



COMMENTARY

Efficacy, Effectiveness and Efficiency in the Health Care: The Need for an Agreement to Clarify its Meaning

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Abstract

There is great interest in ensuring that health system resources are used effectively. Dictionary says *efficacy*, *effectiveness* and *efficiency* are synonymous. Curiously, it is in many scientific fields where there has been imposed an artificial interpretation of these terms. *Efficacy*, in the health care sector, is the capacity of a given intervention under ideal or controlled conditions. *Effectiveness* is the ability of an intervention to have a meaningful effect on patients in normal clinical conditions. *Efficiency* is doing things in the most economical way. Therefore, it would be more correct to define as *strategic efficacy/effectiveness* to the aptitude to produce an effect realized in ideal conditions and *tactical efficacy/effectiveness* to the same effect looked in ordinary circumstances. *Efficiency* would be kept in its current definition.

Keywords

Efficacy, Effectiveness, Efficiency, Strategy, Tactics, Health care

Framing the Problem

Around the world, every health care system is struggling with rising costs and, the lack of economic sustainability of most healthcare systems has contributed to the development of regulation in the health sector. It is even more important that public resources are used in the most efficient and effective way [1,2]. In order to achieve these objectives, there must be an agreement in the used terminology. In the real life, we use the terms efficacy and effectiveness interchangeably and the words efficiency and effectiveness are often considered synonyms. Curiously, it is in many scientific fields where there has been

imposed an artificial interpretation of these terms. Dictionary says efficacy, effectiveness and efficiency are synonymous, and they share many of the same characteristics, making them difficult to differentiate. Oxford Dictionary of English (3 ed) define the mentioned terms: Efficiency is the state or quality of being efficient and it can be used how the ratio of the useful work performed by a machine or in a process to the total energy expended or heat taken in.; Effectiveness is the degree to which something is successful in produced a desired result and Efficacy is the ability to produce a desired or intended result. As we see, terms very seemed in its meaning [3]. The real question is whether employ two words with the same meaning (efficacy and effectiveness) to explain different concepts. The purpose of this work is to clearly delineate that there are not differences in meaning between efficacy and effectiveness. Moreover, we propose new terms that explain and show with a logical form the distinct differences, in the health care systems, between procedures or studies under ideal conditions and studies called pragmatic or real world.

Effectiveness, Efficacy and Efficiency in the Management

Efficiency, effectiveness and efficacy, in formal management discussions, take on very different meanings and were originally industrial engineering concepts that came of age in the early twentieth century. Peter Drucker, an expert of the management, in his book “the effective executive” [4] developed these concepts.



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Effectiveness is doing “the right” things, for example setting right targets to achieve an overall goal (the effect). It is the extent to which planned outcomes, goals, or objectives are achieved as a result of an activity, intervention or initiative intended to achieve the desired effect, under ordinary circumstances (not controlled circumstances such as in a laboratory).

Efficacy is getting things done. It is the ability to produce a desired amount of the desired effect, or success in achieving a given goal.

Efficiency is doing things in the most economical way. It is the ratio of the output to the inputs of any system (good input to output ratio).

Effectiveness, Efficacy and Efficiency in the Health Care Sector

These economic concepts were incorporated in the health care sector. Distinction between effectiveness, efficacy and efficiency is due to Archie Cochrane in his book “Effectiveness and efficiency: Random reflections on health services” (1972) [5]. Since then, it is admitted the followings terms:

Efficacy, in the health care sector, is the capacity for beneficial change (or therapeutic effect) of a given intervention (for example a drug, medical device, surgical procedure or a public health intervention) under ideal or controlled conditions.

Effectiveness links to the notion of external validity, in that it refers to patients who are visited by physicians in their everyday practice.

Real or Not Real Conditions in Trials

Therefore, observational studies and randomized controlled trials are the main types of studies used to evaluate treatments. In the last ones, patients are assigned to active or control group by through randomization. Nowadays is assimilated efficacy with randomized controlled trials and effectiveness with observational studies [6]. Guidelines are mostly based on evidence gathered from randomized controlled trials [6-8]. Currently, effectiveness can be defined as the extent to which a drug achieves its intended effect in the usual clinical setting. It can be evaluated through observational studies of real practice. In real practice studies (“how the drug works in a real-world situation”) there are interactions with other medications and interactions with health conditions of the patient. A treatment is effective if it works in real life in non-ideal circumstances [7,8]. Effectiveness cannot be measured in controlled trials, because the act of inclusion into a study is a distortion of usual practice [9,10]. On the contrary, observational studies (usually called pragmatic trials, real-world trials, naturalistic trials) do not require randomization.

Nevertheless, nowadays we cannot obviate the con-

cept evidence-based medicine. It was initially developed by Guyatt, et al. [11]. Evidence-based medicine is the conscientious, explicit and reasonable use of best evidence and making decisions about the case of individual patients. Evidence-based medicine integrates clinical experience with the best available research information [12]. It categorizes different types of clinical evidence and ranks them according to the strength of their freedom from the various biases that beset medical research. One of the scales most used is the Scottish Intercollegiate Guidelines Network (SIGN) which use a code together the study type for decide the level of evidence [13]. High level of strength of recommendation is assigned to randomized control trials with a very low risk of bias and low levels of recommendation correspond to observational studies. Hence, the called “pragmatic studies” or “real-life studies” might be qualified as low evidence. This would suppose a contradiction, the most effectiveness studies would be the lowest level of evidence.

Efficiency as a Consolidated Concept in the Health Care Sector

Efficiency is the ratio of the output to the inputs of any system. An efficient system or person is one who achieves higher levels of performance (outcome, output) relative to the inputs (resources, time, money) consumed [14]. Historically, efficiency measurements come from engineering science where performance had to be measured. The result has been typically displayed as physical units per resource used. Achieving efficiency, which is defined as maximizing the outputs achieved per unit of input invested [14] is naturally of great interest to national governments, international donors and other stakeholders in the health sector. As what type of product is being evaluated, we distinguish between two types of outputs: Health services (visits, drugs, admissions) and health outcomes (by way of example: Preventable deaths, functional status, clinical outcomes such as blood pressure or blood sugar control).

Efficiency measures must also explicitly identify the inputs that are used (or will be counted) to produce the output of interest. Inputs can be measured as counts by type (by way of example, nursing hours, bed days, days supply of drugs) or they can be monetized (real or standardized dollars assigned to each unit). We refer to these, respectively, as physical inputs or financial inputs [14,15].

A New Proposal to Clarify

As can be seen, there have developed a series of artificial meanings on the use of efficacy, effectiveness and efficiency that impede the comprehension of the topic. Especially, in the field of the medicine there has spread the use of these words with a meaning different from that of the real language. Hereby, there is understood that effectiveness is an inter-

vention or initiative intended to achieve the desired effect, under ordinary circumstances (not controlled circumstances such as in a laboratory). Similarly, efficacy is an objective achieved under controlled circumstances. As a consequence, this artificial use does (that do not correspond to an intuitive meaning) that we should know before the previously arbitrary definitions to be able to understand medical topic about which it treats. We are forced to know before these artificial meanings to understand the subject which is discussed. In addition, the artful meaning that gives to the term's efficacy and effectiveness, corresponds to other perfectly catalogued terms. That is, words we are looking for to denominated ideal circumstances or real circumstances are already invented: strategy and tactics [3]. The phrase "strategy and tactics" is military in origin. This way, the term strategy means a plan in ideal situation and not place in practices yet or, a plan of action designed to achieve a long-term or overall aim. Strategies are the ideas and broad approaches that support the goal, a detailed plan for achieving success in situations such as war, politics, business, industry or sport. Strategy defines, or outlines, the desired goals and why you should go about achieving them. The term tactics would be the putting in practice of the plan conceived by the strategy in ideal situation. Tactics are the specific action items, details and activities that must occur for the strategy to be successful. Tactics are the actions you take in implementing your strategy. These actions comprise what is to be done, in what order, using which tools and personnel. In summary, strategy is the what and why. Tactics is the how. Therefore, would be more correct to define as strategic efficacy/effectiveness to the aptitude to produce a looked effect realized in ideal conditions and tactical efficacy/effectiveness to the same effect looked in ordinary circumstances. Efficiency would be kept in its current definition as the ratio of the output to the inputs of any system. By means of these new proposed terms, we would avoid the use of slightly precise others as pragmatic or conducted in real conditions to refer to certain clinical tests in not so strict conditions.

Conflict of Interest

The authors declare no conflict of interests.

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