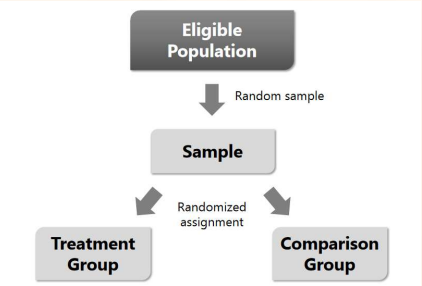
Evaluating Treatment Impact in Medicine

In medicine, the goal is to provide effective treatments to patients. To ensure that the treatments we administer are truly making a difference, we need to understand how to measure their effectiveness.

Two questions: (1) can I rely on the conclusions of this study? (internal validity), and (2) can I apply these conclusions to my patients? (external validity).

Γιάννης Κωστόπουλος PhD
yiannis.kostopoulos@gmail.com

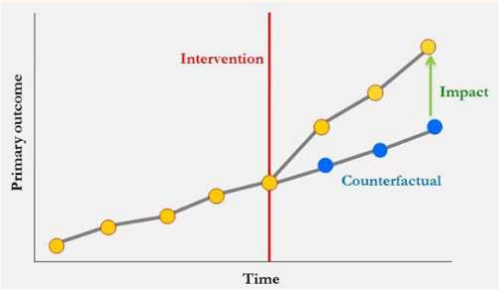
The Importance of Attribution in Medicine



As orthopedic practitioners, the aim is to help patients recover and regain their mobility and quality of life. But how can we be sure that the treatments we choose are responsible for their improvement and not just natural healing or other factors?

Attribution is key in medicine. It involves determining whether the treatment we administer is the primary reason for the patient's improvement. This is crucial to ensure that the treatments we provide are effective and valuable.

The Counterfactual Question



The counterfactual question is fundamental in assessing the true impact of any medical intervention. It asks us to consider what would have happened to the patient if they had not received the treatment.

For example, let's say we're treating a patient. We need to determine whether the surgery we performed was responsible for their successful recovery or if other factors, such as their overall health or natural healing processes, played a significant role.

Challenges in Measuring Treatment Impact

Scenario 1: Comparing Pre- and Post-Treatment

One approach is to compare the patient's condition before and after the surgery. However, improvements may be due to various factors, such as the body's healing capabilities or general health. So, we cannot attribute the entire recovery to the surgical procedure.

Scenario 2: Comparing Treated and Untreated Patients

Another option is to compare the recovery of patients who received the surgery with those who did not. However, differences in outcomes may not be solely due to the surgery but could be influenced by variations in health condition or stage of the condition, introducing selection bias.

Example: Counterfactual Impact Evaluation

Step 1: Identifying the Treated Group

The treated group consists of patients who underwent a new surgical technique.

Step 2: Defining the Control Group

The control group is made up of patients who did not receive the new surgical technique but closely resemble the treated group in terms of key characteristics like age, gender, and overall health.

Step 3: Outcome Assessment

Both groups are monitored and assessed over time, tracking factors like pain levels, range of motion, and quality of life.

Step 4: Comparing Outcomes

By comparing the outcomes of the treated and control groups, we can determine the true impact of the intervention. Any significant differences can be attributed to the surgical technique.

Addressing Selection Bias

To ensure the reliability of treatment impact assessment, researchers often employ methods that mimic randomization, as conducting randomized controlled trials (RCTs) in medicine can be ethically and logistically challenging.

RCT example

Participants

Patients with diagnosed rotator cuff tears who are suitable candidates for surgical intervention.

Randomization

Eligible patients are randomly assigned to either the open repair group or the arthroscopic repair group. This ensures statistical similarity between the groups in terms of key factors.

Blinding

The study may be double-blind, meaning neither the patients nor the doctors and researchers know which patients are in which group, reducing bias.

Interventions

Open repair involves traditional surgery, while arthroscopic repair is a minimally invasive procedure.

Outcome Measures

Assessments include pain levels, shoulder function, and range of motion at various time points after the surgery.

Data Collection and Analysis

Data on outcomes are collected and analyzed to determine the effectiveness of each surgical technique.

RCT challenges

RCTs are the gold standard for evaluating treatment impact, but they come with difficulties:

1. Ethical Considerations: Randomly assigning patients to different treatments may not be acceptable when both procedures have risks and potential benefits.
2. Informed Consent: Obtaining informed consent can be challenging when patients may not fully understand the nuances of different surgical techniques.
3. Sample Size: Recruiting a sufficient number of patients for an RCT can be challenging, especially in limited patient populations.
4. Long-Term Follow-up: Assessing long-term effects requires resources, funding, and patient cooperation.
5. Practitioner Expertise: Variations in surgical skill and technique can introduce confounding factors.
6. Placebo and Blinding: Providing a placebo surgical procedure is difficult, making blinding challenging.

Non-Random Assignment

Strategies to Address Selection Bias

In cases where random assignment is not feasible, non-random assignment is used. This presents challenges due to potential selection bias.

Propensity score matching can be employed to minimize selection bias:

1. Study Design: Collect information on patient characteristics that may influence treatment choice and outcomes.
2. Propensity Score Matching: Calculate the probability of receiving each treatment based on patient characteristics and match patients with similar propensity scores.
3. Interventions: Patients receive either open repair or arthroscopic repair.