REVIEW ARTICLE
pISSN 0976 3325 | eISSN 2229 6816
Open Access Article
www.njcmindia.org

Efficacy, Effectiveness, and Efficiency

Prakash Patel[1]

[1]Assistant Professor, Department of Community Medicine, SMIMER, Surat, Gujarat

ABSTRACT

Efficacy, Effectiveness, and Efficiency are widely used term in health care management. Efficacy means getting things done (is it working?), effectiveness means doing the proper things (is it actually working well?), and efficiency means doing things right (is it working within the most economical way?). It's helpful to consider them during this particular order. First, confirm the answer can actually achieve the specified result, albeit that efficacy requires very specific conditions. Then, test your solution during a real-world environment. Finally, if the answer is effective, find out ways to form it more economical more efficient. This article describes the meaning and usage of these three terminologies in context of health care setup.

Keywords: Efficacy, Effectiveness, Efficiency, Health management

"Efficacy describes the technical relationship between the technology and its effects (whether it actually works), whereas effectiveness concerns the extent to which application of an efficacious technology brings about desired effects (changes in diagnoses, altered management plans, improvement in health)… Efficiency is an economic concept which relates efficacy and effectiveness to resource use. Assessment of efficiency is concerned with whether acceptable efficacy and effectiveness are achieved with the most prudent or optimal mix or resources."

– MacKenzie and Dixon (1995)[1]

To understand the health care management in the state or country, it is particularly important to understand three terminologies, i.e. Efficacy, Effectiveness, and Efficiency.

Around the world, every health care system is stressed with rising costs and, the shortage of economic sustainability of most healthcare systems has contributed to the event of regulation within the health sector. it's even more important that public resources are utilized in the foremost efficient and effective way.[2] so as to realize these objectives, there must be an agreement within the used terminology. Within the real world, we use the terms efficacy and effectiveness interchangeably and therefore the words efficiency and effectiveness are often considered synonyms. Curiously, it's in many scientific fields where there has been imposed a man-made interpretation of those terms. Dictionary says efficacy, effectiveness and efficiency are synonymous, and that they share many of an equivalent characteristic, making them difficult to differentiate. Oxford Dictionary of English (3 ed) define the mentioned terms: Efficiency is that the state or quality of being efficient and it are often used how the ratio of the useful work performed by a machine or during a process to the entire energy expended or heat taken in.; Effectiveness is that the degree to which something is successful in produced a desired result and Efficacy is that the ability to supply a desired or intended result. As we see, terms very seemed in its meaning.[3] the important question is whether or not employ two words with an equivalent meaning (efficacy and effectiveness) to elucidate different

How to cite this article: Patel P. Efficacy, Effectiveness, and Efficiency. Natl J Community Med 2021;12(2):33-39

Financial Support: None declared **Conflict of Interest:** None declared

Date of Submission: 14-02-2021; **Date of Acceptance**: 21-02-2021; **Date of Publication**: 28-02-2021

Correspondence: Dr. Prakash Patel (Email: drpbpatel@gmail.com)

concepts. The aim of this work is to obviously delineate that there aren't differences in meaning between efficacy and effectiveness. Moreover, we propose new terms that specify and show with a logical form the distinct differences, within the health care systems, between procedures or studies under ideal conditions and studies called pragmatic or world.

Three terms that are often encountered in the literature dealing with evaluation of health services are *efficacy, effectiveness*, and *efficiency*. Before we glance at what these terms specifically mean in epidemiology, let's take a glance at what the terms efficacy and effectiveness mean generally.

What does effectiveness mean?

Effectiveness is that the main noun sort of the adjective effective, which suggests "adequate to accomplish a purpose; producing the intended or expected result." (Another, less noun sort of effective is effectivity.)

So, if you're measuring something's effectiveness, you're watching how well it does whatever its alleged to do. If dish soap only kills a little amount of germs on dishes, for instance, it's poor effectiveness.

The adjective effective comes from the noun effect. An impact is "something that's produced by workplace or cause; result; consequence." The word effect is usually confused with the word affect, which is usually used as a verb to mean "to produce an impact or change."

If we administer the agent in a "real-life" situation, is it effective? For example, when the vaccine just referred to is tested in a community, many individuals may not come in to be vaccinated. Or, an oral medication may have such an undesirable taste that no one will take it (so that it will prove ineffective), despite the fact that under controlled conditions, when compliance was ensured, the drug was shown to be efficacious.

What does efficacy mean?

Efficacy is "capacity for producing a desired result or effect; effectiveness." The adjective form efficacious means "capable of getting the specified result or effect."

You may have encountered the word efficacy utilized in the term self-efficacy, which refers to a person's belief that they will accomplish what they began to try to. As an example, a student has high self-efficacy if they combat a challenging job, fresh out of school with little experience, convinced they'll succeed.

Efficacy and effectiveness are close enough in meaning that they're often used interchangeably generally contexts. However, effectiveness is usually more specifically utilized in the context of how well something accomplishes a task whereas efficacy conveys the extent to which something accomplishes its task in the least. If a dish soap doesn't kill any germs in the least, for instance, we might say it's not an efficacious germ-killer.

Does the agent or intervention "work" under ideal, "laboratory" conditions? We test a new drug in a group of patients who have agreed to be hospitalized and who are observed as they take their therapy. Or a vaccine is tested in a group of consenting subjects. Thus, efficacy is a measure in a situation in which all conditions are controlled to maximize the effect of the agent.

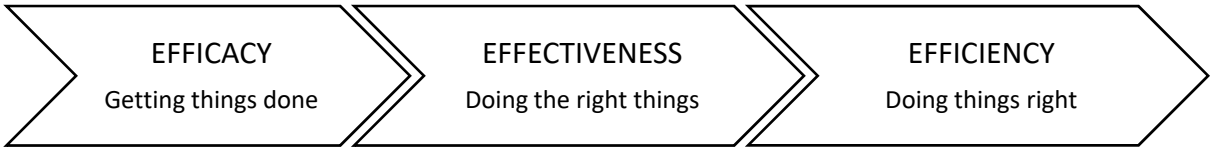
What does Efficiency mean?

If an agent is shown to be effective, what is the cost-benefit ratio? Is it possible to achieve our goals in a cheaper and better way? Cost includes not only money, but also discomfort, pain, absenteeism, disability, and social stigma. If a health care measure has not been demonstrated to be effective, there is little point looking at efficiency, for if it is not effective, the cheapest alternative is not to use it at all. At times, of course, political and societal pressures may drive a program even if it is not effective. However, this chapter will focus only on the science of evaluation and specifically on the issue of effectiveness in evaluating health services.

Efficacy, effectiveness, efficiency… These terms sound confusingly similar. Commonly utilized in medical research, project management, and decision science, they're often involved in everyday conversations. If you're during a hurry, here's the difference:

- Efficacy means getting things done
- Effectiveness means doing the proper things
- Efficiency means doing things right

Sounds confusing? Don't worry; I felt an equivalent before I looked into it. Let's undergo each of those terms one by one, and you'll understand the difference, and why it matters.



Efficacy means getting things done

The word "efficacy" is usually utilized in a scientific setting. Asking about the efficacy of an intervention is asking whether it are able to do the specified result, albeit that's under very specific and controlled conditions. Efficacy is that the ability to make the expected effect.

For instance, a medicine could also be ready to improve a patient's symptoms in a perfect environment, where they're closely monitored in order that they stick perfectly to the prescription. If that's the case, the drug has demonstrated efficacy. Yes, you'll need a really specific protocol, but if you are doing , the medication does get the thing done (where "the thing" is improving a patient's symptoms).

As Dr Enrique Burches and Dr Marta Burches explain: "Efficacy, within the healthcare sector, is that the capacity for beneficial change (or therapeutic effect) of a given intervention (for example a drug, medical device, surgery or a public health intervention) under ideal or controlled conditions." Because it's a highly specific measure which makes little sense in everyday situations, you'll rarely hear the term efficacy outside of pharmacological and clinical trials.

Effectiveness means doing the proper things

But getting things done isn't necessarily enough. A drug that improves a patient's symptoms under perfect conditions is technically getting things done, but is it getting the proper things done?

For instance, you'll create a treatment for diabetes with high efficacy, where the patient must stay plugged to a machine day each day for 6 months, under close supervision from a medical team. After six months of treatment, you measure a clear improvement. Sure, the treatment demonstrates efficacy it's getting things done but if your goal is to realistically treat people with diabetes, you wouldn't be ready to call it effective it's not getting the proper things done.

In clinical trials, effectiveness trials are about how well a treatment works within the world, outside of the superbly controlled conditions of efficacy trials. Intervention studies are often placed on a continuum, with a progression from efficacy trials to effectiveness trials. Efficacy are often defined because the performance of an intervention under ideal and controlled circumstances, whereas effectiveness refers to its performance under 'real-world' conditions. However, the excellence between the 2 sorts of trial may be a continuum instead of a dichotomy, because it is probably going impossible to perform a pure efficacy study or pure effectiveness study.

The concept of efficacy versus effectiveness also can be helpful when brooding about other areas of our lives, like project management and deciding generally. A thought that seems perfect under ideal conditions might not stand the test of chaos which real world inevitably brings about. When choosing between two solutions with similar levels of efficacy, it is sensible to travel with the foremost effective one.

Efficiency means doing things right

Once you've got found an efficient solution, you'll then attempt to improve it by making it more efficient. The Oxford Dictionary offers several definitions of efficiency. The primary one is useless: "The state or quality of being efficient." The second one the technical definition is far more interesting. It defines efficiency as "the ratio of the useful work performed by a machine or during a process to the entire energy expended or heat taken in."

Measuring efficiency is comparing a solution's input to its output. You'll effectively get the proper things done, but in an inefficient manner. Efficiency is about doing things within the most economical way, whether in terms of your time, energy, or money.

In clinical trials, two drugs might be equally effective: they both manage to enhance patients' symptoms within the world. However, if one among them is far more costly than the opposite one, that drug won't be considered as efficient. Similarly, in project management or deciding, you'll come up with an answer that achieves the proper result (effective), but is needlessly costly (inefficient).

In his book The Tipping Point, Malcolm Gladwell describes a modest yet incredibly efficient healthcare solution: "The Band-Aid is a cheap, convenient, and remarkably versatile solution to an astonishing array of problems. In their history, Band-Aids have probably allowed many people to stay working or playing tennis or cooking or walking once they would otherwise have had to prevent. The Band-Aid solution is really the simplest quite solution because it involves solving a drag with the minimum amount of effort and time and price."

Effectiveness, Efficacy and Efficiency within the Management

Efficiency, effectiveness and efficacy, in formal management discussions, combat very different meanings and were originally industrial management concepts that came aged within the early twentieth century. Peter Drucker, an expert of the management, in his book "the effective executive"[4] developed these concepts.

Effectiveness is doing "the right" things, for instance setting right targets to realize an overall goal (the effect). it's the extent to which planned outcomes, goals, or objectives are achieved as a result of an activity, intervention or initiative intended to realize the specified effect, under ordinary circumstances

(not controlled circumstances like during a laboratory).

Efficacy is getting things done. It's the power to supply a desired amount of the specified effect, or success in achieving a given goal.

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

Effectiveness, Efficacy and Efficiency within the Health Care Sector

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

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

Effectiveness links to the notion of external validity, therein 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 most sorts of studies wont to evaluate treatments. Within 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 supported evidence gathered from randomized controlled trials.[6-8] Currently, effectiveness are often defined because the extent to which a drug achieves its intended effect within the usual clinical setting. It is often evaluated through observational studies of real practice. In real practice studies ("how the drug works during 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 world in non-ideal circumstances.[7,8] Effectiveness can't be measured in controlled trials, because the act of inclusion into a study may be a distortion of usual practice [9,10]. On the contrary, observational studies (usually called pragmatic trials, real-world trials, and naturalistic trials) don't require randomization.

Nevertheless, nowadays we cannot obviate the concept evidence-based medicine. it had been initially developed by Guyatt, et al.[11] Evidence-based medicine is that 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 simplest available research information.[12] It categorizes differing types of clinical evidence and ranks them consistent with the strength of their freedom from the varied biases that beset medical research. one among the scales most used is that the Scottish Intercollegiate Guidelines Network (SIGN) which use a code together the study type for decide the extent of evidence.[13] High level of strength of advice is assigned to randomized control trials with a really low risk of bias and low levels of advice correspond to observational studies. Hence, the called "pragmatic studies" or "real-life studies" could be qualified as low evidence. This is able to suppose a contradiction, the foremost effectiveness studies would be rock-bottom level of evidence.

Efficiency as a Consolidated Concept within the Health Care Sector

Efficiency is that 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 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 of course of great interest to national governments, international donors and other stakeholders within the health sector. As what sort of product is being evaluated, we distinguish between two sorts of outputs: Health services (visits, drugs, admissions) and health outcomes (by way of example: Preventable deaths, functional status, clinical outcomes like vital sign or blood glucose control).

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

A New Prospective

As are often seen, there have developed a series of artificial meanings on the utilization of efficacy, effectiveness and efficiency that impede the comprehension of the subject. Especially, within the field of the drugs there has spread the utilization of those words with a meaning different from that of the important language. Hereby, there's understood that effectiveness is an intervention or initiative intended to realize the specified effect, under ordinary circumstances (not controlled circumstances like during a laboratory). Similarly, efficacy is an objective achieved under controlled circumstances. As a

consequence, this artificial use does (that don't correspond to an intuitive meaning) that we should always know before the previously arbitrary definitions to be ready to understand medical topic about which it treats. We are forced to know before these artificial meanings to understand the topic which is discussed. Additionally, the artful meaning that provides to the term's efficacy and effectiveness corresponds to other perfectly catalogued terms. That is, words we are trying to find 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 an idea in ideal situation and not place in practices yet or, an idea of action designed to realize a long-term or overall aim. Strategies are the ideas and broad approaches that support the goal, an in-depth plan for achieving success in situations like war, politics, business, industry or sport. Strategy defines, or outlines, the specified goals and why you ought to set about achieving them. The term tactics would be the fixing practice of the plan conceived by the strategy in ideal situation. Tactics are the precise action items, details and activities that has got to occur for the strategy to achieve success. Tactics are the actions you're taking in implementing your strategy. These actions comprise what's to be done, in what order, using which tools and personnel. In summary, strategy is that the what and why. Tactics is that the how. Therefore, would be more correct to define as strategic efficacy/effectiveness to the aptitude to supply a looked effect realized in ideal conditions and tactical efficacy/effectiveness to an equivalent effect looked in ordinary circumstances. Efficiency would be kept in its current definition because the ratio of the output to the inputs of any system. By means of those new proposed terms, we might avoid the utilization of slightly precise others as pragmatic or conducted in real conditions to ask certain clinical tests in not so strict conditions.

How is it, that guidelines for treatment often seem unrelated to the patient sitting ahead of the doctor? Guidelines are mostly supported evidence gathered from randomised controlled trials. These trials are excellent at assessing efficacy - that's, can a treatment work? Despite this, trials aren't without substantial biases. Many of us could also be screened before a couple of are chosen to be included during a study, yet the results of the study are going to be applied to the very people that were excluded. The population studied in trials tends to be young, male, white, affected by one condition and employing a single treatment. Most patients, a minimum of generally practice, don't fit this description. They often have multiple illnesses, take multiple medications and are either too young or too old to possess been included in clinical trials. Perhaps we should always accept a proposal to define efficacy in reference to medications because the extent to which a drug has the power to cause its intended effect under ideal circumstances, like during a randomised clinical test.

Efficacy isn't an equivalent as effectiveness. A treatment is effective if it works in real world in non-ideal circumstances. In real world, medications are going to be utilized in doses and frequencies never studied and in patient groups never assessed within the trials. Drugs are going to be utilized in combination with other medications that haven't been tested for interactions, and by people aside from the patient - the over the garden fence syndrome. Effectiveness can't be measured in controlled trials, because the act of inclusion into a study may be a distortion of usual practice.

Effectiveness are often defined because the extent to which a drug achieves its intended effect within the usual clinical setting. It is often evaluated through observational studies of real practice. This enables practice to be assessed in qualitative also as quantitative terms.

Australia is compatible to conduct observational studies because we've a high standard of relatively unrestricted practice and good national databases, like those held by the insurance Commission. These databases are often used for validating researchers separate database effectiveness studies. In America there are very large patient databases held by the Health Maintenance Organisations. Their size is impressive, but size isn't everything. the info may are collected primarily for billing and that they could also be incomplete. Clinical practice is usually governed by protocols, and medications are limited to those supplied by the present preferred providers. The reimbursement mechanism for doctors may mean that they code conditions at the very best severity level. Patients belonging to at least one of those organisations might not represent the American population as an entire. In Britain, the overall Practice Research Database, compiled from practice electronic records, is extremely useful, especially for studies in pharmaco-epidemiology. British enjoy relatively unrestricted clinical practice, but they are doing not have readily usable national datasets against which to see the validity of their database studies.

It is an understatement that drugs are licensed to be used almost exclusively on the results of controlled trials, yet they're withdrawn from use due to observational data that might not be acceptable to licensing authorities. Biases are present in observational studies, even as they're in trials, but they will be defined and sometimes controlled for, giving these studies a way greater value than that currently awarded to them.

Efficiency depends on whether a drug is worth its cost to individuals or society. The foremost efficacious treatment, supported the simplest evidence, might not be the foremost cost-effective option. It's going to not be acceptable to patients. In every country, rationing of health care may be a reality. There's no country, however wealthy, which will afford to deliver all the health care possible to the entire of its

population in the least times. Rationing could also be implicit or explicit, but it'll happen. Good effectiveness and efficiency studies will make this rationing more informed.

Good practical guidelines, like the Therapeutic Guidelines series, are clearly vital and very useful. they might be made even more relevant to the patient ahead of the doctor, by being less hooked in to efficacy studies. we should always make more use of effectiveness and efficiency studies and abandon the censorship of the evidence drawn from them.

Careful: effective isn't an equivalent as efficient

Neither of those words should be confused efficiently, which is "the state or quality of being efficient, or ready to accomplish something with the smallest amount waste of your time and effort; competency in performance." you'll consider efficiency as going a step further: it not only measures how well something does its job, but how quickly and/or cheaply it can roll in the hay.

Discerning the difference (and similarities!) between effective and efficacious are often tricky, especially once you consider related words like efficient and effectual. One effective thanks to understand these terms? Take a fast check out our Synonym Study of the word effective that separates out all of those very similar words. As always, you'll thank English language and its roots in Latin for making learning these definitions and connections so, er, fun?

Before we advance, it's important to means a couple of reasons why these words are so confusing. The primary is that all of them, of course, need to do with getting stuff done. Second, all of those words look really almost like one another. There's actually a reason behind it: all of them ultimately come from Latin verbs supported the basis ex- (in this case meaning "thoroughly") and therefore the verb facere ("to do" or "to make").

Vaccine efficacy vs. vaccine effectiveness

While efficacy and effectiveness may function as synonyms generally usage, they really have a selected difference in epidemiology, especially when pertaining to vaccines.

Vaccine efficacy may be a measure of how well a vaccine lowers the prevalence of a disease under ideal, controlled conditions. In other words, it's a measure of how well a vaccine prevents the spread of an epidemic when tested during a lab or highly controlled study.

Vaccine effectiveness may be a measure of how well a vaccine lowers the prevalence of a disease under "real world" conditions. In other words, it's the measure of how well a vaccine prevents the spread of the disease when actually used call at society.

Putting it all at once, scientists have an interest within the difference between these two measurements because it provides important information about the vaccine which will not be testable during a lab, like if the vaccine is just too expensive or if it's suffering from a condition (e.g., a patient's age) that the researchers might not have accounted for.

Scientists are now keeping an in-depth eye on COVID-19 vaccine distribution and seeing how the vaccine effectiveness compares to the vaccine efficacy. no matter which word we use, one thing is for sure: we all want this pandemic to be over!

CONCLUSION

To summarise: efficacy means getting things done (is it working?), effectiveness means doing the proper things (is it actually working well?), and efficiency means doing things right (is it working within the most economical way?). It's helpful to consider them during this particular order. First, confirm the answer can actually achieve the specified result, albeit that efficacy requires very specific conditions. Then, test your solution during a real-world environment. Finally, if the answer is effective, find out ways to form it more economical more efficient.

REFERENCES

1. Mackenzie R, Dixon AK. Measuring the effects of imaging: an evaluative framework. Clin Radiol. 1995 Aug;50(8):513-8. doi: 10.1016/s0009-9260(05)83184-8. PMID: 7656516.
2. Salkeld G, Davey P, Arnolda G (1995) A critical review of health-related economic evaluations in Australia: Implications for health policy. Health Policy 31: 111-125.
3. Angus Stevenson (2010) Oxford Dictionary of English (3 ed). Oxford University Press, Oxford, ISBN: 978-0-19-957112-3.
4. Drucker PF (1966) The Effective Executive. Harper & Row, New York.
5. Cochrane AL (1972) Effectiveness and Efficiency: Random Reflections on Health Services. Nuffield Provincial Hospitals Trust, London.
6. Anglemyer A, Horvath HT, Bero L (2014) Healthcare outcomes assessed with observational study designs compared with those assessed in randomized trials. Cochrane Database of Systematic Reviews 2014: MR000034.
7. Benson K, Hartz AJ (2000) A Comparison of Observational Studies and Randomized, Controlled Trials. N Engl J Med 342: 1878-1886.
8. Roland M, Torgerson DJ (1998) What are pragmatic trials? BMJ 316: 285.
9. Treweek S, Zwarenstein M (2009) Making trials matter: Pragmatic and explanatory trials and the problem of applicability. Trials 10: 37.
10. Revicki DA, Frank L (1999) Pharmacoeconomic evaluation in the real world. Efectiveness versus efficay studies. Pharmacoeconomics 15: 423-434.
11. Guyatt GH, Sackett DL, Sinclair JC, Hayward R, Cook DJ, et al. (1995) Users' Guides to the Medical Literature: IX. A method for grading health care recommendations. JAMA 274: 1800-1804.

12. Sackett DL, Rosenberg WMC, Gary JAM, Haynes RB, Richardson WS (1996) Evidence based medicine: What is it and what it isn't. BMJ 312: 71-72.

13. Harbour R, Miller J (2001) A new system for grading recommendations in evidence-based guidelines. BMJ 323: 334-336.

14. Palmer S, Torgerson DJ (1999) Economics notes: Definitions of efficiency. BMJ 318: 1136.

15. Kernick DP (2003) Introduction to health economics for the medical practitioner. Postgrad Med J 79: 147-150.