Chapter 07 / Capítulo 07

Evidence-based biomedicine: methodology for research, standardization, and scientific procedural aspects (English Edition)
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Health Outcomes Research / Investigación en Resultados de Salud

Health Outcomes Research (HOR) emerged at the end of the 1980s due to the notable discrepancy between the results obtained under experimental conditions (efficacy) in randomized clinical trials (RCTs) and the results in routine clinical practice (effectiveness). This difference, estimated at approximately 30 %, is attributed to factors such as sample selection in RCTs and the actual conditions of intervention implementation, which are rarely ideal (e.g., exact compliance with drug treatment). In addition, HTA emerged in response to the limited range of outcomes evaluated in RCTs, which focused on intermediate outcomes and often excluded outcomes important to patients, such as mortality or event prevention. With the patient becoming the center of the healthcare system, there was a need to identify the outcomes that really matter to patients and to develop reliable and valid methods to measure them.

The first and most significant HRA studies were promoted by the Agency for Health Care Policy and Research (AHCPR) in the United States to improve the quality of care, the effectiveness of health interventions, and the health outcomes of patients and the population. To this end, Patient Outcomes Research Teams (PORTs) were established, groups dedicated to evaluating high-cost and highly variable conditions.

IRS is closely related to Evidence-Based Medicine (EBM). While EBM focuses on decision-making in clinical research, IRS evaluates the consequences of these decisions in routine practice, translating evidence into clinical practice. HRI is defined as the study of the outcomes of health interventions in everyday clinical practice, clarifying and expanding knowledge of their effects and the factors that determine the transition from disease to health. It focuses on three key aspects: a) identifying the most relevant outcomes to measure; b) developing valid and reliable measurement tools; and c) quantifying the part of the effect attributable to the intervention.

In summary, Health Outcomes Research (HOR) is characterized by several key aspects: it focuses on everyday clinical practice, evaluates the effectiveness of health interventions, and prioritizes patient benefits, emphasizing variables relevant to patients, such as quality of life and satisfaction with health or treatment. In addition, it employs established research methods, similar to those used in epidemiology and biomedical research, and adopts both individual- and population-based approaches.

7.1. Contributions of health outcomes research to the practical work of healthcare professionals

For a physician in clinical practice, Health Outcomes Research (HOR) is essential, as it provides information on the effectiveness of diagnostic, preventive, and therapeutic interventions implemented daily. HOR provides results of these interventions in terms of outcomes relevant to patients, such as survival, years of life without disease, improvement in quality of life, cost reduction, and treatment tolerance.

In addition, it allows physicians to link the physiological variables they deal with in their daily work with humanistic aspects. Clinical practice acts as a real-world laboratory for testing biomedical science hypotheses. Every day, it generates enormous amounts of data that are

often underutilized for validating or refuting scientific theories.

When an experienced clinician compares accepted theories in biomedicine, such as the effects of a drug, with observed reality, they often find discrepancies that challenge these "accepted truths" and generate new hypotheses. Clinical practice is the ideal environment for asking questions and proposing new theories. For healthcare professionals seeking to remain intellectually active, the challenge is to integrate a methodology for validating innovations into their daily routine. IRS is the perfect tool for this purpose, allowing us to question the real effectiveness of the procedures and therapies used in clinical practice.

7.2. Types of outcomes and studies in Meath outcomes research

The appropriate selection of the type of outcome to be studied is fundamental in Health Outcomes Research (HOR), as it seeks to correlate the results of interventions with the attributes and process of the care provided. The type of outcome to be measured in each study will depend on the intervention being evaluated and whether it focuses on an individual patient or groups of patients. The outcomes measured influence the study design and the precision characteristics needed to determine the extent to which the effect is due to the intervention rather than to prior health status.

The type of outcome investigated (e.g., morbidity, mortality, prevalence, costs, quality of life) will determine the nature of the study. Studies based on clinical effectiveness variables evaluate the performance of an intervention or treatment, using a clinical indicator as an outcome measure (e.g., cholesterol reduction). Economic evaluation studies can measure the economic impact of a disease or intervention or compare the cost and effectiveness of different treatment options in routine practice. In addition, studies focusing on patient-important health outcomes are gaining popularity among healthcare professionals.

Patient-reported outcomes (PROs) include subjective assessments of any aspect of patients' health. They may include symptoms, functional status, overall well-being, health-related quality of life, satisfaction with care, preferences, and adherence, among others. PROs offer unique information and have become a valuable research tool for several reasons. First, some effects of treatment can only be perceived by patients, such as decreased pain or improved well-being. Second, they provide an overview of treatment effectiveness. Third, data collected directly from patients is often more reliable than data obtained by external observers. Two important aspects of PROs are patient satisfaction and health-related quality of life.

7.3. Patient satisfaction

A common practice for assessing healthcare outcomes is through measuring patient satisfaction. In this way, patients evaluate the quality of care, attention, and treatment received from healthcare personnel, as well as accessibility, comfort, and other elements related to the structure and organization of healthcare institutions.

7.3.1. Concept

Several experts argue that among the numerous psychological theories, the "expectancy-disconfirmation theory" best describes the concept of patient satisfaction. According to this theory, satisfaction is related to the patient's expectations regarding the care, staff, or facility providing treatment. It is determined by the discrepancy between what they expect and what they experience. In short, satisfaction is considered an indicator of patient behavior, influencing

decisions such as whether to attend a medical appointment or follow a prescribed treatment.

7.3.2. Applications and measuring instruments

The most common way to assess patient satisfaction is through satisfaction surveys. There are several variants of these surveys, including those specific to treatment satisfaction, such as the Diabetes Treatment Satisfaction Questionnaire (DTSQ), which assesses satisfaction with treatment and changes in satisfaction, and others focused on treatment adherence, such as the Morisky-Green Test. They are also used to measure service quality, such as SERVQUAL and its adaptation to the healthcare sector, SERVQHOS. In addition to surveys, there are other methodologies for measuring satisfaction, such as focus group techniques, nominal groups, interviews with managers (to identify strengths and areas for improvement), quality circles or improvement groups, suggestion boxes, audits, and the free choice of doctor and healthcare center.

7.4. Quality of life in relation to health

Health-related quality of life (HRQoL) has become one of the most interesting and rapidly developing areas within Health Outcomes Research (HOR) in recent decades. This measure of health status, unlike more traditional indicators such as mortality, morbidity, and clinical parameters (all of which are objective measures), incorporates the added value of quantifying how individuals perceive their illness and health status, as well as the impact of these on their lives.

7.4.1. Concept

Although various factors can affect population health, the concept of health-related quality of life (HRQoL) as a health outcome indicator focuses specifically on aspects closely linked to health. The objective is to measure "the value that individuals, groups of individuals, and society in general place on the duration of life, adjusted for limitations, functional states, perceptions, and social opportunities that are affected by diseases, injuries, medical treatments, or health policies."

7.4.2. Applications and measuring instruments

Over the past 40 years, the use of health-related quality of life (HRQoL) has expanded in several areas, notably: 1) In descriptive studies, where the HRQoL level of participants is commonly detailed; 2) In clinical trials, to assess how interventions affect the HRQoL of subjects; 3) In the context of health policy, using it to measure the impact of health planning and programs on the HRQoL of the population; 4) In clinical management, as a tool for setting priorities when resources are limited; 5) In population follow-up studies, where HRQoL serves as an indicator for assessing and tracking the health of the population over time; 6) In clinical practice, to monitor patients' HRQoL and in therapeutic decision-making.

Questionnaires are fundamental tools for measuring health-related quality of life (HRQoL) based on information provided by individuals themselves.

A person with better HRQoL is considered to have the optimal health status across all its dimensions (physical functioning, mental health, emotional state, pain, and social functioning).

These questionnaires are divided into two broad categories according to their target audience: generic and specific.

Generic questionnaires are designed to be applied to patients with various pathologies. Their main advantage is that they allow for the comparison of groups of patients with different diseases. In addition, they are useful for identifying problems or unexpected effects in specific pathologies, as they cover a wide range of dimensions. On the other hand, specific questionnaires are created for specific diseases, symptoms, or populations.

They are more relevant and acceptable to individuals because they include only dimensions relevant to their pathology or situation and are considered more sensitive in detecting the effects of therapeutic interventions than generic measures. However, in research projects, it is ideal to combine a generic instrument with a specific one.

Among the best known and most widely used generic questionnaires are the Short Form Health Survey (SF) family of instruments and the EuroQol-5 Dimensions (EQ-5D).

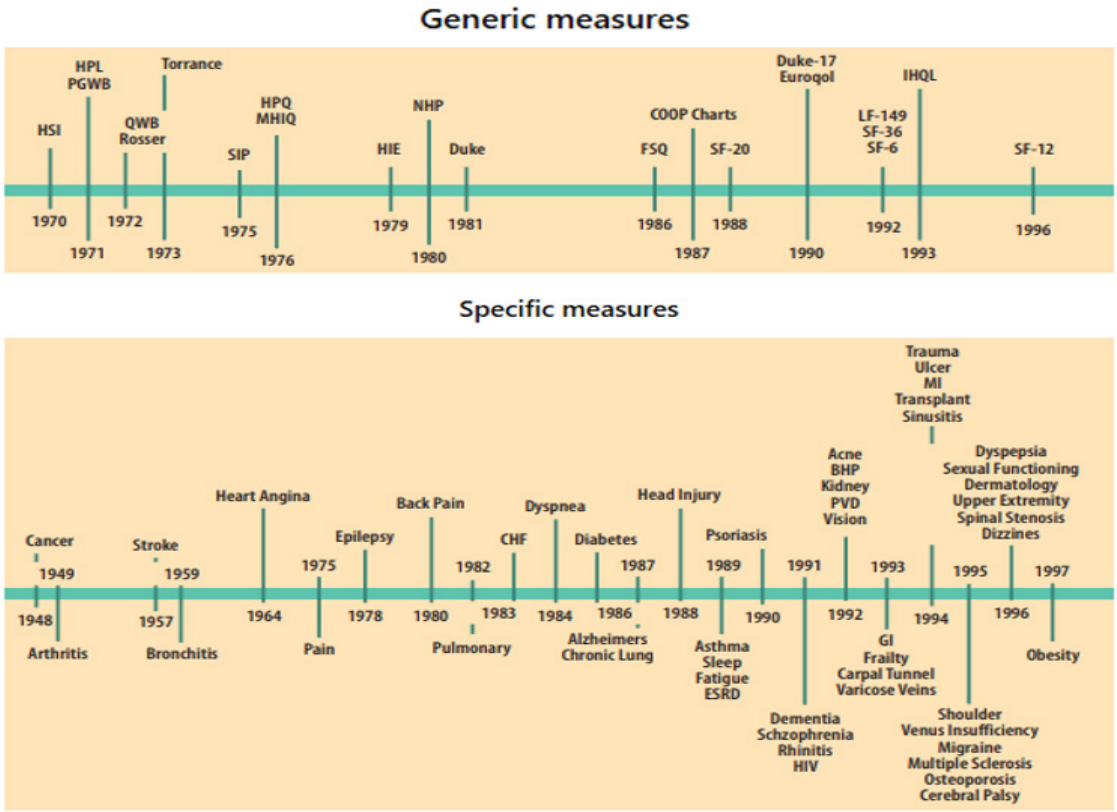


Figure 7.1. Timeline of health-related quality of life questionnaires

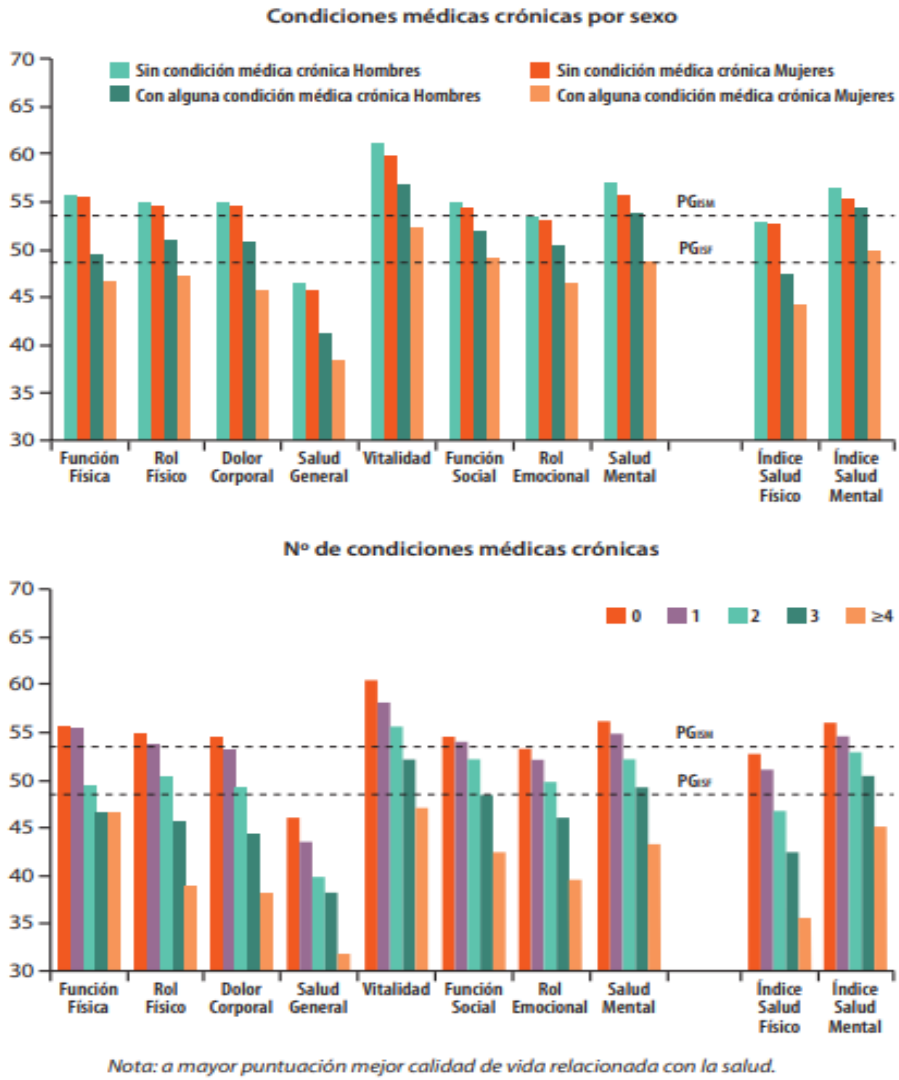
7.4.3. Family SF

The "SF" series of questionnaires has been a key model for the development and validation of other instruments, both generic and specific. This is due to several notable reasons: 1) These questionnaires are available in different lengths (36, 12, and 8 items), making them suitable for use in a variety of contexts, such as clinical practice, research studies, chronic or acute diseases, and the economic evaluation of healthcare interventions; 2) They have been shown to have excellent metric properties in a wide range of patient populations. In addition, they offer

different versions depending on the time frame to which the questions refer: the perception of the last week (acute) or the last four weeks (standard). With the SF-36 and SF-12v2, as illustrated in figure 7.1, it is possible to obtain both a health profile of the population (based on its eight dimensions) and health indices, which summarize these dimensions into physical and mental components.

7.4.4. EQ-5D

The EQ-5D is a multidimensional instrument that stands out for its brevity and ease of use. It covers five dimensions of health (mobility, self-care, usual activities, pain/discomfort, and anxiety/depression), with three levels of severity in each dimension, yielding 243 (3 to the power of 5) distinct health states. The Spanish version of the EQ-5D has demonstrated reliability and validity in several studies, which is why it has been widely used in health research, as well as in cost-effectiveness and cost-utility analyses.



Nota: a mayor puntuación mejor calidad de vida relacionada con la salud.

Figure 7.2. Health profile scores and quality of life indices (example, Spanish population)

In conclusion, the interest in developing HRQoL measurement stems from the need for instruments that are simple, easy to use, and, above all, relevant and useful for everyday clinical practice. Therefore, the important thing for healthcare professionals is not so much to create new instruments, but to use those that are already available, valid, reliable, and manageable so that they can be integrated into everyday medical practice. This is crucial, as it is the only way to obtain reliable data to evaluate real improvements in patients' quality of life.