpretation of clinical, microscopic,

Item 16 of 40
Question Id: 1275

Mark

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Full Screen Tutorial Lab Values Notes Calculator Reverse Color Text Zoom Settings

Observer bias (also known as expectancy bias) occurs when investigators **misclassify data** due to preconceived expectations or **prior knowledge** concerning the study or its participants. This type of bias is particularly important when outcomes are **subjective** (ie, involve personal interpretation of clinical, microscopic, or radiographic findings).

In this case, the pathologists who work at the hospital where the study is being conducted may know that the study is investigating diabetic nephropathy, or they may have access to additional medical records that indicate diabetes status. Conversely, the pathologists at other institutions are more likely to be blinded to the study's objectives and the patients' medical history.

(Choice A) A confounder is an extraneous variable that is related to the exposure under investigation but that is also an independent risk factor for a disease. Confounding distorts the relationship between the exposure and the disease. For example, fatty food intake is a potential confounder in a study evaluating the association between physical activity and obesity, as people who do not exercise regularly may be more likely to consume a high-fat diet.

(Choice B) **Lead-time bias** refers to the apparent prolongation of survival in patients who undergo a screening test that allows for earlier diagnosis but does not actually improve prognosis. For example, a patient with pancreatic cancer presents with metastases at age 58 for which he receives treatment, but he dies at age 60. Had the same patient been screened and diagnosed with pancreatic cancer at age 55, he would have received earlier treatment, but the disease could have progressed such that he would have still died at age 60 (ie, age at death would not have been affected by screening).

(Choice D) Recall bias results from inaccurate recall of past exposure by people in a study; it applies most often to retrospective designs such as case-control studies. People who have experienced an adverse outcome are more likely to recall previous risk factors than people who have not experienced an adverse outcome.

The screenshot shows a mobile application interface for a medical study. At the top, there's a header bar with a back arrow, a double arrow, and a refresh icon. The URL 'apps.uworld.com' is displayed in the address bar. Below the header is a toolbar with various icons: a menu icon, 'Item 16 of 40', 'Question Id: 1275', a 'Mark' icon, 'Previous' and 'Next' arrows, 'Full Screen', 'Tutorial', 'Lab Values', 'Notes', 'Calculator', 'Reverse Color', 'Text Zoom', and 'Settings'. The main content area contains text about the relationship between physical activity and obesity, followed by three choice options (B, D, E) describing different types of bias.

between physical activity and obesity, as people who do not exercise regularly may be more likely to consume a high-fat diet.

(Choice B) Lead-time bias refers to the apparent prolongation of survival in patients who undergo a screening test that allows for earlier diagnosis but does not actually improve prognosis. For example, a patient with pancreatic cancer presents with metastases at age 58 for which he receives treatment, but he dies at age 60. Had the same patient been screened and diagnosed with pancreatic cancer at age 55, he would have received earlier treatment, but the disease could have progressed such that he would have still died at age 60 (ie, age at death would not have been affected by screening).

(Choice D) Recall bias results from inaccurate recall of past exposure by people in a study; it applies most often to retrospective designs such as case-control studies. People who have experienced an adverse outcome are more likely to recall previous risk factors than people who have not experienced an adverse outcome.

(Choice E) Selection bias can occur with inappropriate (ie, nonrandom) selection methods or through selective attrition of the study participants. For example, many patients drop from a clinical trial due to the severity of side effects associated with the treatment. This type of attrition is selective (ie, different attrition rates between groups) and reduces the generalizability of the study.

Educational objective:

Observer bias occurs when the investigator's evaluation is affected by preconceived expectations or prior knowledge, typically leading to overestimation of the disease association or treatment effects. This type of bias can be reduced by conducting a blinded study in which observers are unaware of study details and patient characteristics that could unduly influence them.

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Item 17 of 40 Question Id: 1276

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Researchers are studying the relationship between essential hypertension and a common mutation in the structure of a sodium channel protein. A study population is randomly selected and blood samples are obtained for leukocyte genotyping. The prevalence of hypertension is determined based on mean blood pressure measurements obtained using standardized ambulatory blood pressure monitoring conducted over 1 week. Based on the analysis results, the researchers conclude that the sodium channel structure mutation is associated with hypertension. Which of the following best describes the study design used by the investigators?

- A. Case-control study (10%)
- B. Cross-sectional study (56%)
- C. Prospective cohort study (17%)
- D. Randomized clinical trial (9%)
- E. Retrospective cohort study (6%)

Omitted
Correct answer
B

56%
Answered correctly

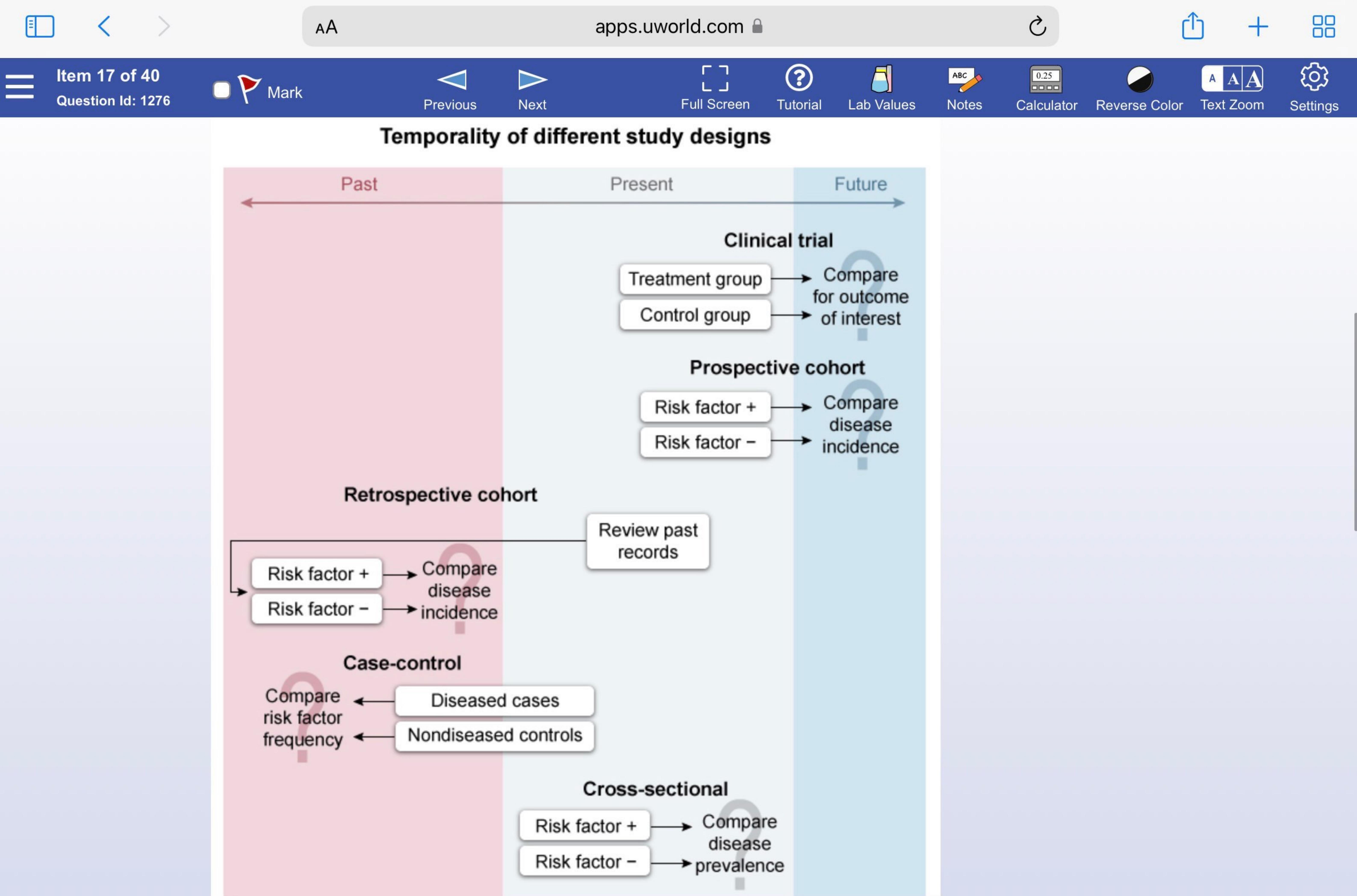
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Time Spent

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Explanation

Temporality of different study designs





Item 17 of 40 Question Id: 1276

Mark

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A **cross-sectional** study (also known as a prevalence study) simultaneously measures exposures and outcomes. The cross-sectional study has a "**snapshot**" design that is frequently used in surveys, mostly because it is inexpensive and easy to perform. In this example, a snapshot was obtained of individuals randomly selected from the population; their blood samples were analyzed for the presence of the sodium channel protein mutation and the prevalence of hypertension was calculated. The subjects' blood pressure was measured over 7 days to obtain an average measurement (likely to limit the results being impacted by white-coat hypertension and other transient causes of hypertension). The major limitation of a cross-sectional study design is that a temporal relationship between exposure and outcome is not always clear. However, in this case, demonstrating a temporal relationship was straightforward because the possession of a specific genotype clearly precedes hypertension.

(Choice A) A case-control study is designed by selecting individuals with a particular disease (cases), individuals without that disease (controls), and then evaluating previous exposure status. This study would have been classified as a case-control study if it had explicitly recruited individuals with (cases) and without (controls) hypertension (rather than randomly selecting a sample from a population) and evaluated their sodium channel mutation patterns.

(Choices C and E) A prospective cohort study would have taken individuals without hypertension from the population, analyzed their blood samples to determine the distribution of the sodium channel mutation, and followed them over time (years) to determine the proportion of new cases of hypertension in patients with and without the mutation. In this example, although hypertension was measured over several days, the objective was not to document incidence of new cases of hypertension (which would be unlikely to develop over 1 week) but to measure the prevalence of hypertension while ensuring that average blood pressure measurements were obtained.

The top navigation bar includes:
- A back arrow, forward arrow, and a double arrow icon.
- A font size icon 'AA'.
- The URL 'apps.uworld.com' in the center.
- A refresh/circular arrow icon.
- A blue square icon with a white upward arrow.
- A blue plus sign icon.
- A blue square icon with four smaller squares inside.
Below the navigation bar, the main content area has a blue header with the following items:
- 'Item 17 of 40'
- 'Question Id: 1276'
- 'Mark' icon (a red flag)
- 'Previous' and 'Next' navigation icons.
- 'Full Screen' icon.
- 'Tutorial' icon.
- 'Lab Values' icon.
- 'Notes' icon.
- 'Calculator' icon.
- 'Reverse Color' icon.
- 'Text Zoom' icon.
- 'Settings' icon.

(Choices C and E) A prospective cohort study would have taken individuals without hypertension from the population, analyzed their blood samples to determine the distribution of the sodium channel mutation, and followed them over time (years) to determine the proportion of new cases of hypertension in patients with and without the mutation. In this example, although hypertension was measured over several days, the objective was not to document incidence of new cases of hypertension (which would be unlikely to develop over 1 week) but to measure the prevalence of hypertension while ensuring that average blood pressure measurements were obtained.

A retrospective design would have also assessed incidence (not prevalence) of hypertension compared to an earlier period of time, based on a chart review of historic data. Prospective and retrospective cohort studies are organized by selecting a group of individuals (ie, a cohort) who do **not** have the disease of interest (eg, hypertension), determining their exposure status, and then following them (forward in time or from a point in the past to the present) to assess for the development of the disease.

(Choice D) A randomized clinical trial directly compares ≥ 2 treatments. Usually, the subjects are randomly assigned to experience a specific exposure (eg, a medication) or no exposure (eg, placebo) and are then followed to assess for the outcome of interest (eg, disease).

Educational objective:

In a cross-sectional study, exposure and outcome are measured simultaneously at a particular point in time ("snapshot study"). In other study designs, a certain time period separates the exposure from the outcome.

Biostatistics

Biostatistics & Epidemiology

Subject

System

Study designs

Topic

apps.uworld.com

Item 18 of 40 Question Id: 19406

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An investigator is conducting a randomized, double-blind, placebo-controlled clinical trial of a new drug for the treatment of peripheral neuropathy in adults with multiple myeloma (MM). One hundred fifty patients with MM are enrolled in the trial and randomized to receive either the new drug ($n = 75$) or placebo ($n = 75$). Trial protocol requires that patients in both groups take 1 pill per day and keep a pain diary. After 3 months of treatment, each patient is interviewed, and the pain diaries are reviewed; 9 patients taking the new drug and 3 patients taking placebo did not take the pills as instructed. The investigator decides to conduct an intention-to-treat analysis of the study data. Which of the following best describes how the data pertaining to all patients who did not adhere to protocol should be treated?

- A. Conduct separate analyses of the 12 nonadherent patients and the 138 adherent patients (12%)
- B. Exclude all 12 nonadherent patients from analysis (28%)
- C. Exclude the 3 nonadherent patients in the group taking placebo from analysis (1%)
- D. Exclude the 9 nonadherent patients in the group taking the new drug from analysis (3%)
- E. Keep all 12 nonadherent patients in their respective groups for analysis (55%)

Omitted
Correct answer
E

55%
Answered correctly

02 secs
Time Spent

2023
Version

Explanation

An **intention-to-treat** (ITT) analysis compares treatment groups in a randomized trial by **including all subjects**

An **intention-to-treat** (ITT) analysis compares treatment groups in a randomized trial by **including all subjects** as initially allocated after randomization **regardless of what happens** during the study period. The rationale is that if subjects have such a poor treatment response (or are so inconvenienced by treatment administration or side effects) as to not follow protocol specifications or drop out of the study, then their outcomes should be attributed to that intervention (ie, nonadherence is related to the practical benefits of treatment). ITT analysis also helps avoid the effects of attrition (eg, loss to follow-up, dropout) and crossover (eg, switching to another assigned intervention), which may disrupt the benefit of randomization and introduce bias in the estimation of the effect of the intervention.

ITT analysis may lead to a more **conservative estimate** of the effect of the intervention. If attrition or crossover is significant, ITT analysis may be less likely to identify a statistically significant difference between treatments (ie, shift toward the null hypothesis). However, results will reflect the **expected effect** of the intervention in a **practical clinical setting**.

(Choices A, C, and D) In accordance with ITT analysis, the investigator in this case should keep the 12 nonadherent patients in their respective groups for analysis.

(Choice B) An analysis that includes only those patients who strictly adhered to a research protocol (ie, excluding all nonadherent patients from analysis) follows a per-protocol (PP) principle. In PP analysis, the benefit of randomization can be lost with significant subject attrition (eg, sicker subjects selectively dropping out, introducing bias). PP analysis can also overestimate the effect of the intervention in a practical clinical setting, particularly when the treatment is somehow aversive to patients.

Educational objective:

Intention-to-treat analysis includes each subject in their initial randomization group even if subjects stop the intervention or shift to a different intervention. This approach tends to provide a conservative but more valid

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Question Id: 19406

Mark

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also helps avoid the effects of attrition (eg, loss to follow-up, dropout) and crossover (eg, switching to another assigned intervention), which may disrupt the benefit of randomization and introduce bias in the estimation of the effect of the intervention.

ITT analysis may lead to a more **conservative estimate** of the effect of the intervention. If attrition or crossover is significant, ITT analysis may be less likely to identify a statistically significant difference between treatments (ie, shift toward the null hypothesis). However, results will reflect the **expected effect** of the intervention in a **practical clinical setting**.

(Choices A, C, and D) In accordance with ITT analysis, the investigator in this case should keep the 12 nonadherent patients in their respective groups for analysis.

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Educational objective:

Intention-to-treat analysis includes each subject in their initial randomization group even if subjects stop the intervention or shift to a different intervention. This approach tends to provide a conservative but more valid estimate of the intervention effect in real-world scenarios (ie, clinical settings).

Biostatistics
Subject

Biostatistics & Epidemiology
System

Randomized control trials
Topic

apps.uworld.com

Item 19 of 40 Question Id: 1279

Mark Previous Next Full Screen Tutorial Lab Values Notes Calculator Reverse Color Text Zoom Settings

A new estrogen receptor agonist is being evaluated for the treatment of postmenopausal symptoms. A prospective study shows that the drug increases the risk of deep vein thrombosis (DVT) in treated women who smoke compared to untreated women who smoke, with a relative risk (RR) of 1.70 and p-value of 0.01. In nonsmokers, no increased risk of DVT is evident with use of the drug (RR = 0.96; p-value = 0.68). Which of the following describes this phenomenon?

- A. Confounding (48%)
- B. Effect modification (40%)
- C. Latent period (2%)
- D. Observer bias (2%)
- E. Selection bias (6%)

Omitted

Correct answer

B



40%

Answered correctly



01 sec

Time Spent



2023

Version

Explanation

Effect modification occurs when the effect of an exposure on an outcome is modified by another variable. It can be identified using **stratified analysis** (analyzing the cohort as different subgroups), as the different strata will have **different measures of association**. In this scenario, smoking status modified the effect of the new estrogen receptor agonist (exposure) on deep vein thrombosis (DVT) incidence (outcome). Using stratified

The screenshot shows a mobile application interface for a medical question. At the top, there are navigation icons: back, forward, and a double arrow. The title bar displays "apps.uworld.com". Below the title bar, the header includes: "Item 19 of 40", "Question Id: 1279", "Mark" (with a red flag icon), "Previous" and "Next" buttons, "Full Screen", "Tutorial", "Lab Values", "Notes", "Calculator", "Reverse Color", "Text Zoom" (with a magnifying glass icon), and "Settings" (with a gear icon). The main content area contains the following text:

analysis by smoking status:

- Among smokers, there was a statistically significant association between taking the new estrogen receptor agonist and risk of developing DVT with a relative risk of >1 , indicating higher risk, and a p-value of <0.05 , indicating statistical significance.
- In contrast, among nonsmokers, there was no statistically significant association between taking the medication and risk of DVT (p-value >0.05).

Effect modification is not a bias (**Choices D and E**), as it is not due to flaws in the design or analysis phases of the study. It is a natural phenomenon that should be described, not corrected.

Effect modification is most easily confused with **confounding (Choice A)**, but stratified analysis can help distinguish between these 2 scenarios. With effect modification, the different strata will have different measures of association, as seen in this example of the association between taking the estrogen receptor and the risk of DVT among smokers compared to nonsmokers. In contrast, with confounding, stratification usually reveals no significant difference between the strata. [For instance](#), in an analysis of primary school students (of all grade levels), age can be a confounder that muddies the association between shoe size and intelligence. Children with bigger shoe sizes may appear to be more intelligent on initial analysis. However, this association is likely not due to shoe size but rather to age because older children tend to have both bigger feet and more intelligence. When older and younger children are analyzed separately (stratification based on the confounder), the association between shoe size and intelligence disappears.

(Choice C) The latent period is the time required for an exposure to begin having an effect. However, there is no information on how latency was handled in this study.

Educational objective:

Effect modification is present when the effect of the main exposure on the outcome is modified by the presence

medication and risk of DVT (p -value >0.05).

Effect modification is not a bias (**Choices D and E**), as it is not due to flaws in the design or analysis phases of the study. It is a natural phenomenon that should be described, not corrected.

Effect modification is most easily confused with **confounding (Choice A)**, but stratified analysis can help distinguish between these 2 scenarios. With effect modification, the different strata will have different measures of association, as seen in this example of the association between taking the estrogen receptor and the risk of DVT among smokers compared to nonsmokers. In contrast, with confounding, stratification usually reveals no significant difference between the strata. [For instance](#), in an analysis of primary school students (of all grade levels), age can be a confounder that muddies the association between shoe size and intelligence. Children with bigger shoe sizes may appear to be more intelligent on initial analysis. However, this association is likely not due to shoe size but rather to age because older children tend to have both bigger feet and more intelligence. When older and younger children are analyzed separately (stratification based on the confounder), the association between shoe size and intelligence disappears.

(Choice C) The latent period is the time required for an exposure to begin having an effect. However, there is no information on how latency was handled in this study.

Educational objective:

Effect modification is present when the effect of the main exposure on the outcome is modified by the presence of another variable. Effect modification is not a bias.

Biostatistics

Subject

Biostatistics & Epidemiology

System

Confounding, effect modification, bias, errors

Topic

apps.uworld.com

Item 20 of 40 Question Id: 1174

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A physician research group is evaluating the efficacy of a new lipid-lowering drug, Superstatin, which is being marketed directly to consumers as a groundbreaking new therapy to prevent heart attacks. The drug manufacturer claims that the drug is more effective than existing hypolipidemic agents for primary prevention of myocardial infarction. Results of a 5-year, randomized, double-blinded, controlled study to evaluate the efficacy of Superstatin are shown below.

	Number of patients treated with Superstatin	Number of patients treated with control medication
Myocardial infarction	10	25
No myocardial infarction	990	975

Compared to the control medication, how many patients need to be treated with Superstatin to prevent one additional myocardial infarction?

- A. 2 (7%)
- B. 5 (11%)
- C. 23 (5%)
- D. 48 (4%)
- E. 67 (60%)
- F. 92 (2%)
- G. 100 (7%)

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Item 20 of 40 Question Id: 1174

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Common measures of therapeutic efficacy		
Term	Definition	Calculation
Absolute risk reduction (ARR)	Percentage indicating the actual difference in event rate between control & treatment groups	$ARR = \text{control rate} - \text{treatment rate}$
Relative risk reduction (RRR)	Percentage indicating relative reduction in the treatment event rate compared to the control group	$RRR = ARR / \text{control rate}$
Relative risk (RR)	Ratio of the probability of an event occurring in the treatment group compared to the control group	$RR = \text{treatment rate} / \text{control rate}$
Number needed to treat (NNT)	Number of individuals who need to be treated to prevent a negative outcome in 1 patient	$NNT = 1 / ARR$

The **number needed to treat (NNT)** represents the number of patients that need to be treated with a medication (eg, Superstatin) in order to **prevent** an additional **negative outcome** (eg, myocardial infarction). NNT is calculated by dividing 1 by the **absolute risk reduction (ARR)**.

$$NNT = 1 / ARR$$

ARR represents the actual difference in control and experimental group event rates. The data is *not* presented in the standard format of a [contingency \(2 × 2\) table](#), so care should be exercised in selecting the appropriate