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Efficacy calculation in clinical trial

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Abstract

Efficacy is the response of a drug or intervention in an ideal and control condition against the disease or condition. It is very important for medical and public health professionals to understand the efficacy and the process of calculating it especially when they involve in drug trials, clinical practice, and epidemiological research. This paper briefly discusses the term efficacy and the epidemiological process of its calculation.

Key words
Attributive risk percentage, clinical trial, efficacy, epidemiology,

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Background

Population Efficacy and Effectiveness are very popular terms in epidemiology. The significance of these terms are the highest in clinical trial. First two phases (I & II) in clinical trials completely revolves around efficacy and effectiveness 1. Efficacy is a biological effect of a drug or intervention in an ideal and controlled condition. Efficacy assesses in second phase of the clinical trial, which comes after the first phase called Maximally Tolerated Dose (MTD) setting [1, 2]. The effectiveness deals with the response rate of the drug or intervention in real condition and assessed in phase III. Evaluation of efficacy is carried out on a small number of patients. These participants are recruited after a strong inclusion and exclusion selection criteria. Participants are also controlled for any other intervention or cofounders [1, 3, 4].

Every year, several clinical trials are conducted across the world and the number of such studies are growing [5]. Presently, 192, 367 clinical trials are registered in National Institute of Health in all 50 states of the United States and 185 countries [5]. Professionals involved in drug trial, disease management and prevention need to understand efficacy for the betterment of health care and biomedical research. In general, medical professionals who deal with drug trials and management have less familiarity and academic inputs on efficacy, and research design in the world [6]. Compared to developed nations, low and middle-income (LAMI) countries are more disadvantaged in this matter as they lack academic knowledge in area of epidemiology, biostatistics and research [7, 8]. This topic is highly relevant for LAMI countries, because now many clinical trials are multi-centered and conducted internationally [9]. Since, population in LAMI countries is readily available for recruitment, and available infrastructure is cheaper than the developed nation, therefore, more clinical trials are projected to be conducted in developing countries in the future [10 - 12].

Efficacy is calculated in same manner as Attributive Risk Percentage (ARP). So efficacy can also be known as ARP. ARP and efficacy do not carry equal weight and meaning in research because their application is not same. ARP computed in cohort and sometime in cross-sectional studies while efficacy in randomized controlled placebo trials. Efficacy measures the highest level of biological effect of the drug against the disease or the condition [13, 14]. Calculation of efficacy can be learned from following epidemiological terms and procedures [15]. We are following clinical trial prototype about a hypothetical drug "A" to treat aggressive behavior in intellectual disability.

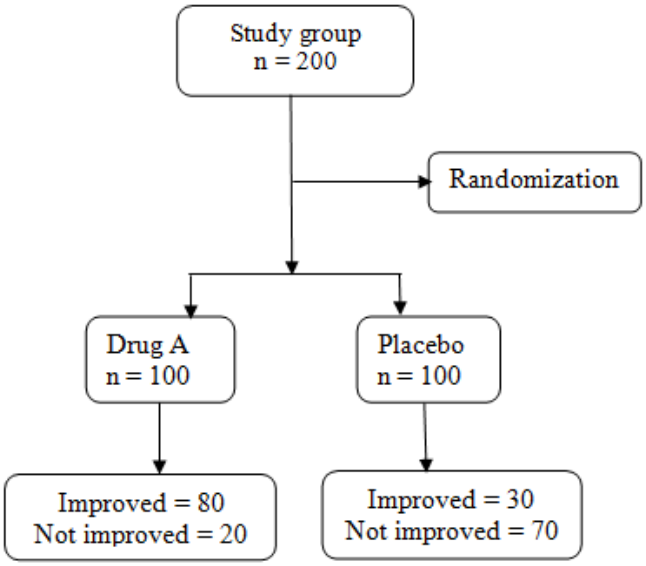

Figure 1- Clinical trial diagram

Table - 1 Contingency table drug vs. placebo

Outcome / Treatment		Improved		Not improved
Drug "A"	*a*	80	*b*	20
Placebo	*c*	30	*d*	70

Step 1: calculate cumulative incidence of exposed and non-exposed group

Once numbers are entered in contingency table then Cumulative Incidence (CI) for drug A and placebo can be computed with following formulas

$$\text{Cumulative Incidence (Drug A)} = \frac{\text{total number improved}}{\text{total number recieved drug A}} \quad \text{or} \quad CI = \frac{a}{a+b}$$

$$\text{Cumulative Incidence (Drug A)} = \frac{80}{100} = 0.8$$

$$\text{Cumulative Incidence (Placebo)} = \frac{\text{total number improved}}{\text{total number recieved placebo}} \quad \text{or} \quad CI = \frac{c}{c+d}$$

$$\text{Cumulative Incidence (Placebo)} = \frac{30}{100} = 0.3$$

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Step 2: calculate attributive risk

Attributive risk is difference measure of two cumulative incidences. It is calculated by subtractive CI of non-exposed group from CI of exposed group.

Attributive Risk (AR) =

Cumulative Incidence $_{\text{exposed}}$ - Cumulative Incidence $_{\text{non-exposed}}$

Attributive Risk = 0.8 – 0.3 = 0.5

Step 3: calculate Efficacy

$$\text{Efficacy} = \frac{\text{Attributive risk}}{\text{Cumulative Incidence of exposed group}} \text{ X } 100$$

$$\text{Efficacy} = \frac{0.5}{0.8} \text{ X } 100$$

$$= 62.5$$

The efficacy of drug "A" is 62.5% in the group. This translates that drug "A" is effective in treating 62.5% of aggressive behaviors in people with intellectual disability. Further, the confidence interval can be calculated using statistical methods. The efficacy should be reported as per the guidelines of journal [16, 17].

Abbreviations

Attributive Risk Percentage (ARP), low and middle-income (LAMI), Maximally Tolerated Dose (MTD).

Competing interests

None

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