# Comparative Effectiveness and Efficacy Research and Analysis for Practice (CEERAP)

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## Applications in Health Care

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# Foreword

## Evidence-based Health Care, Systematic Reviews, Effectiveness and Efficacy Research to Face Challenges of Healthcare Systems

Peter Kranke

Shortage of resources is an irrevocable constant in almost every industry. In the healthcare industry it affects the macro-, meso- and micro-level and thus each clinical department and individual healthcare provider. Therefore, evidence-based health care (EBHC), including dentistry and nursing, with its claim to apply the "current best evidence in making decisions about the care of individual patients" (1) has gained its widespread acceptance as a suited basis for clinical decision making. This is true for "rich" and "poor" countries in terms of their gross domestic product alike. When the idea and concept of EBHC was developed and the term was first introduced to the healthcare system, common misconceptions included the assumption that all applied interventions and diagnostic procedures need to be thoroughly investigated in many clinical trials with a superior external validity to deserve being mentioned under the heading "evidence-based". Later on, the conception of EBHC as a long and winding road towards greater transparency regarding the reasoning for or against specific interventions gained more popularity and the prevailing picture nowadays can be described with the saying: "The journey is the reward", which means that EBHC does not require a bundle of meta-analyses, but that clinicians are aware of the fact how well-proven or not the applied interventions and diagnostic tests are in their discipline.

But why is it of utmost importance for every healthcare system to embrace the principles of evidence-based health care? The reasons can be viewed from a more economic viewpoint or a more humanitarian point of view: Apart from being effective, the challenging goal for every health professionals and healthcare system is to be efficient and thus to guarantee not only that good things are done, but to do more good than anything else that could be done with the same resources; this is true for both publicly financed as well as private financed healthcare insurance systems and for the poor and rich countries alike. "Costs" can be viewed as monetary costs but

imply further aspects. Monetary costs are only one aspect of being efficient, not necessarily less meaningful is the potential harm of diagnostic tests and interventions that need to be weighed against the potential benefit.

One of the key drivers of the profound "success" of evidence-based practice is the rapidly increasing amount of clinical evidence that demands quality assessment and the correct tools to summarize the findings as well as to elucidate a clinical "bottom-line" that really helps clinicians to improve patient care. When aiming to base clinical decision-making on the current best evidence, systematic reviews, especially when performed in an adequate way, are considered of paramount importance. The reader of this book may find some examples in the field of endodontics that may help to illustrate the importance of systematic reviews and evidence-based clinical decision making.

The "hierarchy" of evidence and thus the decision why a dedicated piece of evidence is more useful for clinical decision-making than another one is mainly determined by various suggestions of levels of evidence. In this context the concept and description of the Oxford Centre of Evidence Based Medicine has gained widespread acceptance (http://www.cebm.net/index.aspx?o=1025).

If transferred to a clinically oriented search algorithm, the recommendations simply mean that clinicians should first search for systematic reviews and if these or other higher ranked resources of evidence are not available, to try and locate case series, expert opinion without formal quality-assessed recommendations or even case reports (which can help a lot, if there is no information as far as anesthetic management is concerned for uncommon diseases). Furthermore, well-informed patients have increasing access to the same evidence that function as the basis for clinical decision making. This influences the decision-making process and forces clinicians to "keep up with them" so as to prevent us being taught by patients what the "state of the art" is in distinct clinical situations.

But critical clinical thinking demands more than just applying evidence. It demands us that we re-assess our practice and question the added-value and potential harm of so-called "routine interventions" and "cutting-edge technology" alike. It further necessitates that even the methodology that is applied to make decisions will be continuously re-assessed.

Systematic reviews of high-quality randomized controlled trials are one way of dealing with uncertainty and especially the over-information with respect to new as well as traditional interventions in every healthcare system.

However, these trials usually only reflect to which extent a drug or any other intervention in healthcare has the ability to bring about its intended effect under ideal circumstances.

Effectiveness, on the other hand, can be defined as the extent to which an intervention in healthcare, e.g. a drug, achieves its intended effect in the usual (not the ideal) clinical setting. Both approaches cover different aspects of validity: While randomized controlled trials are usually needed due to their emphasis on internal validity, observational studies or more pragmatic trials of real practice put emphasis on the external validity with respect to their findings.

Readers of this book may find more sophisticated elaborations on the differences of these terms. But despite all these explanations there will always remain a kind of constructive vagueness in these terms. Therefore, for the author of this foreword the question whether "efficacy" or "effectiveness" is the key in guiding our therapy may be just splitting hairs. It is important for us to remember that only those both pieces of validity taken together form a complete picture of the value of an intervention for our healthcare systems.

What seems to be clear is the paramount importance of valid systematic reviews that summarize both of these pieces of evidence. This is especially true for healthcare providers struggling each day to base clinical decision-making on the current best evidence in an age where "information explosion" is a common buzzword in medicine and other industries alike. Thus, the presented book may help as a valuable introduction and auxiliary aide-memoire on how to improve healthcare with special focus on endodontics and nursing.

## Reference

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Critical intellectual assistance was provided to us in the compilation of the materials describing the fundamentals of CEERAP, and EBDM by my graduate students, Andre Barkhodarian, MS, Reem Ajaj, DDS, MS, Manisha H. Ramchandani, DDS, MS, and Oluwadayo Oluwadara, DDS, MS, PhD. Their constant and persevering efforts in my research group have generated and continue to generate excellent novel and cutting-edge developments in knowledge in evidence-based research, translational evidence-based practice, and translational effectiveness. Consequently, I also regard these graduate students of mine as the principal and key "movers" in the writing of this work and reiterate that any laud of this work must be attributed to them, more perhaps than to this author, for their discussions, contributions and participation in our weekly research meetings, where issues and concepts ranging from systematic reviews, evidence-based clinical decision-making and comparative effectiveness analysis are routinely entertained.

Among my undergraduate students whose most salient contribution to the present endeavor I wish not to forget, I cite with praise particularly Sohrab Danaie (predent) for his excellent assistance in the editorial processo of this compilation. The superb contribution of his directed independent research, and that of Nora Ghodousi (pre-dent), and Linda Phi (dental student), in particular, cannot be over-emphasized. Without their input, and especially Sohrab's dedication and excellence, this work could not have been assembled.

I join my co-editors in thanking Ms. Irmela Bohn and Dr. Sverre Klemp, Clinical Medicine at Springer-Verlag, and their dedicated staff, for their confidence and trust that this important collection of timely and critical chapters would see the light of day, despite the plethora of delays, due to a variety of reasons under nobody's direct control. The unwavering support that I and the co-editors received from Irmela's and Sverre's "Ateam" throughout the many months that led to the production of this

book is a vivid demonstration of the excellence of the editorial office of Clinical Medicine at Springer-Verlag.

It is with a sense of scientific diligence that I started this project. Today, as I complete it, I am overwhelmed by a sense of awe – again, as so often, in my scientific career – for the incredible depth and beauty, complexity in its simplicity, and simplicity in its complexity of translational research and of translational effectiveness. I can only compare the intricacies and beauty that I see in the study of comparative effectiveness and efficacy research for practice to that of the poet who stated….

*…avant donc que d'écrire, apprenez à penser…*
(Nicolas Boileau –Despréaux, 1636–1711, L'art Poétique, 1674)

Last but not least, I dedicate this work, as all of my academic endeavors to Olivia, and to Aymerica and Fredi. And this writing, as all, only and most humbly serves to further honor

*…la gloria di Colui che tutto move / per l'universo penetra e risplende / in una parte più e meno altrove…*

(Dante Alighieri, 1265–1321; La Divina Commedia, Paradiso, I 1–3).

Los Angeles, CA, USA Francesco Chiappelli

# Contents

# Part I

# Introduction

# Fundamentals: Building Communities of Practice in Comparative Effectiveness Research

1

Carl A. Maida

**Core Message**

A community of practice is comprised of individuals who share a common concern for a specific domain of knowledge. This chapter focuses on building communities of practice among clinicians and scientists engaged in comparative effectiveness research, an emerging field requiring collaborative partnerships among researchers, practitioners, and consumers for greater transparency in planning and implementing a broad-based and inclusive research agenda.

## 1.1 Introduction: Organizational Challenges in Comparative Effectiveness Research

### 1.1.1 The Methodology of Comparative Effectiveness Research

Comparative effectiveness research (CER) synthesizes the evidence about the benefits and harms of preventive, diagnostic, and therapeutic interventions related to a clinical condition, and to the delivery of care, on behalf of informed decision-making by clinicians, consumers, payers, and policymakers, thereby contributing to the improvement of health care of both individuals and populations [1].

The methodology of CER is that of systematic reviews of the literature, which summarize evidence from observational research and randomized clinical trials. However, even with well-defined study methods and guidelines for practicing this

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style of research, many scientists and clinicians have found it difficult to reach consensus on how to evaluate proposed CER studies.

A recent paper on the current capacity to conduct CER in the United States [2] found there were clear differences between academically trained observational researchers and those trained on the job to conduct randomized clinical trials. Moreover, these differences often led to misconceptions by members of study sections and peer review panels as they sought to evaluate proposals on comparative effectiveness studies. As a corrective measure, the report's authors recommended cross training each group in the methodologies of observational studies and randomized controlled trials.

An emergent "collaborative professionalism" [3], on behalf of learning and diffusion of research assumptions and modes of inquiry within a broader community of stakeholders in the CER arena, may eventually break down the resistances of entrenched experts arguing across a methodological divide. This collaborative ethos may yield a more enlightened professional practice of science, as teamwork is more effective in the production of knowledge in virtually all fields [4]. As a result, the emergent communities of practice will be better equipped to pursue comparative effectiveness studies to navigate the rapid growth of knowledge in biomedicine and biotechnology.

### 1.1.2 Effective Health Care Program

To this end, the federal government has expanded the Effective Health Care Program that was created in 2003 and funded by the Agency for Healthcare Research and Quality (AHRQ), the health services research arm of the US Department of Health and Human Services, which produces research reviews, research reports based upon clinical studies, and consumer-oriented summary guides that summarize research review findings, including the benefits and harms of various treatment options, in plain language.

This program was established under the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 with an initial appropriation of $15 million to conduct and support research on behalf of Medicare, Medicaid, and the State Children's Health Insurance Program, all established under the Social Security Act, and in 2008, Congress doubled the program budget to $30 million. The American Recovery and Reinvestment Act of 2009 provided an additional $300 million for CER signaling the importance of program to clinical and personal health care evidence-based decision-making.

The Effective Health Care Program partners include clinical researchers individual investigators, and members of: *Evidence-based Practice Centers*, housed in medical schools and thinks tanks, which focus on producing research reviews; the *Developing Evidence to Inform Decisions about Effectiveness* network of centers focusing on specific treatments; *Centers for Education and Research on Therapeutics*, each with a specific focus on a broad therapeutic theme, such as infectious disease or disorders of the joints and bones, and with a charge to increase awareness of the

benefits and harms of new and existing therapeutics; *Scientific Resource Center*, which is charged with coordinating peer reviews and public input for comparative effectiveness reviews and communicating with a broad range of stakeholders, including clinicians and consumers; *John M Eisenberg Center for Clinical Decisions and Communications Science*, which translates comparative effectiveness reviews and reports into materials appropriate for consumers, clinicians, and policymakers; and a group of stakeholders representing the interests of medicine, biotechnology, public health, law and administration, that provides input and guidance on the program's implementation and policy direction.

Beyond these partnerships is the need for collaborative efforts among researchers, ethicists, health economists, decision-makers, and members of the lay public targeted by new health technologies. Citing the case of effectiveness research in molecular genetic testing, Rugowski et al. [5] acknowledge the need for greater transparency and broader participation in decisions as these innovations move from discovery to clinical practice, once they are adopted by third-party payers and enter various health care systems.

In an effort to broaden the engagement of the lay public as consumers with a stake in evidence-based health care decisions, the National Academy Medicine recommended the following on behalf of greater stakeholder involvement in framing its CER agenda: "The CER Program should fully involve consumers, patients, and caregivers in key aspects of CER, including strategic planning, priority setting, research proposal development, peer review, and dissemination…[and] develop strategies to reach out to, engage, support, educate, and, as necessary, prepare consumers, patients, and caregivers for leadership roles in these activities [6]." However, for laypeople to be effectively integrated into these expert panels and advisory committees, federal agencies will need to find ways to translate sophisticated scientific knowledge to information for nonspecialists on behalf of more meaningful citizen involvement in policy decisions [7].

Full citizen participation in the management of biotechnology will require policy elites to adopt a new set of "social technological" values as they carry out tasks of risk assessment, cost-benefit analysis, and predictive modeling in the current environment of public accountability and participation in technological assessment. Transparency and meaningful interaction between experts and the lay public require "technologies of humility to complement the predictive approaches: to make apparent the possibility of unforeseen consequences; to make explicit the normative that lurks within the technical; and to acknowledge from the start the need for plural viewpoints and collective learning" [8].

## 1.2 Lay Participation in Health Technology Assessment

### 1.2.1 Introduction

Lay-oriented organizations have supported their members' active engagement in the public realm and successfully inculcated civic virtues, such as activism and citizen

participation in science. Civic networks and other mutual help organizations, which have their basis in voluntarism, social trust, and reciprocity, are forms of social capital that facilitate cooperation and communication and are needed to resolve the myriad dilemmas of collective action, especially in the health care arena.

The continued growth of social capital will require broader citizen access to electronically transmitted information and interactive communication technologies to stimulate interest in health affairs and participation in national policy dialogues in the areas of biomedicine, the environment, and food. Nowhere is this more apparent than in the widening gap between information elites, such as scientists and policymakers, and the lay public with respect to knowledge about and access to computing and networked communication resources.

The newer information and communication technologies – such as bioinformatics applications for the management and analysis of molecular biological data – are spreading rapidly throughout the scientific research community. National and global networks of information, including the exchange of data and research findings, define the communications revolution in science. Issues surrounding privacy, confidentiality of data, security, and access are central to a scientific culture increasingly dependent upon computer-mediated communication.

Advanced communication strategies presently support national and international consortia of researchers based at universities, federal agencies, and nonprofit research organizations. To keep consortia of geographically dispersed investigators functioning efficiently, biomedical research programs have pursued an information resources agenda to encourage broad communication and collaboration.

As strategic resources, these information flows have become key sources of power, influence, and competitive advantage in the scientific enterprise. Large research programs that routinely use computer-mediated communication to frame and to maintain everyday interaction also settle controversies within "wired" and "wireless" electronic arenas.

As technologies promoting instantaneous information and data exchange diffuse across the scientific research community, virtual arenas are emerging within research programs to debate various scientific and policy issues. Within these arenas, scientists engage in controversies and resolve disputes surrounding theoretical and research claims; they also approach pragmatic concerns about resources, funding, and, increasingly, public reactions to their work.

### 1.2.2 Virtual Communities

The rapid growth in ownership of personal computers and increasing access by laypersons to networked communication technologies, including Internet-based social networking sites and related online "groups," has led to the formation of diverse lay interest groups, or "virtual communities." These are communities of frequently geographically dispersed individuals, linked together by interactive communication, who share a common concern.

Similar to arenas sustained by scientific and policy elites, lay electronic networks engage and affiliate participants in spontaneous, but also considered, discussion and debate around clearly meaningful issues. Despite the substantial early apprehension of social scientists that computer-mediated communication would further isolate individuals and restrict their participation in the public sphere, alliances built electronically appear to strengthen social and civic ties, but also may blur many visually defined boundaries based on ethnicity, class, gender, and disability.

Electronic alliances can potentially sustain citizen participation within an emerging federalism that increasingly requires the advice of laypersons in the development of scientific and technological policies in fields, such as genomics, stem cell research, and environmental toxics.

However, only recently have expert-run organizations begun to fully incorporate certain laypersons, chiefly in consultative roles, to give voice to concerns over the direction and outcome of professional interventions. This move toward pluralism was stimulated by criticism, from both professionals and laypersons, that many organizations were slow to modify their practices to reflect contemporary ethical and political concerns. To facilitate change, a few professionals set out to foster significant lay participation in governance activities and instill in their colleagues greater respect for the lay "voice."

Federal agencies, such as the National Institutes of Health, require nonscientists to help set research and development priorities and research agendas. Lay members on these panels often find themselves marginalized or intimidated as a result of their limited knowledge of the technical aspects of the research or social program under review. Alternatively, dual expert and lay advisory panels have been established, the latter supported by expert facilitators, to yield two sets of recommendations for policy direction [9]. Collective goods, such as health care, are "indivisible in that their benefits accrue to society at large" [10].

As collective goods and services, these resources are not distributed through the market, but rather through public organizations. Because common resources are not owned by any individual, it will always require collective action to mobilize demand for greater access to them. Mature societies have recently undertaken to address inequities of both knowledge of and access to collective resources through initiatives fostering certain forms of lay involvement in the direction of publicly funded science and technology programs, including biomedicine and biotechnology.

The collaboration of experts and laypersons on technology assessment panels in Europe provides a model of lay participation in decision-making concerning delivery of a variety of public goods. The Danish government has established citizen tribunals where ordinary citizens sit together in a consensus conference to listen to presentations by diverse experts, question them, deliberate among themselves, and produce a set of recommendations.

The Danish model specifies a process of expert-layperson relations that establishes a forum to consider the voices of experts in both the technical and social dimensions of a particular technology, but also those of organized interest or stakeholder groups. However, in all cases, the final set of recommendations is left to the judgments of the informed citizens who sit on the public tribunal [11].

In the United States, technical experts use consensus methods to evaluate and to solve problems in controversial areas of medicine and technology. The National Institutes of Health panels have used consensus strategies to generate state-of-the-art opinions for purposes of evaluating and setting standards of quality for certain medical and surgical procedures.

In contrast to the Danish process, American consensus panels bring together representative professionals who, facilitated by objective and skilled leaders, engage in a group process that yields findings that are "clear and specific guides to action" [12]. However, for these practices to fully incorporate the "lay voice," either in informative or consultative modes of participation, considerable efforts will be required to cultivate "scientific citizenship" through communities of practice focused on understanding and assessing the impacts of new and emerging biotechnologies [13].

## 1.3 Toward Building Communities of Practice

### 1.3.1 Knowledge-Intensive Activities

The "community of practice" is an organizational form that complements the current knowledge economy, namely, the "production and services based upon knowledge-intensive activities" [14], which has accelerated since the late twentieth century with advances in molecular biology, drug discovery, information production and dissemination. With the promise to "radically galvanize knowledge sharing, learning, and change" [15].

A community of practice provides a framework for understanding social learning in complex organizations, specifically the notion of "knowing." For novices and experts alike, knowing within a scientific or clinical community of practice is based upon socially defined *competence*, or the ability to act and to be viewed as a competent member, and ongoing *experience* within the context of the community.

According to Wenger [16], "belonging" to a particular community is based upon engagement, imagination, and alignment within a social learning system that supports and sustains members and the community, itself. Communities of practice provide the framework for "social learning," because members: share a sense of joint enterprise, indicative of the level of learning energy within the community; interact on the basis of mutuality, which points to the depth of social capital generated by mutual engagement; and share a repertoire of resources, indicating the degree of participants' self-awareness. This framework – of knowing, belonging, and social learning through more informal styles characteristic of a communities of practice – provides members with the skills to meaningfully engage in knowledge production, exchange, and transformation in complex organizations.

All communities of practice contain three structural elements:

(a) Domain, or the area of shared inquiry
(b) Community, or the environment where relationships are built
(c) Practice, or the body of knowledge, methods, tools, cases, and stories [17]

A community of practice, therefore, is comprised of individuals who share a common interest in a specific domain of knowledge. As a collaborative peer network based upon a shared area of inquiry, communities of practice are voluntary and focused on learning and on building capacity. They are engaged in sharing knowledge, developing expertise, and solving problems within the specific area. The notion of inquiry-based learning within an informal community derives from the Progressive philosopher and educator John Dewey, who envisioned the school as having features similar to "the workshop, the laboratory, [with] the materials, the tools with which the child may construct, create, and actively enquire" [18].

### 1.3.2 Democracy and Education

For Dewey, the classroom was a "miniature community" that provided students "with the instruments of effective self-direction" [19] tools that would help them to gain greater control over their cognition and social behavior, and over their social and physical environments.

In *Democracy and Education*, Dewey defined education as "that reconstruction or reorganization of experience which adds to the meaning of experience, and which increases ability to direct the course of subsequent experience" [20]. According to Lawrence Cremin [21], Dewey's conception of growth was central to his view of the aim of education as a directive structure that would "expand the range of social situations in which individuals perceived issues and made and acted upon choices." Dewey thus understood the school as a place where students develop the habits of mind that would enable them "to control their surroundings rather than merely adapt to them" [22].

For Dewey, social reconstruction could only occur after individuals used scientific inquiry to reflect upon their experience and to understand the social consequences of their behavior. Hence, it is only in and through a "community of inquirers" – an idea that Dewey received from philosopher Charles Sanders Peirce [23] – that cognitive processes for regulating human behavior could be developed and tested.

Dewey's embedded these ideas in his experimental school, which he founded in Chicago in 1897 and envisioned as a setting where rules, based on such experientially derived knowledge, were socialized and used to guide further inquiry, presumably for community betterment. For over a century, Dewey's thinking has guided research in the ways people learn, inside and outside of school, and especially within communities of practice.

Jean Lave and Etienne Wenger [24] maintain that learning viewed as *situated activity* has as its central characteristic a process they call legitimate peripheral participation (LPP). Learning is therefore a function of the activity, context, and culture in which it occurs (i.e., is situated).

LPP permits learners to develop both hard and soft knowledge: *hard knowledge* can be easily articulated and captured, while *soft knowledge* cannot be learned

simply by demonstration or instruction, but through learning the language and unspoken conventions of the community.

Learners participate in communities of practice, moving toward full participation in the sociocultural practices of a community. LPP, as a process of *co-participation*, provides a way to speak about crucial relations between newcomers and established members, and about their activities, identities, knowledge, and practice.

Lave, in calling for a "rethinking of the notion of learning, treating it as an emerging property of the whole person's legitimate peripheral participation in communities of practice" [25], views the need for "strategies of inquiry" that situate learning within more interactive frames, such as craft apprenticeships that offer more counterintuitive approaches to skill mastery.

For Lave, learning practices across domains share a set of conventions that make for their effectiveness, specifically "breaking down distinctions between learning and doing, between social identity and knowledge, between education and occupation, and between form and content" [26]. Hence, learning through LPP is more enculturation than the acquisition of formal, expert knowledge; learners acquire "the embodied ability to behave as community members" [27].

Moreover, a sense of "knowing-in-action" [28] comes from participation in practice-oriented learning experiences that include mentorships, internships, and apprenticeships in various kinds of skilled work. Beyond cultivating flexible work skills, Halpern [29] understands the pedagogical importance of work-based learning activities, such as apprenticeships, where novices develop habits of mind grounded in a sense of accomplishment and personal responsibility.

These learning encounters between students and their mentors, *across the life course*, can be conceived as dialogues, and a distinctive feature of collaborative learning is that the mentor-student relationship is constructed and negotiated through such encounters. Though them, students may come to perceive the value of collaborative learning, experience this form of learning, and may be rewarded through the responses of others to their collaborative work.

Scientists in the emerging field of social neuroscience view encounters, such as mentoring and tutoring, as ways to enhance social interaction that is essential to learning, which, in turn, is supported by neural circuits linking perception and action for "close coupling and attunement between self and other," and for synaptic plasticity [30].

For organizations confronting the dual challenges of globalization and the knowledge revolution, collaborative action-learning networks, or communities of practice, are ways to promote peer-to-peer collaborative activities in the face of change. These formations can effectively build capacity and broaden the scope and scale of an organization through innovative and pilot initiatives, complementing the formal units that support the core mission.

As informal structures, communities of practice can integrate new talent and support cross-sector collaborations, as they have the capacity to cross boundaries, thereby accommodating "peripheral members who only participate occasionally" [31]. The latter function is central to the task of incorporating new actors into the current mix of clinicians, consumers, payers, and policymakers – the core stakeholder groups in CER initiatives.

## 1.4 Expanding Communities of Practice in Comparative Effectiveness Research

### 1.4.1 Citizen Science

Two constituencies that may support the expansion of communities of practice in CER by bridging researchers, clinicians, and the lay public, across diverse populations and subpopulations, are citizen scientists and community health workers. Both constituent groups approach the production or transfer of critical knowledge through decidedly personal and interpersonal styles, enacted from the "bottom up," and most often at the local level.

"Citizen Science," as conceptualized by Irwin [32] and Bäckstrand [33], involves science initiated and carried out by citizens not trained to be professional scientists. Early lay efforts to monitor common pool resources and common property were carried out by users who depend upon a resource in order to sustain them over longer periods of time.

These efforts focused on meeting local and regional challenges of environmental degradation and resource depletion, threatening watersheds, fisheries, and pasturage, with a goal of building consensus among users of a particular resource "so that joint benefits will outweigh current costs" [34]. Initial interest in citizen science took place in the ecological and the environmental health sciences, as average citizens became more aware of the impact of science and technology on their personal lives and on their community's quality of life [35].

Biomonitoring, or body burden research, came about with increasing public demands for information about human exposure to chemicals in the environment [36]. Community residents working together with environmental health scientists in universities and community-based organizations have monitored workplaces toxics, air and water pollution, household lead, flame retardants in consumer products, and environmental chemicals in breast milk [37].

These studies are most often carried out through community-based participatory research (CBPR), a collaborative approach to community-driven information gathering that focuses on health care disparities. Through this method, academic researchers work together with residents and community representatives to design and carry out research, and to transfer the knowledge gained to the community, presumably to improve health and community well-being through social action and advocacy initiatives [38].

A recent evidence report for the AHRQ, based upon a systematic review of community-based participatory research studies, expanded the definition of this form of research to involve:

(a) C*o-learning and reciprocal transfer of expertise*, by all research partners, with particular emphasis on the issues that can be studied with CBPR methods
(b) *Shared decision-making power*
(c) *Mutual ownership* of the processes and products of the research enterprise [39].

In biomedicine, the move toward citizen science has been spurred by a trend toward the cultivation of autonomy and self-advocacy skills on behalf of the "active patient" as a model of the clinician-patient relationship [40]. As drug discovery and pharmaceutical product development advance along with molecular medicine and human genomics, consumer activism is increasing in the health care arena.

*Active patients* access health information on the Internet and form online communities based upon a common medical condition, and disease-oriented social networks have emerged to share illness and treatment experiences. In some cases, through an informal "gift economy" [41], patients will volunteer for lay-designed research programs that bypass more formal professional efforts to translate research into new treatments.

These efforts by a sophisticated lay public that has gained personal control of electronic health records, together with facile access to online communities of drug consumers, will often drive the next phase of pharmaceutical research and development. Then, there are the challenges associated with the translation of molecular genetics tests into clinical practice within systems of care.

An example is the Personal Genome Project (PGP), founded by Harvard geneticist George Church, which proposes the creation of a public database that will eventually contain genotype and phenotype data on 100,000 people, all of which will be in the public domain [42]. As a "citizen science" initiative, participation in the PGP is voluntary, and there is no charge for data generated by the project, although there is a rigorous eligibility screening process.

A similar initiative is the Coriell Personalized Medicine Collaborative with plans to enroll 100,000 people, as well, so that participants may access, through a secure web portal, "personalized risk results for genetic variants" that may be detectable and treatable [43]. In each instance, researchers, clinicians, ethicists, and genetic counselors collaborate with information technology specialists and the lay public to promote informed and meaningful use of personal genetic information [44].

### 1.4.2 Community Health Workers

Community health workers are specially trained residents of the community, who are culturally, linguistically, and ethnically congruent with members of a specific patient population. The community health worker role builds on the influence of natural helpers, lay persons living in a community that neighbors look to for advice and guidance, and may be paid or volunteer. Their role usually involves program outreach, facilitating access to care or other services, teaching and demonstrating preventive and self-care practices, and serving as role models, coaches, and translators.

Two relatively new titles for the community health worker role are "patient navigator" and "health promoter." In most community health programs, the majority incorporating this role are women who are residents of largely minority ethnic communities, and typically serve as informal culturally congruent intermediaries between professionals and their clients.

A comprehensive study of community health worker interventions for the AHRQ, using evidence-based practice methods of dual review and consensus procedure, found that community health workers have the potential to address two fundamental imperatives in improving health care in the United States: the need to address substantial and persistent health care disparities and the need to translate more research into practice. By virtue of their role as a bridge to the health care system, community health workers can help to disseminate widely efficacious interventions to populations that rarely benefit from health care advances [45].

Lay health educators have proven to be effective in providing social support needed to assist in carrying out self-care behaviors that improve the health of persons living with a chronic disease. *Promotores*, as lay health educators in Spanish-speaking communities, show the potential for supporting chronic disease self-management to improve oral health outcomes [46].

To be effective, clinicians will need to be cognizant of patients' culturally prescribed modes of expressing attitudes toward illness, stigma, and help seeking. To this end, a lay approach trains culturally concordant community residents, selected on both their ability to communicate and their leadership qualities, to engage in health education and social support for chronic disease self-management as *Promotores de Salud* [47]:

(1) *Promotores* are trained to recognize social isolation in their clients, and to implement support strategies to address this isolation by enhancing self-efficacy through verbal persuasion, including encouragement and positive support for personal success in adherence behavior – strategies that were found to be effective in previous studies [48].
(2) *Promotores* trained in chronic disease and its management, self-care practices, and social support may serve as indigenous arbitrators, coaches, and mediators, for patients with chronic conditions, such as periodontitis.
(3) The *Promotores* as lay health educators represent a move toward extending appropriately designed information about the care system and about patients' responsibility in their treatment. The intent is to enable the patient to both navigate the health care system and to overcome the considerable barriers that patients with low health literacy bring to the health encounter.

The central intent of incorporating laypersons, such as citizen scientists and community health workers, in communities of practice on behalf of CER assessment and dissemination is the creation of a bridging network of academic researchers, clinicians, and communicators and educators who translate health science concepts into terms broadly understood by the lay public. This incorporation of the lay voice in collaborative peer networks for greater public transparency in biomedical decision-making is clearly important in the face of legal challenges from governmental health authorities, as in the case of genetic protectionism [49].

The emergent interdisciplinary networks would promote communities of practice comprised of researchers, educational practitioners, and governmental agency staff, and local level community health workers on behalf of health literacy, and greater engagement in science by the public, as citizen scientists, locally, nationally, and globally. The goal is to foster an appreciation of community-based transformational,

interdisciplinary approaches to learning about and communicating the results of innovations in the biomedical sciences, ranging from new drugs to genetic testing across a wide range of venues and their publics.

## 1.5 Disseminating Innovations Across Communities of Practice

### 1.5.1 Learning Pathway and Procedure

As potentially "disruptive innovations" [50] within and across formal organizations and professional workplaces, communities of practice enable accelerated learning, connect learning to action, and facilitate quality and performance improvement. However, these learning pathways can only come about through computer-supported collaborative work. To this end, Hoadley and Kilner [51] posit a fourfold framework for knowledge building within communities of practice:

- Quality content
- Meaningful conversation
- Connections to foster trust
- An information context that facilitates understanding both the source and usefulness of the knowledge

All four elements contribute toward assuring each member's understanding of the specific purpose of their community of practice; however, technology is the key to enabling "collective intelligence" within a community. This form of learning at the collective level – one that breaks down communication barriers through continuous exchange of data, information, and knowledge in a more open and informal manner – is enhanced by social media that promote collaborative inquiry-based learning. Hence, members within these communities are in continuous communication via face-to-face meetings, teleconferencing, and web-based platforms.

Critical to the success of communities of practice, then, is a communication platform that serves as an ongoing learning venue to connect members, transfer best practices, and promote effective partnership arrangements with other knowledge networks. However, while technology may enhance the adoption of collaborative attitudes to support collective intelligence, shifts in social roles among members are key to structural change [52]. Studies of technical work have shown the primacy of social relationships in affecting change within organizational and occupational structures, over the mere mastery of modified work-related tasks brought about by new technologies in the work setting.

Because social change on behalf of the transfer of any technology relies upon a change in ongoing interpersonal relationships, collaborative methods have been developed to disseminate and support the adoption of best practices across diverse stakeholders. These methods emphasize adult learning principles, including peer-based, interactive, and skill-focused learning. The learning process thus brings together diverse teams to work on improving a process, practice, or system, and to learn from their collective experiences and challenges.

Follow-up consultation activities include phone and web-based conferences, which provide feedback to support sustained learning and share progress across a collaborative peer-based network. These "learning communities" are comprised of individuals who share a common interest in a subject and who then collaborate over time to share ideas, find solutions, and build innovations.

The collaboration is sustained through face-to-face and web-based contacts, ongoing sharing among participants of the barriers and solutions to skill development and practice, dissemination of best practices, and support for the incorporation of innovations among participants. An expected outcome is the adoption and sustained use of best practices by stakeholders in diverse settings across systems of care.

An effective learning community will therefore need to employ information resources that facilitate communication and collaboration among clinicians and scientists dispersed across systems of care, regionally and often nationally, through networking arrangements, Internet resources, and enhanced conferencing capabilities.

### 1.5.2 Emerging Evidence-Based Diagnostic and Therapeutic Technologies

A major goal of communities of practice in biomedicine is the dissemination of emerging evidence-based diagnostic and therapeutic technologies, as broadly as possible. Once a technology's potential is fully realized, a community of practice model can be used to stimulate interest among members of primary care professions in widely adopting it, and thereby diffuse the innovation. Based on Rogers' diffusion of innovation theory [53], which posits an incremental process in which different groups sequentially adopt an innovative product or technology.

The first group to adopt a new product or technique is the *innovators*, followed by *early adopters*, *early majority*, *late majority*, and *laggards*. Members of each group possess characteristics that are associated with their willingness to adopt a new technology at a certain stage in the model. For example, innovators (the first group to adopt) are generally more educated about the technology and venturesome, whereas members of the early majority (a group that adopts later than the innovators) are more deliberative when making decisions.

Adoption by one group paves the way for adoption by a successive group. Therefore, dissemination of any innovative technique requires adoption first by a group of innovators before the next group, namely, the early adopters, will use it. If a group of innovators will not embrace an innovation, then the technology will not be disseminated more broadly. The same can be said for all groups: a previous group must first adopt the technology or idea to continue the adoption process to the next group.

Rogers' model is appropriate for dissemination studies of a specific diagnostic or therapeutic technique, such as the combined use genomics (the study of an organism's genome and the function of genes) and proteomics (the large-scale study of protein structures and functions), to understand disease a nascent approach with thousands of genes and proteins can be studied simultaneously [54].

These technologies are cutting-edge and inherently innovative. A dissemination plan would therefore involve introducing the genomic or proteomic technique to targeted groups at appropriate times using Rogers' model as a guide. The strategy is that successively larger groups at each step of the dissemination process will adopt the emerging technology. For example, studies are underway to analyze the array of proteins expressed in endodontic infections, and to determine the diagnostic utility of this approach to modeling the inflammatory process [55].

Should an appropriate diagnostic technology be developed that applies proteomics to periodontal disease treatment, for example, the dentists in a health care system, because they have adopted existing oral diagnostic protocols, will most likely be the first users of the technique, and function as *innovators* in Rogers' model.

Successful adoption of the *hypothetical* proteomic diagnostic protocol by these innovators paves the way for disseminating the technology beyond the first diffusion group to additional and larger groups of adopters beyond dentistry and oral medicine. However, to achieve this goal, provider-specific trainings to primary care physicians, nurses, and physician assistants will need to be implemented to address specific issues and content – for example, periodontal disease risk and its relation to diagnosis and treatment of systemic diseases, such as diabetes – to cultivate an appreciation for the innovation.

The primary care health care providers – as well as the clinical settings themselves – are the *early adopters* in Rogers' model. This step of training and dissemination moves the specific diagnostic technique out of the innovation phase into the next phase where the technology can be used more widely by the variety of providers. At this point, techniques, which are broad and robust, are adapted more specifically for clinical disciplines that are involved in primary care, making the technology more appropriate for providers that are likely to utilize it as part of their clinical encounters with patients. This step increases the applicability and suitability of the specific, applied proteomic diagnostic technique, to providers that are naturally positioned to conduct early diagnosis.

Moving a technology from adoption by early adopters to the early majority (the next, larger group to utilize the technique) generally poses the most difficult challenge in the dissemination process. This will be particularly true with regard to the application of proteomics, as cultural, institutional, and behavioral impediments may be encountered when transferring this diagnostic technology from university and corporate research environments to clinical and community settings.

To deal with the difficulty of disseminating to early majority groups, clinical researchers and consultants in primary care disciplines will be required to help expand the use of diagnostic techniques to members of their professional networks in health care organizations. These researchers and consultants who have successfully adapted and implemented the innovative proteomic techniques, from the bench to the clinic, will be best able to identify dissemination avenues in their professional networks because they are most familiar with those networks (as both members and leaders).

Dissemination to early majority groups will occur through networking and interacting within the formal structures of the various health care organizations, and through the more informal partnerships and relationships that the researchers and

consultants bring to their work on behalf of dissemination of the innovations through communities of practice. Additionally, creating sustainable training materials and a standardized training curriculum will increase the potential for dissemination of a technological advance. Ultimately, the early adopters are well positioned to understand how an innovative proteomic technology can be best disseminated to others working in their field; thus, they will lead this step of the dissemination process.

### 1.5.3 Evaluating Communities of Practice

To evaluate communities of practice as change agents on behalf of a dissemination process will require a logic model – a theory-based approach that helps professionals within systems of care to make explicit links between their theories about what works best in their community, the strategies they plan to implement, and the outcomes they hope to achieve.

According to Hernandez and Hodges [56], a theory of change is the articulation of stakeholders' underlying beliefs and assumptions that guide a local service delivery strategy to incorporate an innovation and are critical for producing change and improving the knowledge of clinicians and consumers, and that of other stakeholders within a system of care.

Hernandez and Hodges [57] characterized a theory of change as having two broad components.

#### 1.5.3.1 Conceptualization and Operationalizing

The first involves conceptualizing and operationalizing the theory's *three core elements*:

(1) Needs and strengths of the population in the context of the environment in which change will occur
(2) Strategies stakeholders believe will accomplish desired outcomes
(3) The outcomes, including the desired change for the population in focus

#### 1.5.3.2 Understanding and Expressing

The second component involves both understanding and expressing how these three core elements are related and articulated by stakeholders in the change process.

In sum, the theory of change approach provides a picture of: what a system of care will look like, the necessary local service delivery and infrastructural changes, the degree of stakeholder vision for the desired change, and the steps necessary to build consensus among stakeholders for optimal engagement in the change process.

A logic model evaluation approach identifies key action steps that will likely result in system change, notably on behalf of adopting a diagnostic or therapeutic technique – identifying a sample within provider and community – based systems for diagnostic or therapeutic intervention, introducing trainings on best practices on behalf of implementing the intervention in these systems, modifying these best practices based upon pilot testing, developing training materials, providing broader training within the targeted systems, and working with these systems to sustain the array of best practices.

One goal of the theory-based approach is documenting progress toward change at the patient, staff, center, community, and service system levels. The theory-based logic model will inform evaluation as it prioritizes those monitoring and evaluation activities that will yield the most useful information about change at each level.

Using this approach, *formative or process evaluation* of focus groups, clinical team meetings, case conferences, consumer and stakeholder collaborations, expert consultations and collaborations, service system consultations and trainings will provide information about short-term outcomes to help monitor progress and make midcourse corrections, and generally improve diagnostic or therapeutic behavior based upon the best practice.

*Summative or outcomes evaluation* will generate data that can demonstrate results of the best practices to stakeholders at each level of change (patient, staff, center, community, and service system), to funders, and the wider community of practice. The focus is on intermediate-term outcomes and impact, as these data will be most helpful in describing the quality and effectiveness of the best practices. The impact of the best practices at the five *levels of change* is thereby documented, including lessons learned from the piloting and early implementation experience, and with outcome data, best practices can be sustained.

One method to assure accountability is through establishing *benchmarks*, or indicators, as markers of success. Within each action step, it will be necessary to measure a number of factors; initially there will be *process metrics* related to the proposed patients to be served using the best practice, logs related to staffing engaged in disseminating the innovation, and rosters of planned activities and reports by staff to compare the status, i.e., quality and effectiveness of the best practices delivered against what was expected.

Finally, there will be *outcome metrics* to determine the impact of the best practices on participants at each *level of change* (patient, staff, center, community, and service system). This will require a comparison of measures at baseline and at the end of program implementation. Central to the evaluation task is the intent to understand how diverse stakeholders engage in a change process around the adoption of innovative therapeutic and diagnostics in complex health care organizations [58].

Within many health care systems, technology assessment and adoption will require sustaining communities of practice that include, together with scientists and clinicians, the participation of citizen scientists, governmental agency experts, and members of community-based organizations that represent the broader population. How these diverse constituents comprehend and express the ways the *three core elements* are related and articulated at each *level of change* will be an essential outcome of the evaluation.

## 1.6 Conclusion

### 1.6.1 Toward Common Knowledge of Health and Disease

Recent debates have centered on increasing public understanding of science through citizen participation in the production of scientific knowledge, and the assessment of its applications in biotechnology and biomedicine, notably genomics, proteomics,

and bioinformatics. To sustain what has been called a "knowledge commons" [59], communities of practice can work toward diminishing the boundaries between the "expert" producers of research within the academic, public, nonprofit, and private sectors, and consumers across these sectors.

Ultimately, the goals are those of translating research into action to promote better decision-making by health care providers and their "active" patients, so that both may meaningfully engage in informed dialogues about the nature and quality of care. However, breaking down the "asymmetries of expertise in the professional-client relationship" [60] can only be achieved through collaborative methods, such as community-based participatory research, citizen science, learning communities, and the participation of lay health workers in dissemination and health literacy activities. In this way, clinicians and scientists may realize a more socially responsive transfer of knowledge derived from comparative effectiveness research across diverse sectors and constituencies.

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# Probability–Utility Model for Evidence-Based Decision-Making

2

Janet G. Bauer

**Core Message**

The probability–utility model is dependent on the clinical practice guideline for its function. This chapter focuses on model functions to provide decision analyses that demonstrate to clinicians and patients how personal preferences change the character of best evidence. Initially, patients are provided a clinical practice guideline based on "average patient" best evidence. The model works to demonstrate how decision, utility, and cost best evidence impact on decisions. It offers to patients an explanation of these impacts.

## 2.1 Introduction: Translational Evidence Mechanism

### 2.1.1 Translational Evidence Mechanism

Many advances have been made in reasoning best evidence, especially in the form of judgments, inferences, and conclusions. While the translational evidence mechanism provides the compact between researcher, clinicians, and patients by which this reasoning is developed, validated, disseminated, and refined, little progress has been made in producing technology to advance its practical use in displaying and utilizing this reasoning.

Pell et al. [1] state that current modes of assistance rarely present or make practical the delivery of best evidence to the shared decision-making process that occurs daily in private practice.

The translational evidence mechanism uses the probability–utility model [2] to provide decision analyses that demonstrate to clinicians and patients how personal

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preferences change the character of best evidence. Using dentistry as the discipline, the model displays and utilizes best evidence at the chair side for shared decision-making between dental provider and patient.

Within the dental assessment, evaluation, and treatment planning appointment, it serves as a patient- and dentist-centered decision aid that exists to augment and improve shared decision-making during the consultation. Thus, negotiations focus on discussing options and trade-offs rather than evidence, arbitration of which is appropriately accomplished with expertise within the translational evidence mechanism.

Secondly, this model structures the inputs and outputs needed for storing and managing information within the central database of the translational evidence mechanism. Thus, the model depends upon and also structures and liaisons with the central database. It operates upon and utilizes its repository of evidence in optimizing clinical decisions for patients by structuring inputs for evidence created from research. Its coordination vector for processing evidence within the central database is the decision algorithm.

### 2.1.2 Decision Algorithm

Decision algorithms are decision trees, developed as expert systems that structure clinical problems, analyze decisions and options, and chose and implement clinical protocols or clinical practice guidelines. For dentistry, the decision tree that we propose for the central database is the clinical decision tree of oral health (CDTOH) [3]. The CDTOH is a systematic understanding of an entire decision process in rendering clinical decisions.

It identifies the order of outcomes involved in diseases, treatments and their protocols, and therapies in impacting positively on the oral health of patients. The outcome is the terminal end of each of its branches. The best evidence that constitutes these terminal branches includes decision, utility, and cost data.

These data variables vest the clinical practice guideline with best evidence for use in shared decision-making. Using the CDTOH, a structure of relationships is made in the central database. This structure consists of nine levels of evidence domains. These levels are developed to coincide with the flow of the decision process used in the CDTOH. Thus, each level of the hierarchy progressively adds information to the decision process.

Thus, the clinical inquiry can follow the information trail until it reaches the actual data needed to respond to the initial inquiry. This is important to understand because the information collected along with the clinical inquiry:

- Patient demographics
- Assessments
- Follow-upswill impact on the data used to vest the CPG. For each level, information is managed to prioritize the evidence that most impacts on the quantitative and qualitative nature of the response to query.

For information technologists, this means structuring at each level requires normalization of the evidence across multiple tables, creating tables for decomposition (evidence stored secondary to the primary field key) and derivation (query responses

that require calculation of the evidence). Information technologists perform these structuring tasks to improve and prevent delayed or time-consuming look-up of evidence in responding to queries. Programming language is used to provide the evidence to the generic template that displays the results of the query.

## 2.2 Nine Levels of Evidence

### 2.2.1 Introduction

The nine levels of evidence are structured into domains that include:

- Decision-maker
- Physical and mental well-being
- Oral conditions
- Dental conditions
- Oral functioning
- Prevention and maintenance
- Judgment
- Values
- Relationships

Each level has sublevels of variables that associate best evidence respective to each branch termini or outcomes. Information technology uses a dynamic process to prioritize each sublevel of evidence such that best evidence is found to vest the CPG. This is a dynamic process because knowledge and relationships between outcomes change; in other words, the branches and their termini within an evidence domain develop new or eliminate previous known branches and termini.

The new developments are in response to changes that are gathered from new clinical inquires, updates in decision analysis, and differences in the meaning of best evidence as analyzed from patient choices, lifestyles, and behaviors. Thus, these changes develop different relationships in response to new findings.

In addition, the process must respond to queries that have single or multiple layers of comparison of evidence outcomes. While complex in visage, the administration of these types of queries is well within the administration of a central database. Using the decision-maker level, as an example, four fields define decision-makers:

(a) Dentist
(b) Patient
(c) Legal surrogate
(d) Family members

Each decision-maker impacts on whether treatment is ordered or not ordered. Often, evidence at this level will determine the outcome of whether a treatment plan is offered, accepted, or not accepted. When treatment effectiveness and efficacy is uncertain, access and utilization of dental services by patients are also uncertain. Often, decision-makers will rely on their personal utilities:

- Previous dental experiences
- Health beliefs and behaviors

- Preferences
- Health goals

In making decisions about access to dental care services. These lack the support from best evidence to clarify these utilities within the context of treatment outcomes. For example, evidence on functional status and accessing dental care services often affects how treatment options are communicated and decided upon by not only patients, but their dentists, legal surrogates, and family members who may weight their personal utilities in adverse to potentially positive, effective, and efficacious outcomes. Evidence on functional status provides estimates on life expectancy.

Life expectancy is predicted by demographic variables such as age, gender, and race. Utility measures include quality-adjusted life years [4] or other utility assessments [5, 6]. Cost is a variable considered later in the decision-making process when trade-offs between treatment options are discussed; unfortunately, cost may impact at this level when older adults, their dependents or caregivers, impose preferences that rate higher estate interests over quality-of-life issues. Thus, the inclusion of multiple decision-makers may make shared decision-making complex for access to dental care services, compliance in utilization, and improvements in health outcomes from potential dental care services.

### 2.2.2 Inputs

Research inputs vest the central database with data. These research inputs are utilized by the translational researcher's role in providing best evidence for the central database, evidence that will be used to respond to clinical queries of dental providers for use in patient consultations. Thus, there are two separate databases within the central database:

- An evidence-based database
- Research-vested database

The research database is important because the data inputted, then subsequently retrieved, is used to systematically derive best evidence for use in structuring outcome evidence for deriving clinical practice guidelines (CPGs). The evidence-based database provides clinical queries with CPGs that conform to a specific organization of decision (outcome), utility (preferences and values), and cost best evidence.

The probability–utility model defines these inputs that are more directed than reasoned in providing research data for peer review by translational evidence researchers. Table 2.1 provides categorical inputs that are requested of the researcher for inputting data.

Utilizing the focus group methodology, the call is for future research to provide and validate with researchers definitive input templates for obtaining published and non-published data for information technology strategies within the central, research-vested database.

**Table 2.1** Categorical inputs specific to researchers for the research-vested database

| Research-vested database | | |
|---|---|---|
| Input | Description | Use |
| Completion date of data collection | The length of time since the production of the data | Utility of data for CPG |
| Completion date of data management | The length of time from production to outcome derivation | Utility of data for CPG |
| Completion date of manuscript composition | The length of time from production of data to preparation of dissemination | Utility of data for CPG |
| Input date of non-published data | The length of time from production to input of data into system | Utility of data for CPG for non-filtered data |
| Input date of published data | The length of time from publication to input into system | Utility of data for CPG for filtered data |
| Expiration date of data significance | Researcher estimate of need to update data | Researcher utility of sensitivity and specificity of data |
| Population | Form input header | Form input header |
| Study N | Number of subjects | Systematic review |
| Analysis N | Number of subjects used for analysis, providing the number of subjects that were unavailable for analysis | Systematic review |
| Functional status | Functionally independent or dependent, or frail | CPG |
| Risk level | Low, equipoise, high | CPG |
| Age | Birth date preferred, specific, range | CPG and systematic review |
| Gender | Male, female | CPG and systematic review |
| Race/ethnicity | American-Indian/Alaska Native, Asian/Pacific Islander, Black/African-American, Latino/Hispanic, White, other | CPG and systematic review |
| Marital status | Single, married, widowed, single/divorced | CPG and systematic review |
| Educational status | Less than high school, some high school, high school graduate, some college, college graduate, graduate school | CPG and systematic review |
| Income level | Current societal levels defining poverty, low middle class, middle class, high middle class, highest income | CPG and systematic review |
| Medical diagnosis counts | Number of medical conditions | Systematic review |
| Specific medical condition | If applies, name of category of medical condition or specific reference | Systematic review |
| Medicines counts | Number of medications | Systematic review |
| Specific medicines | If applies, name of category of medicines or specific reference | Systematic review |

(continued)

**Table 2.1** (continued)

| Research-vested database Input | Description | Use |
|---|---|---|
| Allergy counts | Number of allergies to drugs including environmental (counted as one) | Systematic review |
| Dental insurance status | With or without | Systematic review |
| Known previous insurance coverage | Number of years since last coverage | Systematic review |
| Intervention | Specific intervention | CPG and systematic review |
| Comparison interventions | Comparative interventions | CPG and systematic review |
| Outcome(s) | Specific intervention | CPG and systematic review |
| Comparison outcome(s) | Comparative intervention | CPG and systematic review |
| Probabilities | Estimates | CPG and systematic review |
| Odd ratios | Estimates | CPG and systematic review |
| Measure(s) | Amount or direction | CPG and systematic review |
| Cost schedule(s) | Specific costs to intervention | CPG and systematic review |
| Significant difference | P level | CPG and systematic review |
| Clinical significance | Ranking | CPG and systematic review |
| Utility ranking | Ranking | CPG and systematic review |
| Meaning in practice ranking | Ranking | CPG and systematic review |

### 2.2.3 Clinical Practice

Clinical inputs are clinician-based rankings of clinical significance of CPGs developed by the translational researcher or those returned as products of shared decision-making or follow-up assessments at patient periodic dental examinations. Clinicians are provided a CPG with best evidence specific to a stated clinical question. Clinicians may include the dentist who initially posed the clinical question or a panel of dentist volunteers who have previously consented to rank CPGs for clinical significance.

The posed clinical question was created through a query submitted in which evidence could not be derived from the central, evidence-based database. In this scenario, the translational evidence researcher would access and utilize either the translational evidence research team or provide a call for data needed to vest the central, research-vested database. From these resources, best evidence is developed in response to the posed clinical question.

Once this data has been acquired and evidence systematically reviewed, best evidence is inputted into the central, evidence-based database. From there, a CPG is derived to respond and submitted for review of clinical significance by the clinician or clinician reviewer panel. A ranking is developed and inputted into the central, evidence-based database. From this point, a CPG is provided in response to the

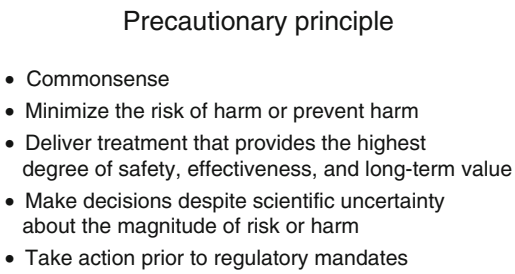



**Fig. 2.1** Precautionary principle

initial query or clinical question for shared decision-making. Now, what does the clinician do in the meantime?

The dentist does what clinicians have done years previously in uncertain clinical scenarios; clinicians use the precautionary principle (Fig. 2.1). The precautionary principle directs the clinicians to gather the resources of intuitive knowledge along with clinical experience and expertise to either logically derived a commonsense approach to the clinical problem while minimizing the risk of harm (prevent harm) in delivering treatment or therapy options that meet the highest degree of safety, effectiveness, and long-term value. Then, the clinician may follow this process in shared decision-making or give it a run through with a clinician with specific clinical expertise in the discipline for which the uncertainty applies.

Other clinical inputs from private practice include CPGs in which patients have rejected the utilities and cost estimates based on "average patient." In replacement of the "average patient" estimates, individual patients have manipulated interactive CPG stated estimates for their own. These estimates have been acquired from patient interaction and manipulation of the CPG during shared decision-making.

Once the optimal clinical decision is made, these estimates are inputted back to the translational evidence researcher for updating best evidence from private practice. With a multitude of private practices simulating research units, revised best evidence from private practice updates central database evidence using Bayesian statistics.

In this manner, the translational evidence researcher is alerted to derived CPG changes and acts accordingly to log, validate, and disseminated in various ways these changes. This dissemination may act as an alert, or flag, to the patient electronic chart from which the clinical question arose, and email alerts to dentists and researchers who have subscribed for this service.

Likewise, at each patient periodic dental examination and when appropriate, updates to decision and utility best evidence are made. These measured outcomes of patient's lifestyles and behaviors become new inputs to the evidence-based database through the electronic chart and using the CPG. Thus, revised data inputs are made using the CPG that was previously attached to the patient's electronic chart.

These inputs are based on observed differences between original patient-determined optimal clinical decisions and actual outcomes, behaviors, and compliance since the CPG implementation.

Within the database, these changes are analyzed to update the CPG. In this clinical scenario, revised data inputs act to provide feedback on the meaning in practice of the original and patient-derived CPG. These updates also serve to inform researchers and clinicians how the original accepted optimum clinical decision performed given the clinical scenario in which it was derived; in other words, the meaning in practice of the patient derived CPG.

Utilizing focus group methodology, the call is for future research to provide and validate with clinicians and patients definitive input templates for obtaining practice and patient outcomes for information technology strategies within the central, evidence-based database.

Prospectively, CPGs may be gamed by clinicians and patients alike in providing treatment or therapy options and outcome scenarios when accessing evidence for individual curiosity or decision-making prior to accessing dental services. This type of service would serve as an informational and educational tool for numerous constituencies including the public, policy makers, professionals, and governmental agencies in testing services and their potential for improved outcomes.

## 2.3 Outputs

### 2.3.1 Research

Research outputs are research questions posed to the translational evidence mechanism in responding to a clinical inquiry. In this scenario, best evidence does not exist to answer the clinical question. Therefore, a research question is postulated to acquire data from the research community for whom the subject of the question applies.

The outputs must be flexible enough to aid in research project development. Thus, focus group methodology is needed to provide and validate with researchers definitive output templates or queries that access data, either derived or not derived in responding to research questions. However, note must be made that this service must be regulated and information protected as to follow HIPAA and moral, ethical, and professional standards of the research community and the American Dental Association. Without these assurances and oversight, this is not a service that would be granted cavalierly.

Clinical outputs are in the form of a CPG. The character of the CPG input template is provided in response to a clinical question (Fig. 2.2). For example, a posed clinical question states: In a population of female subjects, 85 years of age and older, functionally independent, will dental implants, compared to removable partial denture or no treatment, increase chewing function and chewing efficacy at a reasonable cost?

Once the clinical question is submitted to the central, evidence-based database (Click Continue), a CPG is returned and attached to the patient's electronic chart (Fig. 2.3). Table 2.2 displays the outputs specific to the CPG.

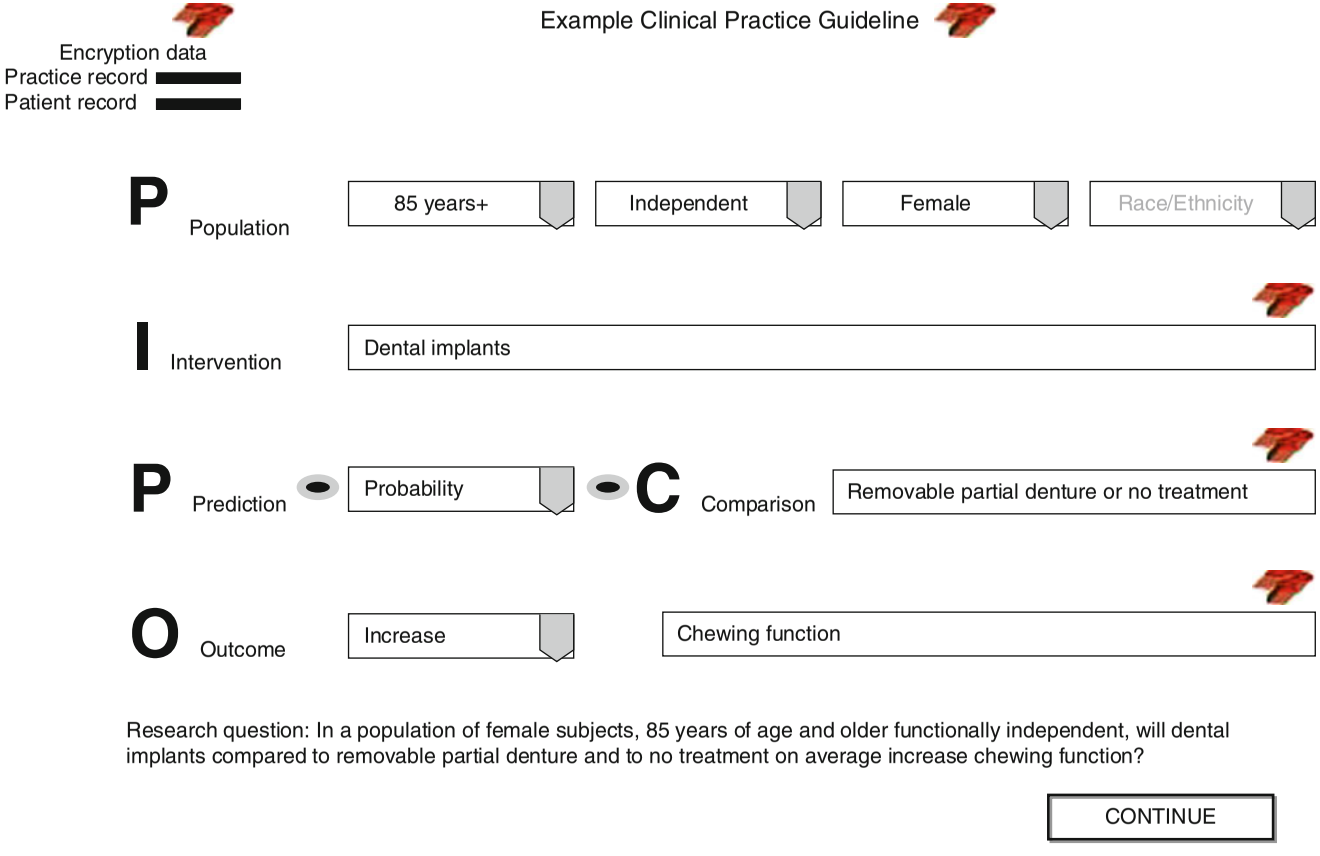
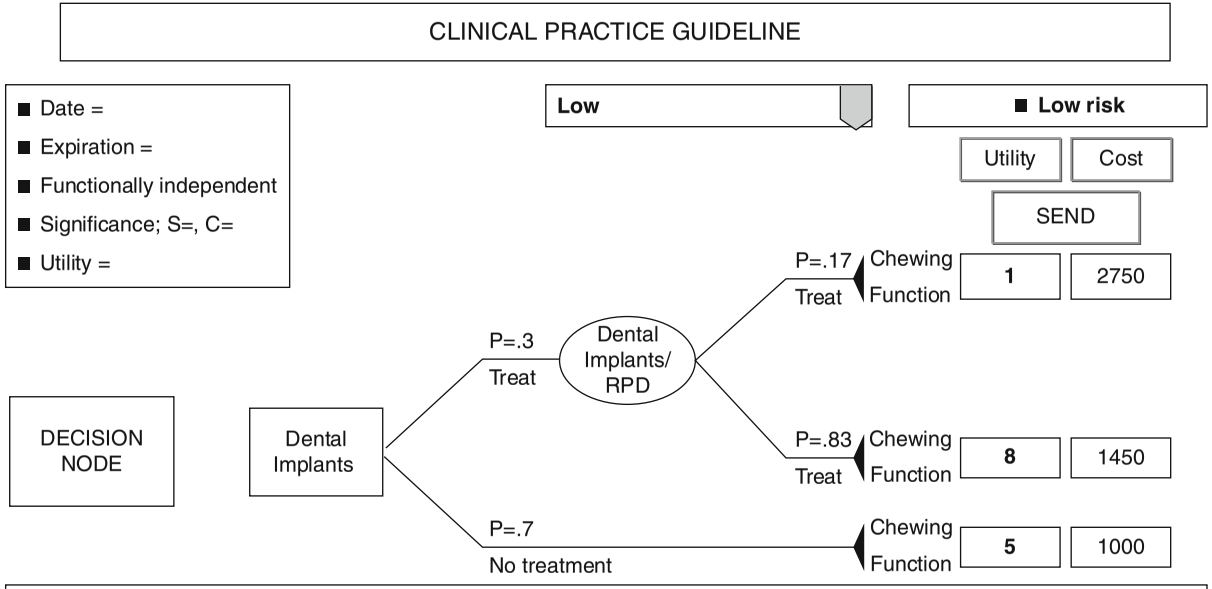



**Fig. 2.2** The CPG template with posed clinical question




| DATA | DENTIST | PATIENT |
|---|---|---|
| Utility:<br>• Rollback calculation for implant (1×17)=.17<br>• Rollback calculation for RPD (8×83)=6.6<br>• Rollback calculation for no treatment=3.5<br>• Calculation for average expected utility (1×17)+(8×83)=8.3 | The service benefit of implant treatment is .17, for RPD is 6.6, and for no treatment is 3.5. The average expected utility when comparing implant or RPD treatment over no treatment is 8.3 Thus, the benefit of RPD (8.3) compared with 6.3 for no treatment is still preferred. | The service benefit to the average patient is .17 for implant treatment, 3.5 no treatment, and 6.6 RPD treatment however, if I want to compare treatments, I will look at the analyses of expected and average expected utilities to see the trade-off analyses for average patients. |

**Fig. 2.3** The CPG returned with best evidence

**Table 2.2** Clinical outputs included in the CPG

| Evidence-based database | | |
|---|---|---|
| Output | Description | Use |
| Date of CPG | Publication date | Utility of data for CPG |
| Expiration date of CPG | Update date for CPG | Flag alert to electronic chart and email alert to dentist |
| Functional status | Functionally independent or dependent, or frail | Functional status to which the CPG applies |
| Risk level | Low, equipoise, high | Risk level to which the CPG applies |
| Significance ranking | High, moderate, low stated both for statistical and clinical significance | How trusted the decision evidence is to the dentist |
| Utility ranking | High, moderate, low | How trusted the utility evidence is to the dentist |
| Meaning in practice | High, moderate, low | How predictable this CPG will be in terms of the patient's outcomes, behaviors, and compliance |
| Decision data measure | Stated in probabilities or odds ratios, as preferred by clinician, the likelihood of an outcome | Best understanding of how to communicate to the patient the likelihood of the outcome based on the "average patient" |
| Utility data measure | Stated ranking of the preferences indicated by the "average patient" in the satisfaction obtained by completing a specific intervention | Best understanding of how to communicate to the patient the satisfaction of going through such a procedure as demonstrated by the "average patient" |
| Cost measure | Cost of a specific intervention | How much the dentist charges for a specific procedure; cost usually inputted to the form from the cost schedule of the practice. The clinician has the option to alter if so chooses and change documented in chart |

### 2.3.2 Probability–Utility Model

The probability–utility model provides derived evidence in the form of decision analyses that facilitate shared decision-making. Thus, the model not only defines inputs and outputs but also the basis for why the central database and CPG product exist. It also provides best evidence modified by implementation in private practice, decision analyses for use in updating the evidence within the central database, and analysis of best evidence performance, or its meaning in practice, to researchers and clinicians.

The decision analysis presents evidence in various ways for visualization and understanding of the CPG. Firstly, the CPG is offered with annotated descriptions of the evidence shown: Fig. 2.4 annotations describe the various chewing function effectiveness outcomes. Figure 2.5 annotates the utilities and Fig. 2.6 the costs associated with each treatment option.

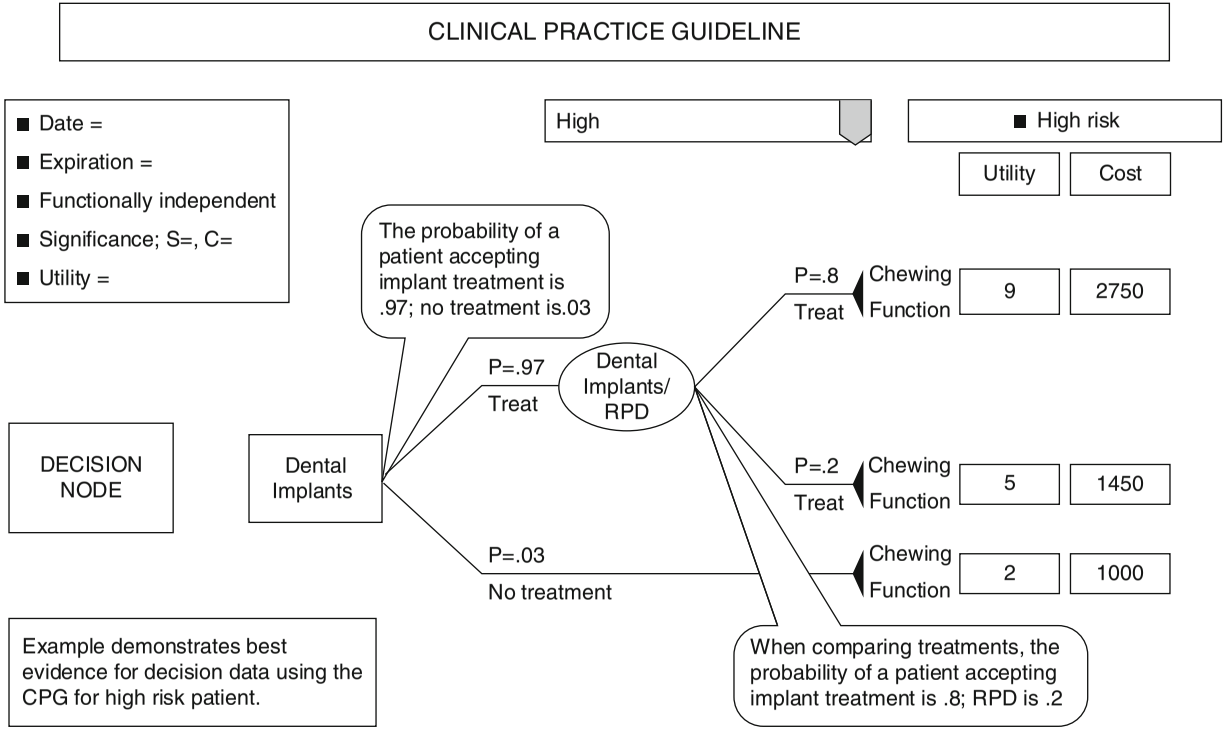



**Fig. 2.4** CPG annotating the effectiveness of chewing function associated with each treatment option

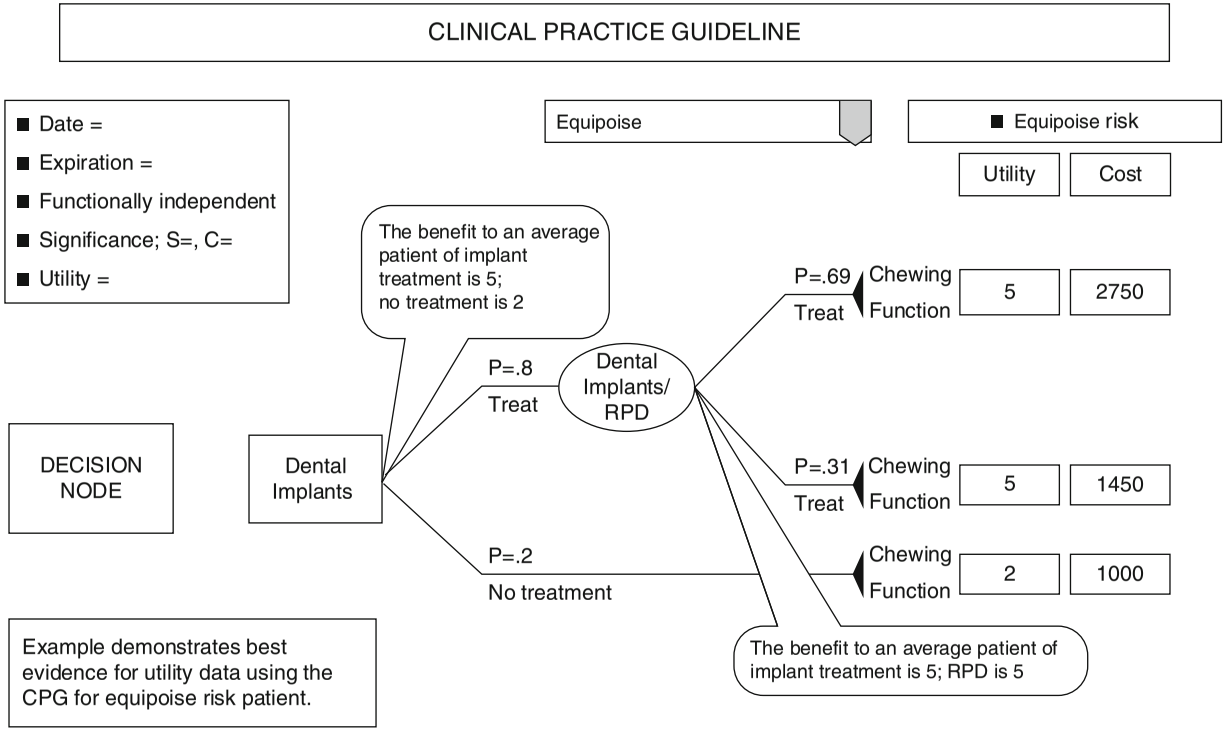



**Fig. 2.5** CPG annotating the utility of chewing function associated with each treatment option

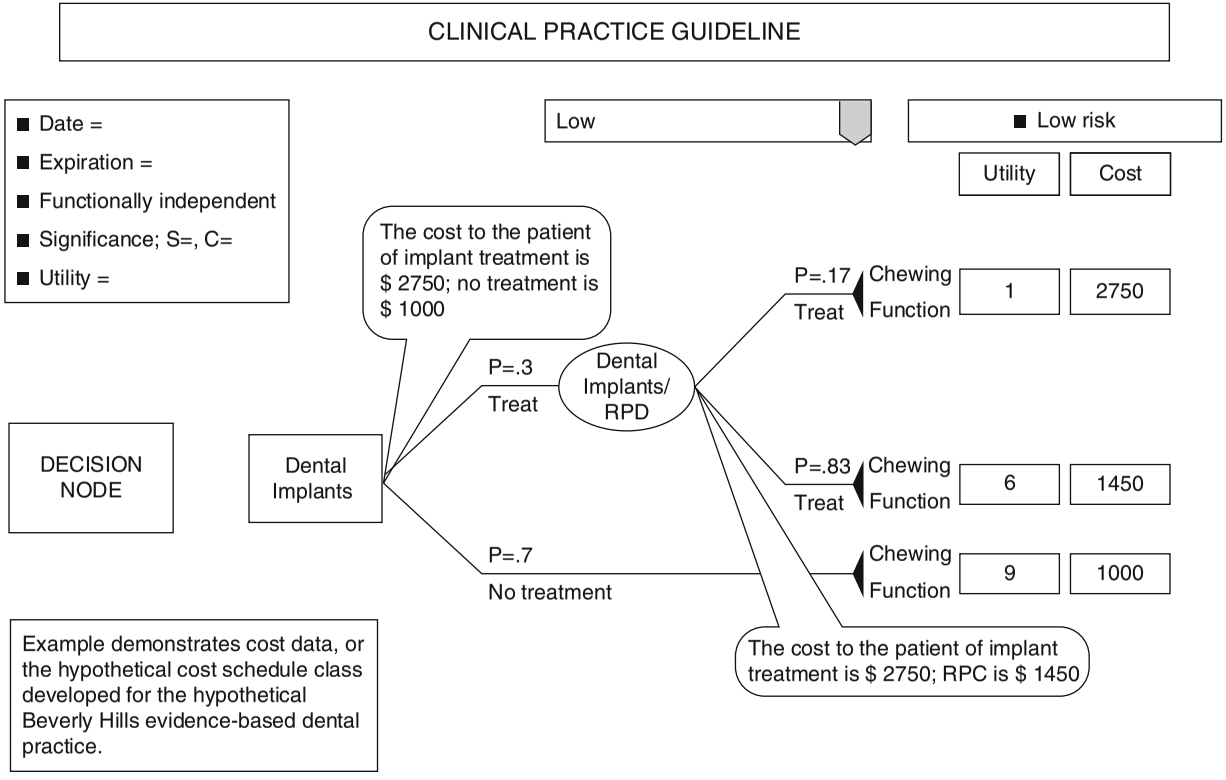



**Fig. 2.6** CPG annotating the cost associated with each treatment option

**Probability-Utility Model**
**Interpretation of Analyses**

| DECISION ANALYSIS-HIGH RISK TAKER | | |
|---|---|---|
| Decision Evidence | Utility Evidence | Cost Evidence |
| Decision:<br>* Rollback calculation for dental implant (.8×.97) =.77<br><br>* Rollback calculation for RPD (.2×.97) =.19<br><br>* Rollback calculation, No Tx = .03 | Utility:<br>* Rollback calculation for dental implant (9×.8) = 7.2<br><br>* Rollback calculation for RPD (5×.2) = 1<br><br>* Rollback calculation, No Tx (2×.03) = .06 | Cost:<br>* Rollback calculation for dental implant ( $2750 ×.8) = $2200<br><br>* Rollback calculation for RPD ($1450×.2) = $290<br><br>* Rollback calculation, No Tx ($1000×.03) = $30 |

**Fig. 2.7** Rollback calculations used in the decision analysis

The probability–utility model provides the trade-offs in considering which treatment option optimizes the clinical decision for the patient. This is done through roll-back calculations. Since the CPG is read from outcome to decision, so proceed the calculations.

To calculate the utility trade-offs between treatment options, the utility ranking is multiplied by the probability of the outcome. To calculate the cost trade-offs, the cost is multiplied by the probability of the outcome. Both provide dentist and patient with a description of the trade-off options for choosing the optimum clinical decision (Fig. 2.7).

In this clinical scenario, comparisons of the stated treatment options in an average women, 85 years and older, functionally independent, and interested in state-of-the-art treatment, dental implants offer this patient better chewing effectiveness far exceeding that provided by a removable partial denture (RPD) or no treatment. With no treatment, there are still costs.

If no treatment is selected, then the patient may experience costs in other or potential treatments due to not replacing teeth. These treatments may include orthodontics, extraction of teeth, or periodontal conditions that may arise due to malocclusions. Using satisfaction rankings that an "average patient" with the same characteristics as this patient, dental implants far exceed that of wearing and eating with an RPD or no treatment.

The cost of a dental implant compared to an RPD far exceeds that for an RPD or no treatment. With the options and their trade-offs, the dentist may discuss with the patient the option that best serves their needs, desires, and wants by comparing effectiveness, satisfaction levels, and cost. Thus, the optimal clinical decision is one that either accepts the option with the lowest costs or the largest expected value.

If the patient, on the other hand, does not agree with the utility rankings, then the patient's rankings may replace those of the "average patient" with immediate revision of all trade-offs of each option having been recalculated. The interpretation of

INTERPRETATION BASED ON RISK LEVEL

| WHAT DOES THIS MEAN TO ME THE AVERAGE PATIENT | |
|---|---|
| Patient Risk Taker Category | Clinical Decision |
| High | I get the greatest chewing function (effectiveness) that meets my highest expectations (efficiency) with implant treatment over all other choices (trade-offs). While this treatment costs the most, its effectiveness and efficiency to my oral health is worth it. |
| Equipoise | I get the best choice with RPD treatment. It provides me a reasonable increase in chewing function and is just as efficacious a treatment as that for implant. The cost is certainly much better and the best trade-off to no treatment. I can be satisfied with my choice because no treatment is the least acceptable and cost about half as much as RPD treatment. In addition, RPD treatment is certainly cheaper than implant treatment while giving me the same satisfaction. |
| Low | I get the best of chewing function and satisfaction with RPD treatment. While it still costs me more, it is not enough for me to accept no treatment. If I loose my job, I know that no treatment is going to cost me later on and I will not get as much chewing function or satisfaction as I would if I accepted RPD treatment. While the dentist's profit margin and expectation of chewing effectiveness is higher with implant treatment, RPD treatment does beat not treatment in a trade-off with no treatment. |

**Fig. 2.8** Interpretation of the decision analysis

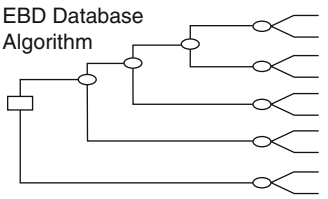
the analyses appends the CPG so that viewing of CPG along with the interpretation may be seen simultaneously. Figure 2.8 provides optimal clinical decisions based on the preferred risk level of the patient.

## 2.4 Revision of Best Evidence

Modification of decision, best evidence within the central, evidence-based database may be performed simultaneously by updating the decision analysis. This revision of best evidence is done using Bayesian statistics. Updating best evidence determines if the prior probabilities of treatment options are mitigated by probabilities or revised probabilities of other events.

In other words, the prior probabilities of events are conditional on probabilities based on observed outcomes witnessed in private practice. The observed evidence is updated through the electronic chart using the COG. This analysis is performed using Bayes' theorem. Bayes' theorem is a formula for calculating the conditional probability of one event from the conditional probability of another event. For example, a CPG provides treatment options for a disease event as treatment or no treatment (Figs. 2.9 and 2.10).

The probability that the treatment will again be accepted (New pA) is the product of the probability of acceptance with the presence of the disease (pAdisease) and the




**BAYE'S THEOREM**
Baye's theorem is a formula for calculating the conditional probability of events from the conditional probabilities of other events.

$$\text{New } p\text{A} = \frac{p\text{Adisease} \times \text{Prior } p\text{A}}{(p\text{Adisease} \times \text{Prior } p\text{A}) + (\text{Prior } p\text{Adisease} \times \text{Prior } p\text{D})}$$

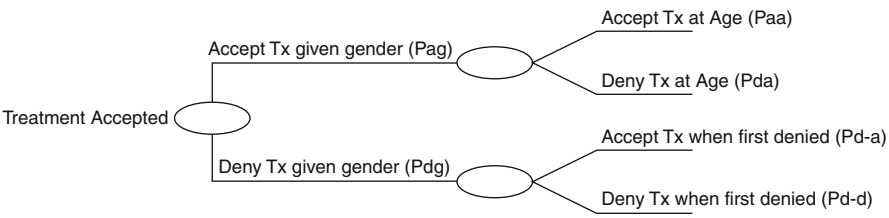
OR

$$\text{New } p\text{A} = \frac{p\text{Adisease} \times \text{Prior } p\text{A}}{(p\text{Adisease} \times \text{Prior } p\text{A}) + (1 - \text{Prior } p\text{Adisease})}$$

TERMS
P = Probability
pAcceptance = pA
pDeny = pD
pAcceptance with disease present = pAdisease

**Fig. 2.9** Bayes' theorem

## Bayesian Statistic for Decision-Making




PROBABILITY THAT TREATMENT IS ACCEPTED IS CONDITIONAL ON:

**NUMERATOR**: The fact that treatment was accepted when factoring Age (Prior probability that treatment was accepted given Age, Paa) times the probability treatment was accepted when factoring gender (Probability that treatment was accepted given gender, Pag)

**DIVIDED**

**DENOMINATOR**: (Paa x Pag) plus (1 minus the probability that treatment was accepted, factoring age, when it was first denied, Pd-a)

**Fig. 2.10** Bayesian statistic for decision-making

prior probability of acceptance (Prior pA). This product is divided by the sum of two products:

(1) The first is the product of the probability of acceptance with the presence of disease (pAdisease) and the prior probability of acceptance (Prior pA).
(2) The second product is the probability of denying treatment with the presence of disease (pDdisease) and the prior probability of denying treatment (Prior pD).

Aside from updating probabilities, patient's decision-making and new knowledge change the understanding of evidence. With sensitivity analysis, the translational researcher evaluates for clinicians and researchers patterns that characterizes changes in decision-making.

These changes occur as a function of changes in probabilities, utilities, and costs. Sensitivity analysis also predicts the type of evidence that was most relevant to patients when considering trade-offs between two or more presenting treatment options. This information serves to improve understanding of the differences observed in patient preferences and values, behaviors, and compliance.

## 2.5 Conclusion

### 2.5.1 The Synergistic Effect of Probability–Utility Model

The probability–utility model is synergistic with the translational evidence mechanism. It is the technology that provides clinicians and patients with an organized presentation of best evidence in calculating trade-offs or determining cost/benefit treatment options during shared decision-making. Similarly, the process provides active participation and the venue for quantifying and qualifying informed consent in reaching a negotiated, optimized patient-determined clinical decision during treatment planning.

Additionally, the model quantifies and qualifies changes in best evidence based on individual patient outcomes, behaviors, and compliance. These changes are returned to the central database for analysis. The analyses are used by translational researchers to update best evidence vested within the central database using Bayesian statistics. Other uses include dissemination of trends associated with changes or differences in components of the CPG.

These trends convey to clinicians an understanding of best evidence and its usefulness to patients when negotiating treatment options through the CPG. Lastly, the model is the dynamic by which best evidence is manipulated to serve the public, policy makers, and researchers in understanding new knowledge and technological advances. This understanding extends to servicing patient changes in patient needs, wants, and desires for improvement of health as well as the commitment on the part of patients to act on choices once expressed.

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# Part II

# Implication for Endodontics

# 3 Clinically Relevant Complex Systematic Reviews in Endodontics: Relevance to Comparative Effectiveness Research and Evidence-Based Clinical Decision-Making

Francesco Chiappelli, Manisha H. Ramchandani, Linda Phi, and Xenia Maria Caldeira Brant

**Core Message**

This chapter discusses the procedural issues associated with the performance, analysis, and interpretation of complex systematic reviews, specifically in the context of clinical relevance. Complex systematic reviews are instruments that derive from the combinatorial process of several homogeneous systematic reviews, and in that respect pertain to a higher level of research synthesis.

## 3.1 Introduction: The Clinically Relevant Complex Systematic Review: A Novel Study Design of Research Metasynthesis

### 3.1.1 The Systematic Review: An Unfortunate Misnomer

Stated succinctly, the purpose of the scientific field of research synthesis is to search, coalesce, and synthesize the best research evidence that is presently available and that directly pertains to a given scientific question under study, in order to make it immediately available and accessible to the interested readership [1–5]. As discussed below, the process is complex and intricate, and involves a judicious examination of current and past evidence.

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As the field of research synthesis has evolved over the past decades, a distinct path, a specific set of steps, a commonly accepted process has been outlined and designed to ensure the validity and the reliability of all research synthesis protocols, across subjects and research groups.

The systematic nature of these guidelines for research synthesis endeavors follows the scientific process and proffers a specific object-directed and stepwise progression of *sine qua non* activities, in the absence of which the outcomes of the research synthesis process are uninformative and of limited use.

These activities, which are examined in some details in this chapter, instruct and inform about how the current and past evidence was systematically obtained, evaluated, and synthesized. The keyword, "systematic," emphasizes and ensures, as noted, the validity and the reliability of the inquiry.

Research synthesis pertains to the process of pooling together all of the available current and past evidence about a given scientific query [1–5]. Three methods are available for that purpose:

- Published literature – The published literature refers to scientific reports published in peer-reviewed scientific journals and which can be retrieved through the National Library of Medicine (Medline, pubmed) and other search engines of similar scientific standing.
- Unpublished literature – The unpublished literature refers to what is often termed the "gray literature," that is scientific reports that are published in non-peered reviewed journals and which are available through nonscientific search engines (e.g., Google). Other examples of "gray literature" include master's degree and doctoral degree theses and dissertations, which are available in university libraries in the form of either full-text hard copies or microfilms.
- Unrecorded observations – Unrecorded observations refer to data recorded in the personal notes of individual investigators and that may be communicated to colleagues as "personal communications," but that are not compiled into print either in a peer-reviewed or non-peer-reviewed report.

In an exhaustive research synthesis project, all of the available current and past evidence, in the form of published literature (peer-reviewed), unpublished "gray" literature (non-peer-reviewed), and unrecorded observations will be included for review and synthesis. The keyword, "review," ensures that the totality of the available evidence undergoes critical evaluation and assessment of the strength of the presented evidence.

In brief, the term "systematic review" was coined to describe the product of an all-encompassing and systematically critical research synthesis investigation [1–5].

It could be argued that, whereas the intent is laudable, the terminology is inadequate and misleading. In the scientific literature in general, and the health sciences in particular, the term "review" is generally associated with a literature review: that is a comprehensive paper that may be written either for the scientific community, and in this case that is most often peer-reviewed, or the lay community, in which case it is most often non-peer-reviewed.

Literature reviews, while they do, optimally, rest on a clear writing style, do not require crafting along a stringent and systematic protocol. Literature reviews in science present a body of text that aim to describe the critical points of current

knowledge on a particular topic. They do not include research methods, although they may discuss and compare methodological approaches.

Literature reviews are considered "secondary sources" of scientific information because they do not generally report new or original experimental work. A compilation of literature reviews may come to form the core content of "tertiary" scientific communications: reviews of reviews, such as those published in prestigious peer-reviewed organisms (e.g., Annual Reviews).

The product of a research synthesis research investigation is primary scientific information that arises from systematically following the scientific process toward the elucidation of carefully crafted research question, by means of an appropriate sampling process of the subject of inquiry, careful assessment by means of reliable and valid tools of measurement, skillful statistical analysis of the results, and cogent inference and data interpretation.

The product of a research synthesis research investigation is a research paper *in se* and *per se*,[1] a piece of primary and novel scientific knowledge that ought not be misconstrued as a "review."

Nor is the term "systematic" informative here since all scientific pursuits from the earliest times are "systematic" in nature. To label the product of a research synthesis research investigation as "systematic" yields no added informational value as to what it is.

Granted, some will argue that the term "systematic review" is meant to be a compounded name, which specifically describes the nature of the product of a research synthesis research investigation. That might be so, although it must be remarked that such product is, as noted, neither a review nor uniquely systematic in the universe of scientific writing.

Consequently, the term "systematic review" is an unfortunate misnomer, which creates confusion among scientists not fully aware of the purpose and inherent power of the science of research synthesis.

For lack of a better term, and because of its increasingly widespread use, we continue its usage in the chapters in this volume. However, it may behoove the field to consider the following: peer-reviewed scientific publications that report fundamental primary research in molecular biology are often called "molecular biology papers," similar publications that report, say, novel primary research findings in immunology are often termed "immunology papers," publications that do the same in the field of, say, psychology are recognized as "psychology papers," etc.

They all report new research findings systematically derived through the scientific process, appropriately analyzed statistically, and carefully crafted to integrate

[1] Cf., Baruch Spinoza (1632–1677): a "thing" can be defined *in se* and *per se* – *in se*, meaning that its totality effectively defines what it is, and *per se*, indicating that it actually can define itself as the concept of itself. That is to say, the product of a research synthesis endeavor is, in its totality, a fundamental primary research product, and it so defines its own essence because it follows the scientific method, the body of scientific techniques we concur to utilize in the pursuit of investigating phenomena, acquiring and creating new knowledge, and of correcting and integrating previous knowledge, as originally crafted by Aristotle (384 A.C.–322 A.C.), and which is the foundation of modern science.

the novel knowledge into a review of the pertinent body of existing science. Therefore, it may be time to abandon the use of the term "systematic review" to refer to the product of a research synthesis research investigation, and replace it instead with the more correct and precise term of "research synthesis paper."

### 3.1.2 From Research Synthesis to Research Metasynthesis

The science of research synthesis is as complex and intricate as the science of psychology, immunology, or molecular biology. It is concerned with the principles specific to it, as every scientific realm is. To demean it turns out to be as nonsensical ignorance as demeaning any other of the biomedical sciences.[2]

Research synthesis follows the scientific method [1–5], which can be outlined in brief as follows:

- Statement of the hypothesis and research question
- Crafting of the research approach to test the hypothesis and answer to the research question (i.e., research design, sampling issues, tools of measurement)
- Presentation of the findings, and summary of the results by means of descriptive statistics
- Statistical analysis of the data
- Inferences, discussion of limitations and intervening variables, identification of future research toward further testing the hypothesis, and answering the research question in greater details

We discuss the role each of these steps of the scientific method play in the pursuit of a research synthesis investigation in the next section of this chapter. Before that, however, we must recognize that the subjects of study in a piece of research synthesis investigation are the elements of published and unpublished literature, and unrecorded observations discussed above.

When we speak of research synthesis being performed on the best available evidence, the term "available" underscores the fact that we limit the subjects of study in a piece of research synthesis investigation, in the same manner as any other piece of research, to the accessible sample: that is to say the accessible published and unpublished literature, and unrecorded observations that pertain specifically – i.e., that target – the question under study.

Whereas we will examine these issues of sampling in greater depth in the next section, it is important here to recall that the very essence of research synthesis calls for the evaluation and synthesis of the identified primary literature that pertains to the clinical question of interest.

[2] Some years ago, I had a dialectical discussion on science and philosophy with a humanist, world-renown for his ground-breaking work in linguistics and literature of the middle ages. His assertion that "biochemistry does not exist as a science" was as grounded on his ignorance of the fundamentals of the science of biochemistry, as the assertion of many that "research synthesis is not a science" rests on their lack of awareness of the rich complexity of the science of research synthesis.

As noted above, the "research synthesis paper" is still called, at present at least, a systematic review. The two fundamental properties of a systematic review are that it presents the primary research deemed "available" by the investigator at a given moment in time, and that it synthesizes this available evidence on certain criteria to render a consensus statement of the "best" evidence.

It should be self-evident that, as the body of scientific information grows, and because of differing criteria of establishing the quality of research reports (*vide infra*), the scientific literature is becoming replete with multiple systematic reviews that pertain to the same original clinical question, but that may differ in their conclusions.[3]

This observation leads to the realization that the science of research synthesis needs to grow, therefore, to include design and data analysis protocols for the synthesis not only of primary research report but also of multiple systematic reviews.

To draw a parallel, one might recall that several decades ago, the field of biology was relatively simpler than we conceptualize today. For example, we used to study systems largely independently for each other: we concerned ourselves with the endocrine system or the nervous system or the immune system, to mention only three.

As our understanding of the fundamental biological mechanisms grew, our knowledge evolved into the principal domains of systems biology. Among the first of those was probably the concept of neuroendocrinology, as the study of the interactions between the nervous system and the endocrine system by Geoffrey Harris and Berta Scharrer.

Of course, the melding of Ehrlich's antibody formation theory (1900) and Smith's discovery of the first monoclonal T cell population (1979) into the immune surveillance system that we comprehend today is another example of the growth of science beyond its original boundaries. Whereas Filipp, Szentivanyi, and Mess wrote the first recorded research report demonstrating the neural control of immunity through hypothalamic endocrine products in 1952 [6], and George Solomon coined the term "psychoneuroimmunology" in his seminal 1964 paper [7].

It was only three decades ago that Ader first established the field of study that characterizes the interplay and interdependence between the immune, the neuroendocrine, and the psychocognitive systems [8], which is recognized today as the science of psychoneuroendocrineimmunology, a metascience of sorts. The integration of psychoneuroimmunology in dentistry is even more recent [9].

The same evolution is bound to occur in the domain of the science of research synthesis, as the plethora of systematic reviews continues to mount. We must engage in the gargantuan task of establishing methodologies, designs, modes of statistical analysis, and appropriate inferential criteria for the process of synthesis of systematic reviews into "metasystematic reviews."

We need to go beyond the current protocols of research synthesis that pertain to primary research reports, and develop and validate new and effective procedures for

[3] This property is indeed not unlike what commonly occurs in the other health sciences: multiple reports by several groups of investigators may be discordant or concordant – hence the need for replicative studies, literature reviews, etc.

"research metasynthesis" for the evaluation of the best available evidence now existing in the form of systematic reviews.

That is not a new realization. It has been stated elsewhere. It was proposed that the term "complex systematic review" be used to designate the product of research metasynthesis [10]. Of course, and in line with the arguments presented above, it should be self-evident that this terminology of "complex systematic reviews" is as much of a misnomer as the previous, if not actually more confusing.[4]

Be that as it may, because this terminology has become widely used, and in an effort to focus the discourse on research metasynthesis as it pertains specifically to the pursuit of clinical efficacy and treatment effectiveness, we sought to clarify the domain of inquiry by specifying the objective of our pursuit as clinically relevant complex systematic reviews (CRCSRs) [11]. This is the remaining focus of this chapter, and of this book at large.

### 3.1.3 Fundamentals of a Novel Research Design in the Health Sciences

The design of a piece of research, the design – that is – of a study (i.e., the research design, the study design), encompasses and represents the very foundations of the research process to be undertaken. It is the inherent structure of study, the elements that harmonize and intertwine the individual components of the research endeavor into the *gestalt*,[5] the wholeness of the project.

The validity of the design ensures the validity of the research study. That is to say, the principal methodological requirements of any piece of investigation, from the sample to the tools of measurement, constitute the determinant criteria of the design that dictate the strength, power, and reliance of the findings.

Catastrophic failures of designs cannot be salvaged even by the most erudite statistical analysis. In fact, as Stuart Pocok cogently observed in his discussion of the group sequential analytical approach[6] in clinical trials [12], to propose that poor

[4] The term "complex systematic review" to indicate a research meta-analysis paper (i.e., a synthesis of systematic reviews) might suggest that a "systematic review," a research synthesis paper (i.e., a synthesis of primary research reports) is in some manner or another simple or simplistic, or at least not at all complex and complicated. This statement is misleading and far from the reality of things, and goes to reinforce the points about misconception, misinformation, misunderstanding, and frankly ignorance (cf., footnote 2) about the science of research synthesis and metasynthesis made above.

[5] Gestalt – from the German (Berlin School of Psychology, late nineteenth century), meaning the entirety of the essence or being of an entity's complete form; e.g., the mind, viewed as holistic, parallel, and analog, with self-organizing tendencies.

[6] Sequential statistical hypothesis testing is not bound by a predetermined sample. Rather, data are evaluated as they are collected. Sampling is aborted if statistical significance is obtained, based on criteria established at the onset of the study. In other words, the end of a study may be attained at a much earlier stage, with a smaller sample size, and at a lesser overall cost than if the traditional power analysis has estimated the required sample size prior to the onset of the study.

design can be corrected by subtle analysis techniques is untenable and contrary to good scientific thinking. A good and solid research design is fundamental to good science.

That said, the basic elements of research designs can be noted as follows [2]:

- Optimally, a "good" research design is characterized by having a fully formed, clearly stated, focused research question, which yields a single primary outcome measure.
- The research design should propose the groundwork and the foundation of an inquiry process that is feasible, in terms of manpower, time, supplies, and money expenditures.
- The study must be designed in such a manner that the pertinent information is recorded and measured on the appropriate subjects (cf., sampling and measurements concerns noted above) in a precise, reliable, and valid manner that avoids, minimizes, or altogether eliminates bias.
- Effort must strive to maintain the study design as simple as possible, because, if left unchecked, the complexities of research soon attain gigantesque enormity.
- John F. Kennedy once said that "the great enemy of the truth is very often not the lie – deliberate, contrived and dishonest – but the myth – persistent, persuasive and unrealistic." That is to underscore that a "good" research design is one that ensures that the study will generate new information, will create new knowledge, will produce data that are not only statistically significant, but relevant to society at large. That is to say, in the realm of the health sciences, the fundamental goal of research designs must be to produce results that are clinically relevant.

Two general types of research studies are commonly employed in health science research: diagnostic studies seek to characterize the mode of establishing the presence or the absence of a condition. Prognostic studies seek to follow up the course of a condition. Further subcategorizations distinguish, for example, observational prognostic studies (e.g., cohort, cross-sectional, case–control investigations) from interventional prognostic studies (e.g., experiments, quasi-experiments, clinical trials) [2].

Although this is by no means a new idea,[7] the notion has now become relatively widely accepted that that the results of a particular research study cannot be interpreted with any confidence unless they are considered systematically together with the results of other studies addressing the same or similar questions [1–5].

[7] Luc de Clapiers Vauvenarques (1715–47) stated in *Réflexions et Maximes* that *…il est plus aisé de dire des choses nouvelles que de concilier celles qui ont étés dites* (it is easier to say new things than to reconcile those things that have been said). John William Strutt, 3rd Baron and Lord of Rayleigh (1842–1919), said "The work which deserves, but I am afraid does not always receive, the most credit is that in which discovery and explanation go hand in hand, in which not only are new facts presented, but their relation to old ones is pointed out."

Therefore, we propose that the science of research synthesis constitutes a family[8] of research designs in its own right. It is in that light that we discuss the remainder of this chapter and of this book, as we explore the role and relevance of the product of the research synthesis design, the systematic review, and the product of the research metasynthesis design, the CRCSR to health care in general, and endodontic dentistry in particular.

## 3.2 The Scientific Process in Research Metasynthesis

### 3.2.1 From a Question to a Hypothesis Testing Paradigm

The process of research synthesis and of research metasynthesis must be one that produces valid and reliable results, worthy of publication, that meets with the approval of our peers in the peer-review system, and that contributes significantly to the clinical outcome of interest.

As for all research designs, the design of a study in research synthesis or metasynthesis must begin with a carefully crafted research question that clearly places in perspective and evidence the sample, the methodologies, and the clinical outcome sought.

The process of research synthesis and of research metasynthesis for the generation of systematic reviews, and of clinically relevant complex systematic reviews (CRCSR) respectively are the common research designs for comparative effectiveness and evidence-based decision-making. Stated succinctly, the comparative effectiveness process is one that seeks to meet the practical needs of stakeholders.

It addresses concerns ranging from cost/benefit ratios, to safety effectiveness of clinical interventions. By contrast, one could argue that the evidence-based process seeks to meet the clinical needs of stakeholders, and so doing it addresses concerns of treatment efficacy for the individual patient.[9]

[8] One could conceive of the following: the (simple) design of research synthesis yields the (simple) systematic review by pooling and evaluating primary research (e.g., clinical trials); the (complex) research synthesis, which we have called research *meta*synthesis, yields the complex systematic review, also termed CRCSR, and results from the pooling and evaluating existing systematic reviews. Yet, a compounded or *mixed-form* research synthesis would yield a clinically relevant *mixed* systematic review (CR*M*SR) and pool and evaluate together both existing systematic reviews *and* primary research (e.g., clinical trials) that have not yet been incorporated in any existing systematic review. CRMSR promise to be the most arduous and challenging of the research synthesis designs because they will coalesce two fundamentally divergent research entities: primary research reports (e.g., clinical trials) and secondary research reports (i.e., systematic reviews). Study validity issues will be particularly difficult to untangle in CRMSR. Special care must be given not only to the sampling and the measurement processes but as well as to the data analysis component. Case in point, the meta-analysis of a CRMSR will involve a notably greater level of difficulty than even the Bayesian meta-analytical inference required for CRCSR.

[9] *Vide infra*, Part III, 1 and III, 2.

Whereas comparative effectiveness queries pertain to the probabilities of success and cost, and of benefits and risks of given interventions within a given timeframe and setting, evidence-based queries address concern of treatment efficacy in a personalized, patient-centered for clinically relevant outcomes [5, 13].

In that light then, it becomes apparent that the fundamentals of the research synthesis and metasynthesis design are common to comparative effectiveness and evidence-based pursuit of the best available evidence for treatment [5, 13, 14], except for the clear emphasis that the former attributes to the timeline and settings of the intervention.

Consequently, the study question of research synthesis and metasynthesis designs must concretely specify the patient population, the interventions being considered, and the clinical outcomes under scrutiny. Hence, it is rendered by the acronym P.I.C.O. (patient, interventions under consideration, outcomes). The more specific nature of the comparative effectiveness question as it entertains as well timeline and settings considerations, engenders a more specific acronym from those studies: P.I.C.O.T.S. [15].

The scientific method directs research to be hypothesis-driven. In general terms, a hypothesis proposes an explanation for a given phenomenon or observation. More specifically to the point of research synthesis, the hypothesis pertains to the suggestion of a relationship or a set of relationships among phenomena or observations. Practically speaking, the hypothesis of a study most often simply restates the research question in an affirmative[10] format.

This new form explicates the methodologies that will serve to test the hypothesis – from sample to variables, from tools of measurement to data analysis issues, and from reliability concerns to validity and generalization.

Specifically in the context of research synthesis and metasynthesis designs, it is clear that the P.I.C.O. and P.I.C.O.T.S. research questions lead to specific hypotheses about which intervention under consideration may or may not be more effective or efficacious for the particular patient population targeted in the study, and in light of the specific clinical outcome of interest.

It also clear that the P.I.C.O. and P.I.C.O.T.S. questions drive the research synthesis and metasynthesis process in terms of the sample of publication to be scrutinized to obtain the available evidence, the tools of evaluations that serve to assess the best evidence, the statistical analysis required to establish reliability and validity of the results, and the inference of the findings for immediate implication to clinical practice, and for deductive reasoning for incremental progress of research in the future.

In brief, the method of science instructs that the creation of new knowledge that is obtained through research is driven by the scientific method. The scientific method consists of a series of sequential steps that arises from a theory, a hunch, or a simple observation. The scientific inquiry involves a translation of that initial characterization of experiences into a well-crafted research question, which then, when stated as a research hypothesis, sets the stage for systematic scientific inquiry [2].

The case of clinical observations is no different. As they are translated into either a P.I.C.O. or P.I.C.O.T.S. format, clinical queries become statements of research

[10] Rather than inquisitive.

hypotheses that drive the scientific process through a well-characterized set of methodological *sine qua non*s, which signify the very systematic scientific protocols of research synthesis and metasynthesis.

### 3.2.2 Sampling Issues

A well-stated P.I.C.O. and P.I.C.O.T.S. question will reveal embedded keywords for the literature of interest. These keywords can serve as medical subject headings (MeSH) that instruct the search engines in the identification of the sample.

That is to say, in research methodology parlance, it is clear that from the population of the available research literature (i.e., peer-reviewed, non-peer-reviewed, unpublished observations[11]) generally pertinent to the clinical query of interest, only a target sample will be obtained, which is defined specifically by the keywords that arise from the P.I.C.O. or P.I.C.O.T.S. questions.

When the sample of literature thus obtained is very small, a reconsideration of the P.I.C.O. or P.I.C.O.T.S. questions will be required to make them broader, and therefore encompassing a larger segment of the available research bibliome.[12] That is so, principally, because a research synthesis or metasynthesis protocol on a sample of literature that is less than 5 may lead to meaningless statistical analyses of the resulting data.[13]

By contrast, when the resulting sample of literature is very large, then inclusion and exclusion criteria can be utilized, in a manner akin to primary observational and experimental studies, to control and constrict the sample size. It is self-evident that it will behoove research synthesis and metasynthesis designs that follow a P.I.C.O. question, and that seek an evidence-based, patient-centered efficacy outcome to have clearly delineated inclusion/exclusion criteria so that the yielded best available evidence has the strongest likelihood of being directly pertinent to the patient case whence the question arose. It is also obvious that this restriction is less important when one pursues a comparative effectiveness line of inquiry.

It actually may occur that the sample of literature that is produced by the initial search remains too gargantuan, following and despite stringent inclusion and exclusion criteria. Then, a process of random sampling of the resulting literature subpopulation may be confidently entertained, and the research synthesis and metasynthesis designs conducted on the random sample thus obtained.[14]

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[11] *Vide supra*, Part I.

[12] The "bibliome" is the body of pertinent research literature available for any given systematic review [16].

[13] That is, and purely on statistical grounds, if, following acceptable sampling analysis and homogeneity analysis, the number of papers fed into a meta-analysis is less than, say, 2 or 3, one may wonder as to the validity of the generated forest plot.

[14] The process of random sampling can be expected to have exactly the same effects when one randomly samples literature in a research synthesis design, as when one obtains a random sample of subjects in an experimental design.

Of course, sampling in research synthesis and metasynthesis suffers from the same threats and limitations as the process of sampling in other research designs. For example, the threat of selection bias adulterates the sampling process in experimental studies when sampling is driven by convenience rather than by chance. Sampling of the literature suffers from selection bias, when, for instance, our evaluation capabilities (i.e., critical reading, assessment tools) fail to be all-inclusive.[15]

Moreover, a hidden selection bias, which we have discussed elsewhere, refers to the preferential print of statistically significant research, to the detriment of clinically relevant findings [2, 3, 5, 11, 17]. Thus, preferentially, the sample that results from the literature pertaining to a P.I.C.O. and P.I.C.O.T.S. question is primarily composed of statistically significant research, whether or not it presents clinical relevant observations.

That inherent characteristic of the available research literature causes a profound dilemma in the field, which has not found satisfactory resolution as of this date:

> if research synthesis and meta-synthesis designs seek to find the best available evidence in pursuit of a clearly stated clinical outcome, based on the P.I.C.O. or P.I.C.O.T.S. question, then does the fact that the sample of literature that is evaluated in this process preferentially reports and defends statistical, rather than clinical significance, de facto invalidates scientific research?… scientific research as a body of knowledge in its entirety. This dialectical conflict arises because the preferential inclusion of studies that report statistical, rather than clinical significance, in the pursuit of the best available evidence for a given clinical outcome, and the preferential reporting of only what fits certain criteria of one particular theory – in this case election bias as we understand and utilize it today – is a prime violation of the scientific process.[16]

Clearly, that is a query that pertains to contemporary philosophy of science, and that need not be pursued here. While aware of this fundamental dissonance, we must continue in our exploration of the fundamentals of clinically relevant complex systematic reviews (CRCSR) as the product of the research design of research metasynthesis, specifically as they are driven by a P.I.C.O.-initiated hypothesis for an evidence-based efficacy patient-centered decision, or a hypothesis that emerges from a P.I.C.O.T.S. question for a comparative effectiveness clinical decision.

## 3.3 Measurement Dialectic

### 3.3.1 What Is Dialectic?

Dialectic (Gr., διαλεκτική) is the branch of philosophy that concerns itself with the contrast of opposing propositions – usually two, presented as a "thesis" and an "antithesis" – through a balanced exchange of arguments. It is the art of dialogue

[15] Such selection barriers include language, search engine, and library availability, among others.

[16] That is, choosing and picking the scientific findings that we want to disseminate because they fit our preferred theory.

(Latin: *ars discutandi*) aimed at attaining a rational resolution of differences of through a thoughtful process of evaluation of each argument.

In the tradition of Socrates, Plato, Aristotle, Hegel, and others, the outcome of a dialectic exchange may be the refutation of the irrelevant point, or more commonly the synthesis of the opposing assertions into an improved view on the concept under discussion.

In contemporary philosophy of science,[17] the dialectical process is presented as an approach to addressing fundamental scientific problems. This approach, akin to the scientific process outlined above, sets specific questions and queries about research.

It proposes specific, widely agreed upon steps of scientific inquiry whose goal it is to protect against dogmatism and predetermined answers or opinions. The rightful place of the dialectical relationship in the scientific endeavor is to bring into full considerations all the "parts" that constitute a "whole" (or totality).

The same can be said here: as the science of research synthesis becomes increasingly better described, understood, and disseminated, it is critical and timely to bring forward all the "parts" that constitute the totality of the process of systematic reviews and complex systematic reviews.

It is necessary and important to have a constructive dialectic exchange of seemingly opposing assertions, not with the intent of refuting one or the other, but rather of reconciling them and synthesizing them into a new and improved proposition that enables the forward progress of the field.

No other aspect of the scientific domain of research synthesis and metasynthesis is more in need of this sort of dialectical "reconciliation" than the process of measurement. As stated above, the goal of research synthesis is to obtain the best research evidence pertaining to any given scientific question, and making it available and accessible.

At issue, therefore, lie the specific definition and the practical quantification of the term "best." What is the best research evidence, and how do we evaluate it in a quantifiable manner – that is the dialectical question.

Two contemporary schools of thought can be succinctly described as such: on the one hand, there are those who defend the original proposition that a ranking system can be arbitrarily devised to evaluate the strength of the results of a study purely on the basis of the nature of the design.

That system, which inevitably establishes one research design as superior and another as inferior, has evolved in a pictorial representation that is as ludicrous as it is useless, and in fact damaging to the pursuit of the best available evidence.

To represent a ranking system as a pyramid, which places clinical trials about the top and animal studies about the bottom, is to ignore two fundamental facts of research methodology: firstly, animal studies are a *sine qua non* to clinical trials – every and any intervention clinical trial on a group of patient cannot be initiated unless the proper safety and toxicity studies have been run on animal models.

[17] Cf., Levins R, Lewotin R. *The Dialectical Biologist*. Harvard University Press, 1985.

Clinical trials encompass in fact a family of research protocols that begin with fundamental mechanistic studies on human materials,[18] continue on testing with animal subjects, and, only when deemed safe, are tested for efficacy and for effectiveness with human normal subjects first, only then is a sample of patients tested (clinical trial, phase III), and ultimately with a larger group of patients across study centers (phase IV).

The Level of Evidence pyramid simply ignores that and assigns a rank close to the best to any study that tests an intervention on patients. The very top level of the pyramid is given to the systematic reviews, perhaps because early on in the establishment of research synthesis in evidence-based and comparative effectiveness research, it was presumed that systematic reviews in the health sciences ought to incorporate clinical trials exclusively.

Case in point, the consolidated standards of reporting trials (CONSORT) [2, 17], which, as the Pyramid itself, suffers from being qualitative, served as the foundation for A. Jadad and his group in the feeble attempt to provide some type of quantification of these measures.

The Jadad scale [2, 18], though widely used, is a weak psychometric instrument because it is not reliable (i.e., unsatisfactory interrater and intrarater reliability, unsatisfactory Cohen κ coefficient of agreement) and frankly unsatisfactory in terms of construct or content validity.

Nevertheless, proponents of the assessment of the Level of Evidence to establish the "best available" evidence continue to use the Level of the Evidence Pyramid and the Jadad scale, rather oblivious of the fact that the science of research synthesis, as all the sciences, continues to evolve.

So now, as we deal with research meta-analysis, the evaluation and combinatorial synthesis of systematic reviews, is it fair to ask where does that stand on the pyramid? Are we to construct an obelisk on top of the pyramid such that clinically relevant complex systematic reviews will stand erect above systematic reviews? Nonsense, indeed, as all ranking artifices are!

We must realize that, as the field evolves, so does our understanding of the complexities of the fundamental process of answering the P.I.C.O. or P.I.C.O.T.S. question. It is now clear that, in many instances – actually, this is particularly true in dentistry in general and in endodontics in particular – clinical trials are often impractical, if not impossible to conduct. The research designs of choice for primary studies are then observational, rather than experimental.

As such, they are ranked lower in the Level of Evidence Pyramid, and cases are on the rise as of late of systematic reviews in dentistry that report no noteworthy overall evidence simply on the grounds that the primary literature consists of observational studies, a lower level of evidence. This type of blank statement based on an artificial and uninformative concept such as the Level of Evidence is simplistic and evidently obstructionistic to the forward evolution of the multifaceted domain of clinical dentistry [3, 11] and evidence-based endodontics.

[18] That is why, even at that very early stage, the National Institutes of Health refers to this research as "clinical research."

Research in the health sciences utilizes all the possible and available study designs. The choice of research designs must not be dictated by the misconception that some designs are better than others. The choice of a design is driven purely by research methodology issues and concerns, and reflects the optimal methodological approach to obtain a reliable and valid quantifiable answer to the research question in a manner that can withstand the rigors of statistical analysis and generate clinically relevant new knowledge. That is the call of the scientific method.

The "best available" evidence is that research evidence that best achieves this. It is not what stands atop a pyramid. The "best available" evidence is that which emerges from a research methodology, design, and data analysis that answers the research question and tests the hypothesis in a scientific approach that is the most sound possible, considering all the limitations, intervening variables, and other possible confounders.

The second school of thought about how to obtain the best available evidence proposes that we must assess the quality of the evidence, rather than the ranking of the research design used to generate that evidence. It is not so much "what" design was used, but rather "how" the research was conducted, including all pertinent issues of design, methodology (e.g., sampling, measurement), and data analysis.

Increasingly, systematic reviews address the concern of the quality of the evidence. Usually, that is done by means of a checklist of some sort; sometimes it is obtained by means of a quantifiable instrument. Most often, it is an in-house tool developed *ad hoc* and only briefly described in the methods section of the systematic review. We are encouraged to note that increasingly systematic reviews utilize well-constructed, psychometrically tested (i.e., for reliability and validity) instruments that generate continuous (or semicontinuous) score measurements that span over research quality issues [2, 5, 13, 14, 19].

We have contributed to that branch of the science of research synthesis with the characterization of the Wong scale [20], a simple instrument that quantifies the overall quality of any type of research paper (i.e., clinical trial, observational design) along the three fundamental dimensions of methodology (i.e., sampling, measurement, significance, clinical relevance), design, and statistical analysis by means of the process of systematic evaluation of the statistical analysis (SESTA), which we characterized. The Wong scale was characterized for reliability and validity, and was recently revised (R-Wong) [21].

An adaptation of the R-Wong scale for use in animal studies and fundamental experimental bench research is now being finalized (cf., Oluwadara-Kossan, OK scale).

The R-Wong and the OK scales are instruments that permit the reliable and valid assessment of the quality of the research evidence, along the widely accepted criteria of research excellence for research methodology, design, and statistical analysis.

The advantage of these scales is that they quantify the quality of the evidence, such that each research report under investigation, be it a peer-reviewed or non-peer-reviewed publication, or a unpublished observation, receives an overall score (not a rank!). Furthermore, since each domain of these instruments pertain to research methodology, design, and statistical analysis, subanalyses of the scores for

each of the questions lead to the assessment of the relative strength or weakness of the bibliome sample under study with respect to these three cardinal foundations of research.

That is to say, the R-Wong and OK scales generate not only an overall score for each report, corresponding to the overall quality of the evidence it presents, but also provides information about relative strength, *lacunae*, and deficiencies inherent to the body of report under examination for answering a given P.I.C.O. or P.I.C.O.T.S. question [2].

The question then arises as to how to assess the quality of set of existing systematic reviews when performing a research metasynthesis for the purpose of generating a CRCSR. There have been extensive efforts over the past decade to establish and to validate instruments that would reliably assess the quality of systematic reviews. The endeavor is complicated by the multidimensional nature and the very intrinsic complexities of systematic reviews. A measurement tool for the assessment of multiple systematic reviews (AMSTAR) was developed by pooling the better elements of first generation instruments [22, 23].

While the AMSTAR proved to be useful when tested preliminarily, it also evinced a fundamental flaw: that is, the qualitative and descriptive (and often inexplicably enigmatic) nature of the assessments it produced. We recently revised the AMSTAR (R-AMSTAR) so as to make it quantifiable and interpretable in a manner similar to the R-Wong and OK scales, and we demonstrated its reliability and validity [16].

Whether or not R-AMSTAR will prove to be sufficiently powerful to quantify the assessment and evaluation of CRCSR remains to be tested. It is too early in the process of development of this branch of science to address this question in a meaningful and pragmatic manner because there are simply too few existing CRCSR to operationalize fully a process of quality assessment for these instruments.

### 3.3.2 Data Analysis Concerns

It is customary to think of research synthesis and meta-analysis as one and the same. This is an error. It is as if the painter thought of the canvas and the color blue as one and the same. The canvas is the structure, if you will, that the painter utilizes to obtain, onto which the painter will generate and obtain the painting. The color blue is that which the painter will utilize judiciously to obtain one specific portion of the painting: the sky, the sea, and the eyes of the person in the portrait. There may actually be paintings that do not have any blue hues.

In the same manner, research synthesis is the structure by which the investigator obtains the systematic review. The meta-analysis is one of the protocols that the investigator will utilize judiciously to obtain one specific aspect of the systematic review. There may, actually, be instances where a meta-analysis is not needed or impossible to conduct in a given systematic review. That, in and of itself, does not diminish the value of the systematic review product and the strength of the evidence it presents [4, 9, 11].

Meta-analysis is a complex statistical protocol, which was recently reviewed elsewhere in the context of systematic reviews in dentistry and medicine [24]. Suffice to say that, since it is a combinatorial process of analysis, it is extraordinary sensitive to several properties of the data. The two principal properties that deserve mention in the context of this discussion are homogeneity and quality.

To be clearer, let us present an example: if a systematic review on root canal looks for quality of life of the patient as the outcome in the P.I.C.O. question, reports might be found that quantify "quality of life of the patient" after the intervention as the absence of pain. Other studies might quantify "quality of life of the patient" as measurement of stress reduction. Others yet might measure the outcome as the number of months before a return visit to the endodontist with complaint on the same of a neighboring tooth.

The point is, clinical outcomes, whereas they may seem to clear and crisp measurable entities, more often than not can be quantified in more than one way. The heterogeneity in outcome measure is one clear danger for the validity of any meta-analytical reasoning: What, really, are we combining together? What really are we making overall inferences about?

There are statistical tests[19] that we must run on the outcome measurements that establish whether or not homogeneity is verified – that is to say, whether or not the extent of outcome measure heterogeneity is within the level of confidence, and is, in fact, not statistically significant.

It is also important that the data pooled together into a meta-analysis be from reports that are deemed of good quality. If an endodontist performs two root canals on the same patient on the same day and does a superb job in both teeth, expectations are that the patient will be satisfied. If the endodontist performs a terrible job on both teeth, it is fair to expect that the patient will be displeased. If the endodontist performs an excellent job on one tooth, but the second root canal is not well done, it is probable and even likely that the patient will be irate just the same.

The same is true in a meta-analysis: if the data in the input are all of high quality, then the variability due to residual inexplicable error will be small, and the effect, if there is one, will be apparent and clearly statistically significant. If, on the other hand, the data that are used in the meta-analysis originate from studies that are fraught with serious quality issues, then each of these sets of data will carry into the meta-analysis its contribution of residual inexplicable error, and the total overall variability will be large and negate the ability of a statistically significant overall effect to become apparent over this residual error "noise."

Similarly, albeit not as dramatically, if a meta-analysis should incorporate some solid and good studies and a few studies with serious quality issues, the contribution of the former to the variability due to residual inexplicable error will be small, but the contribution of the latter to the overall error will be disproportionately large. That will, more often than not, mask a statistically significant overall effect.

[19] Cf., Cochran Q, and its transformation as the $I^2$ test.

For that reason, many[20] investigators argue in favor of a two process of data analysis for systematic reviews.

- Firstly, establish the quality of the research evidence (e.g., R-Wong, OK Scale, R-AMSTAR), and based on these assessment, eliminate the studies that demonstrate excessive flaws, as determined by the score of the quality of evidence assessment tools (i.e., acceptable sampling analysis).
- Secondly, for the studies that remain, test for homogeneity, and if no significant heterogeneity is noted with the accepted studies, then run the meta-analysis.

The forest plot thus generated has the best likelihood of evincing overall significance, if there is one to be shown. Stated in statistical terms, it is necessary to perform both acceptable sampling and homogeneity analyses in order to ensure the power of a meta-analysis.

The question then arises as to what might be the recommended statistical approach to follow when performing a CRCSR. In fact, the answer is simpler that one may anticipate: a CRCSR is a synthesis of several systematic reviews. Therefore, the first step must be to assess the research quality of each one of these. That is obtained, as noted above, by means of the R-AMSTAR. An acceptable sampling step identical to that just described will need to be performed.

The systematic reviews deemed of a quality sufficiently high to be utilized in the CRCSR must be tested for homogeneity for the same reason, and in the same manner that was described in the preceding paragraphs. If homogeneity is established, then meta-analysis will be permissible.

However, our current conceptualization of the meta-analytical protocol pertains to coalescing data obtained from primary studies (e.g., clinical trials), not secondary studies (i.e., systematic reviews) that themselves present their own individual meta-analyses. The currently available softwares for meta-analysis do not perform a meta-analysis of meta-analytical data.

Attempts have been made to generate "cumulative meta-analyses," which are the product of performing a new meta-analysis every time a new piece of evidence emerges [25–27]. While attractive for its simplicity, this approach has raised caveats in the literature [28], because it appears to be incongruent with statistical theory on several grounds.

For example, the suggested approach implies repeated analytical testing of data set (n), as the data set grows to include the new piece of evidence ($n+1$). As stated, the principles do not proffer any limit to these repeated testing events, which seem at *prima facie* to incorporate the same bias[21] one finds upon performing repeated t tests.

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[20] Not all, it must be emphasized. There exists a school of thought that argues in favor of including all – bad and good – studies in a meta-analysis, akin perhaps to including all – good and bad – materials in the construction of a skyscraper. Should we be surprised if the foundations eventually give, and the edifice crumbles? Should we be surprised if a high proportion of meta-analyses conducted in this manner is likely to evince no statistical significance overall?

[21] In the case of repeated *t* test, this bias lends the analysis increasingly less powerful, by "chipping away" from the level of significance, $\alpha$, as follows: $p(\text{Type I error}) = 1-(1-\alpha)^c$, thus when $c=1$ (one comparison), then $p=1-(1-0.05)^1=1-(0.95)^1=0.05$; but if we were to perform three "cumulative" *t* tests, then $c=3$, and $p=1-(0.95)^3=0.14$.

Further exploration of the theoretical tenets that impinge upon cumulative metasynthesis is urgently needed, lest cumulative meta-analyses accumulate in the literature needlessly.

We argue that it is necessary to step outside of the traditional mold of statistical reasoning in order to uncover an approach that might allow a process of adding a meta-analysis to another existing meta-analysis, provided of course and always that quality and homogeneity have been ascertained.

The Bayesian reasoning, that the knowledge of a whole is attained by means of adding new sets of observations to existing ones (i.e., "priors"), provides precisely the theoretical framework that is needed to conceptualize how a meta-analysis in a CRCSR may obtain from the sequential adding on of the individual meta-analyses from each of the systematic reviews coming to form part of the CRCSR.

Whereas the actual mathematical structure of that process still remains to be finalized, the theoretical conceptualization of the approach for the overarching analysis of data in the process of research metasynthesis will undoubtedly have to be Bayesian in nature.

### 3.3.3 Interpretation and Inference

The question that researchers ask pertains to whether statistically significant differences obtain. This view of the world, as it were, has engendered a fundamental bias of selection of what type of research literature is published in the peer-reviewed domain [2, 29].

It is a view of the world that is fundamentally detrimental to the clinical arena because an effect that is clinically relevant often is not found to be statistically significant. And when that happens, it rarely, if ever, is published in notable form.

Systematic reviews and CSCSR consider the best research and synthesize the findings by means of the purely statistical process of meta-analysis. Then, somehow, forest plot summary data and confidence intervals, which are coalesced and analyzed group data, are transformed by means of the magical – it may seem – process of interpretation and inference into clinical relevance.

More often than not, this complex and convoluted process of translation of data summaries, tables and graphs, of p values, odds ratios, and effect sizes takes several people – each experts in their own field (e.g., statisticians, clinicians, researchers, and sometimes even patient representatives) – and lots of time.

This translation process is complete when some form of consensus among the participating parties has been obtained and generated. Hence the term "consensus statement," which usually is the final and bottom-line statement of the systematic review and of the CRCSR.

The consensus statement must be clear statement of the clinical implication and relevance of the research synthesis and metasynthesis. It must present clearly stated recommendations that the clinician must find practical and realistic updates of current clinical practice guidelines.

It is an emerging recognition in the field that these and related qualities of the clinical recommendations of a systematic review and of a CRCSR are the key strength factor of the entire process, as it pertains to personalize evidence-based treatment intervention, as well as comparative effectiveness analysis [2, 4, 13].

The strength of the clinical recommendation thusly conceptualized may actually be quantifiable.[22] If it were, and if the tool to obtain this quantification were in fact valid and reliable, then one final analysis of a systematic review and a CRCSR could involve assessing the quality of the consensus statement in terms of the strength of the clinical recommendation.

Work along that direction has in fact been ongoing. The GRADE (Grades of Recommendation, Assessment, Development, and Evaluation) approach is an instrument for grading the quality of underlying evidence and the strength of clinical recommendations [30, 31].

In a similar vein, the AGREE (Appraisal of Guidelines and Research and Evaluation, Europe) is an instrument developed to provide a basis for defining steps in a shared development approach to produce high-quality clinical practice guidelines revised based upon the best available evidence [32, 33].

Both the GRADE and the AGREE instruments are laudable efforts in the direction of fostering the growth and expansion of research synthesis and metasynthesis. Our group has expanded the GRADE tool further in an effort to emphasize not only in dual applicability to systematic reviews and to CRCSR but also the solid conceptualization it offers of the strength of the clinical recommendation the instrument proffers. A complete validation of the expanded GRADE (Ex-GRADE) is now available in the literature. We also have proposed that text mining and semantic content analysis by means of specialized software's can serve to quantify divergences and consensus in statements about clinical relevance, and yield data that permit an inferential analysis of clinical significance [35].

## 3.4 Conclusion: Implications and Applications to Comparative Effectiveness and Evidence-Based Decision-Making

### 3.4.1 Convergence and Divergence of Efficacy and Effectiveness [4, 13]

In absolute terms, efficacy refers to whether or not an experimental clinical intervention tested in the context of a clinical trial yielded valid and replicable outcomes. In lay language, we might say that efficacy tells us whether or not the treatment "worked." Of course, that term suggests an expectation of successful outcome along

[22] An attempt toward quantifying clinical recommendations based on R-AMSTAR scores, and translating the outcome into a simple ranking system has been proposed by Kung et al. [16].

the criteria we set in the initial study hypothesis. Thus, the bias that underlies the common language is that by "working" most often we mean "proved our hypothesis true": that, of course, is far removed from the notion that "efficacy" is intended to impart.

Efficacy reports about the quality of the design, the methodology, and the statistical analysis of the data. Efficacy derives from the power of the study, the stringency of the statistics, and the validity of the inferences, and pertains to the stringency of our adherence to the scientific method.

A strong and stringent clinical trial, whose data analysis are based on appropriate statistical approaches and yield valid inferences, will be deemed efficacious. It will have "worked," regardless of whether the null or the alternative hypothesis will have been proven true, and regardless of which, among the possible alternative hypotheses, is demonstrated.

Efficacy relates to the reliability, strength, and power of the study. It is a form of validity of the study that derives directly from how well the study was conducted, which itself follows from how much random error is left unexplained. That is to say, efficacy, yes, tells us if a clinical study "worked," but it does so because of its inherent dependence upon the effort of the investigator in constructing the research project correctly, and fractionating as much as the random error as possible.

In that regard, efficacy is a form of internal validity of a clinical trial. It establishes the replicability of the clinical outcome, within the confines set by the clinical trial requirement of sampling, randomization, dropout, measurement, intention-to-treat, and other intervening variables. Fixed within these elements of the clinical trial, efficacy provides critical information as to the extent to which similar findings could be obtained again, all variables kept unchanged. In that vein, efficacy is akin to the conditions of a fixed model in meta-analysis, as well as the internal validity of the study.

By contrast, effectiveness relates to the experiential reality of the clinical practice. Effectiveness pertains less to concerns relating to whether the clinical trial was performed well, and more to whether or not the intervention minimizes risk, maximizes benefit, and yields these outcomes at the lowest (or at least the most reasonable) cost. It is fair to say that effectiveness does not pertain to a clinical trial study *per se*, but rather to the pragmatic implementations of its findings to the intricate complexities of clinical treatment.

Whereas effectiveness, as was the case for efficacy, tells us if a given mode of intervention "worked," the criteria are diametrically opposed: in the one hand, as we noted, "working" efficacy relates to replicability of the observations, "working" effectiveness means ensuring the highest possible benefits of the clinical outcomes sought at reasonable costs and with minimal risk.

Effectiveness is a broader construct than efficacy. It pertains to a broader domain (e.g., clinical treatment of all patients with a given condition vs. study outcomes on a random sample of patients afflicted with the condition) and is therefore more generalizable than efficacy. As the latter approaches the concept of internal validity, effectiveness is akin to external validity.

In the same vein, as efficacy evokes an inferential model that is fixed within the constraints of the study, effectiveness brings forth the notion of applications and implications that are open to encompass the randomness of the clinical environment. In that light, effectiveness conveys external validity and a random model of inference.

### 3.4.2 Making Clinical Decisions Based on Research Metasynthesis for Effectiveness Versus Efficacy

Perhaps the single most important use of the science of research synthesis and research metasynthesis in the health sciences, such as dentistry in general and endodontics in particular, pertains to empowering the clinician to make fully informed decisions for treatment that rest not only on the patient's wants and needs, clinical tests, and history, or the clinician's experience and personal awareness of the available research, but, as well, on the best available evidence.

It is important to stress the summative quality of this *sine qua non*: in addition to all the previous, which equate the best current clinical practice, reliance on the science of research synthesis and metasynthesis signifies adding to the decision-making process the best available evidence.

If the decision-making process pertains to issues of concern that include the set of risk, the benefits, or the overall cost of a given mode of intervention, then it is likely to proffer the domain of effectiveness. Often a query is proposed that seeks to compare and contrast the effectiveness of diverse interventions.

That is to say, comparative effectiveness research is a research endeavor that utilizes the study design of research synthesis and metasynthesis to use the best available evidence in the pursuit of the optimal treatment, with respect to favoring benefits and decreasing costs and risks. This is obtained by estimating the odds of attaining success in a sequential process, which derives and is akin to the Markovian decision matrix tree.

Comparative effectiveness analysis is obtained through a utility model of decision-making. It is a process based on and driven by numbers (i.e., odds), which proceeds through a process of inclusion and exclusion of modalities based on the probability of their yielding relative success in attaining the most likely low-cost/high-benefit/low-risk desired outcome.

If the clinical decision sought pertains to a personalized, patient-centered query, then the logic model pursuit of evidence-based clinical intervention will be recommended [3, 4].[23]

[23]As noted elsewhere, the research synthesis process for comparative effectiveness typically includes bibliomes composed of cluster randomized controlled trials (CRCT's), whereas better informed patient-centered clinical decisions about comparative efficacy for evidence-based practice results from systematic reviews that arise from the research synthesis of bibliomes consisting of traditional RCT's [36]

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# Efficacy, Effectiveness, and Evidence-Based Dental Decision-Making: Principles and Practice

4

António Mata and António Vaz Carneiro

**Core Message**

In the dentistry, it is essential for the dentist to take the best decision for the patient, and this decision can only happen in the light of evidence-based dentistry. When we talk about evidence-based dentistry, it is very important to know its rules and regulations. It will help the dentist to take the best decision for the treatment both medically and financially.

## 4.1 Introduction: Clinical Decision-Making

### 4.1.1 Type of Knowledge that Supports Clinical Decision-Making Based on High-Quality Evidence

This is truly a different world we are living in. In fact, these days, at our fingertips, on the internet, lies the major repository of information humanity has ever seen. Moreover, the information explosion has led to an increasing consumer movement which is shaping social and economic paradigms.

Health care is no different, and dentistry also has to cope with the rapidity at which products enter and leave the market, new techniques emerge, and patients ask difficult questions with increasing expectations and demand. For the majority of us, in the dental school, the main sources of scientific information were the teacher, the

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expert, and the textbook. Very few of us, if any at all, did an electronic search for scientific validation of a clinical question we would like to answer.

Searching for articles in the library was a titanic task. Later at practice, textbooks and mainly academic teachers and more experienced colleagues generally continued to be the main feeders of clinical information to clinicians. Nevertheless and unquestioningly best intended they may be, these represent unsystematic and thus biased sources of information.

The old and comfortable paradigm "it works in my hands" can no longer face the unavoidably descending clinical slope of clinical competence. Fortunately, in the last decades, new paradigms for clinical practice have emerged. The technological means exist today for the establishment of repositories of clinical evidence, which provide a systematic gathering of all available evidence, appraise it, and distil sound evidence-based clinical recommendations.

Evidence-based dentistry and more recently comparative effectiveness research are such tools, aiming at helping clinicians in delivering the best care, informing patients, and guiding all health-intervening parties to correctly assess cost benefit of clinical interventions. Evidence-based dentistry and comparative effectiveness research are growing to rapidly become, in the aftermath of globalization and world convergence, indispensable features which will contribute to diminish the existence of pernicious, dissimilar health practices.

Finally, this will undoubtedly integrate and shape most of the medical academic curriculum at pre- and post-graduate level. These are therefore promising times from which every clinician will surely benefit.

### 4.1.2 Clinical Decision-Making in the Twenty-First Century: The Problem

Recently, while watching a dental cosmetic extreme make-over type of television program, I heard a colleague stating live that modern dentistry and clinical practice were becoming simpler, credits due to technological development. I could not disagree more with such unrealistic and misleading statement.

This is so because for nearly three decades now, there is an increasing awareness among the clinical community that unfortunately in many ways and for many different reasons, clinical decision-making seldom relies on the best evidence available. Moreover, clinical practice in the new millennium is becoming increasingly complicated and changing so rapidly that clinicians are permanently challenged with sometimes insidious and unperceived problems, which can be threatening not only to patients, but for themselves alike.

Difficulties in clinical practice are located mainly in three areas:

(a) Clinical practice and its expertise
(b) Patients and their preferences and interests
(c) Knowledge acquisition and clinical evidence

Clinical practice is often performed in a pattern recognition base, where clinicians tend to identify signs and symptoms which are suggestive of diagnosis and

then apply established protocols for treatment, expecting a probable prognosis. Most often, this type of practice is performed under looking scientific-based clinical reasoning.

This derives mainly from the archetypes of clinical teaching both at under and post-graduate levels which tends to rely on expert-based transmission of knowledge derived from unsystematic observations compiled over years of experience. Unsystematic observations usually lead to insight development, which may be very respectable, even teachable, but rarely appears on medical literature.

On the other hand, clinical reasoning based on sound evidence and derived from critical appraisal skills of medical literature is much more time- and money-consuming and therefore less used [1].

In this context, clinicians frequently take clinical practice for granted as a linear process full of certainty. However, clinical practice is extremely uncertain, with hypothesis scattering occurring at all steps of the clinical pathway from diagnostic to treatment options. This fact inevitably generates multiple approaches for an enormous number of clinical issues, for example, office versus home vital bleaching, proximal caries restoration versus surveillance and remineralization, or one versus two-time appointment endodontics.

Another difficulty clinicians presently have to face is linked with the increasing number of factors which are external to direct practice but influence it on a daily basis. In fact, clinical practice is actually shaped not only by scientific factors but also by educational, economic, political, or social issues which can be pointed as directly conditioning the uprising of globally dissimilar clinical approaches in dentistry [2, 3]. Each time health insurance policies determine coverage fees (for instance tooth extraction only excluding endodontic procedures), they are strongly biasing clinical decision, and the same example could be further extended to employers, private investors, or policy makers. Even from a political point of view in a global perspective and considering issues like the avian or swine flu, for example, one cannot draw an unequivocal and evidence-based line between real preventive needs and journalistic media-driven mass hysteria.

However, health care stakeholders cannot be put to blame if adequate syntheses of best available evidence are not produced, on which health care cost-benefit assessment and directives should rely upon and where the health care decision is concerned.

Finally, the rapidity at which technological developments driving turnover of new products and techniques, with dental manufacturers marketing and discontinuing dental products and devices at a speed from which independent research is unable to cope with, has an overwhelming burden on clinical decision-making ability.

Moreover, technological development in general and late twentieth century informatics burst in particular have created new legions of patients which are now more inquiring, demanding, and believed to be better informed toward clinical procedures. This may not be entirely true since usual information sources patients recur to are quite different from the traditional repositories of sound clinical research. In fact, traveling amid information junk and distilling good quality clinical

information is daunting even to trained clinicians lest an impossible task to patients, but it is a new reality all of us have to cope with now [4–6].

However, the very same proceedings of clinical practice have changed, leading to the consolidated belief that no medical intervention can be fully achieved without considering and integrating patients' legitimate expectations, demands, and rights into the decision process, this being an essential step if effective bettering of quality of life is to be attained.

In the past years, several studies have strongly suggested the paradox that despite clinical performance tending to ameliorate as time goes by, clinical knowledge deteriorates inevitably [7]. Explanations to this fact are of multivariate nature from difficulties in reading to modus vivendi features where modern life pace and time-consuming carriers take an unavoidable toll in reading time availability.

Fighting the inexorable decline slope of the knowledge curve is therefore an imperative task for any clinician, but difficulties in reading exist and can be placed at several levels ranging from literature dimension to scientific illiteracy.

Although computing and the creation of scientific databases like PubMed have facilitated the access to medical literature, the truth is that confrontation with its dimension also became more frequent. There are more than 25,000 medical journals worldwide, and about 10,000,000 publishing authors between 1 and 2 million papers are published every year, and these numbers are expected to duplicate each 19 years.

Even in the dental field for each dental specialty, numbers vary between 500 and 3,000 papers yearly. So, choosing the right evidence needed seems at the very least quite daunting even for a trained expert [7]. Furthermore, reading is also extremely time-consuming, and studies have proven that clinicians experience diminished reading compared to what would be expected and desirable [2].

Moreover, we have been led to believe that the quality of research presented in scientific journals was secured trough existing systems like peer review. This may not be entirely true since publishing pressure on researchers and publishers are giving rise more frequently to antagonist reports of similar studies, forcing the clinicians to proceed with a peer review of their own.

However, clinicians are often underprepared to undertake this task. Scientific illiteracy is quite common since there is scarce training in critical thinking in under and even post-graduate teaching programs. Terms like odds ratio, absolute and relative risk reduction, confidence intervals, and number needed to treat as most clinical research designs are unfamiliar to the majority of dentists.

The important consequence of the former statements is the many times referred unavoidable slippery slope of clinical competence, as knowledge deteriorates quickly over time, leading to a less rigorous clinical practice and generating clinical entropy at different levels, which if not eliminated will introduce problematic situations for clinicians and consequently for patients.

Therefore, clinical practice is definitely not becoming simpler, and one can only expect that such dissimilar health practice decisions among highly similar patients mean that they cannot all be receiving the best care. Thus, a shift in clinical paradigms has been long advocated in answering these problems.

## 4.2 The Solution – Evidence-Based Practice (EBP)

### 4.2.1 The Principles of Evidence-Based Practice

Evidence-based practice (EBP) is defined as the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients [8]. It is a paradigm for clinical practice, which started for general medicine in 1992 and later extended to the field of dentistry. It has become a movement of increasing popularity, recognized by many as the best and accurate methodology for solving clinical problems.

EBP stresses the examination of evidence from clinical research, suggests that interpreting the results of clinical research requires a formal set of rules, and places a lower value on authority than the traditional medical paradigm [9]. Alternatives to evidence-based medicine, all too common in day-to-day information management, include reliance on the eminence, vehemence, eloquence, or confidence of the source [10]. On the other hand, evidence-based medicine is meant to complement, not replace, clinical judgment tailored to individual patients [11].

As a distinctive approach to patient care, EBM involves two fundamental principles. First, EBM posits a hierarchy of evidence to guide clinical decision-making. Second, evidence-based medicine is not intended to replace clinical judgment. Care for the individual patient includes the best research evidence as a benchmark, tailored to individual circumstances that may include genetic makeup, past and concurrent illnesses, health-related behaviors, and personal preferences.

Therefore, evidence alone is never sufficient to make a clinical decision. Decision-makers must always trade off the benefits and risks, inconvenience, and costs associated with alternative management strategies and, in so doing, consider their patients' values and preferences [12].

EBP is therefore a scientific approach on health care decision-making; it is not self-explanatory and requires specific training. It is a permanent process of problem solving, in which the patient contact generates the need for sound scientific clinical evidence, upon which clinical decisions about diagnosis, therapy, and prognosis (only to name a few) are taken. EBP constitutes itself in a clinical algorithm with 5 components, what has been called by some the five A's:

- Ask
- Acquire
- Appraise
- Apply
- Assess

### 4.2.2 Clinical Question Formulation (Ask)

EBP's first step relies on the correct formulation of a clear and relevant clinical question with an available answer in medical literature [13]. This question arises from patient contact and is the initial premise upon which the next step of the EBP process, namely evidence search, is drawn.

Sometimes, questions may be very simple to formulate without major concept difficulties (like which antibiotic dosage should I prescribe to this particular patient), and therefore, evidence may be easily traced in a therapeutic formulary or a textbook. However, most of the time, questions may not be so clear to formulate and answer.

In these cases, it will be necessary to decompose the question in four classical components:

1. The patient
2. The exposition (to a treatment, a diagnostic procedure), frequently called the intervention
3. A comparison to other intervention, active or inactive, like a placebo
4. One or several outcomes of interest

This method of formulating focused clinical questions is usually referred in the EBP nomenclature as the PICO framing, from which a comprehensive database search for scientific evidence is to be performed in order to obtain studies that may help to find an answer for the question [13, 14].

### 4.2.3 Search and Selection of the Evidence (Acquire)

This is a fundamental step since it is from the search of the best available evidence in databases that a post-critical appraisal process will enable the formulation of a reliable answer to the initial question.

To the individual clinician, a distinction between useful and pointless information implies in a simplistic approach to be able to respond to four topics:

(a) Is this information related with a clinical outcome of interest to my patient?
(b) Is this question customary to my clinical practice?
(c) The recommended intervention is possible for me to undertake.
(d) If this information or recommendation is true, will it force a change in my clinical practice?

High-quality-evidence search implies the establishment of a hierarchy of the relative value of primary and secondary (which synthesize the former) studies since not every study possesses the same methodological internal or external validity. Different types of evidence pyramids have been suggested in the literature with minor differences [1, 2, 6–8].

Secondary studies like systematic reviews or meta-analysis represent synthesis, critical views, and evidence-based clinical recommendations of primary studies. These, along with randomized controlled trials as primary studies, are at the top of the evidence pyramid (for interventions, therapeutic or preventive) and should always be the first type of studies upon which a search should point [15, 16]. There are two types of electronic databases, organized in such a way enabling systematic searches.

The first type from which Medline is a typical representative consists of traditional bibliographic sources in which original papers are indexed for elusive search. Besides Medline, other databases like EMBASE (the online version of the European

medical excerpt) or SCISEARCH (the online version of the Science Citation Index) are available.

The second type of bibliographic databases includes syntheses and appraisals or comments from primary sources or secondary evidence like systematic reviews, meta-analysis, or guidelines. Cochrane Library, Center for Evidence-Based Dentistry, or *Journal of Evidence-Based Dental Practice*, *Evidence-Based Dentistry*, and ADA are among the most reputed sources. Secondary sources have become of greater importance especially for EBP users who should be distinguished from EBP doers.

This importance is outlined by the recent emergence of new evidence pyramids, which place individual studies at the bottom of the pyramid and syntheses, synopses, and computer clinical decision support systems at the higher positions [17].

Search can be direct on databases or helped by special software features like OVID or Winspears; anyway whatever search strategy or software is used, a well-conducted search will unearth a relatively concise and focused number of papers which will be then ready for critical appraising which is the next step on the EBP process.

### 4.2.4 Critical Appraisal (Appraise)

#### 4.2.4.1 Problems and Difficulties in Producing High-Quality Scientific Evidence to Support Practice

Critical appraisal of the retrieved and selected evidence and the assessment of its internal and external validity and clinical usefulness will determine the inclusion (or exclusion) of the obtained information as a complement of patient-based clinical decision, depending on the confidence attributable to the methodological study structure.

The need for a sound and rigorous appraisal criterion comes from the real possibility that most of the published research may have little methodological rigor and practical clinical relevance.

Several critical appraisal systems exist usually in the form of scoring questionnaires. They are considered mainly for evaluation of primary studies. The predefined questions are logic but not self-explanatory, and skills in critical thinking have to be acquired with specific training.

Although differences exist between appraisal systems, the questionnaires tend invariably to answer to three major groups of questions: “Are the results valid?” a set of questions which aim at the internal validity of the study; “Are the results important?” which evaluate not only the outcomes and the statistical significance but mainly the effect size and its clinical importance; and finally, “Are the results applicable to my patients?” which consider the external validity and are extremely important in decision-making.

External validity in a study has been defined as the unbiased inferences regarding a target population beyond the subjects in the study [17]. It is straight linked to the study population and the research hypothesis, and one must always consider that

geographic, genetic, or any other population differences may preclude direct application of the results to the individual patient.

### 4.2.5 Synthesis and Implementation of the Evidence (Apply and Assess)

Once the evidence is gathered, distilled, and appraised, the clinician is now endeared to synthesize it in the form of a clinical recommendation, which may be directly applied to the patient [7].

EBP presents challenges. Despite the fact that EBP has been experiencing an increasing momentum for the past two decades, and being presently viewed as an indispensable tool for health care under very different perspectives ranging from the private clinician to policy makers, some expectations regarding the rapidity of adherence to this paradigm may have been somewhat overoptimistic.

Clinicians are too busy and under prepared to perform lengthy analysis of the primary literature. Almost a paradox, lack of time for literature appraisal was in fact viewed in the early days as driving force for EBP, envisaged as a new quick and efficient way to access best evidence, providing that evidence would be readily available to clinicians who would therefore undoubtedly and swiftly use it in clinical decision-making [17].

Therefore, EBP doer's early challenge and focusing of strength was all about literature availability, search skills, and critical appraisal or probabilistic thinking competences. Accessing better information would assumedly translate into practice change since practitioners would be more knowledged, and downstream benefits would undoubtedly include an increase in treatment effectiveness and cost-benefit efficiency [18].

So, energies were concentrated in developing effective evidence synthesis summaries and computer routes of evidence dissemination and making them available to the clinicians. It is true that secondary information like systematic reviews and its distillation into expectably sound clinical recommendations with expected high clinical impact like evidence-based clinical guidelines have flourished like never before in the past years.

The deep process of clinical behavioral change and widespread adherence to the EBP process was thus envisaged as natural and logic consequence of the availability of more accurate information.

However, in our personal view, EBP training actions which had forcedly different characteristics in different settings and clinical populations were always extremely focused on literate skills, leaving somewhat faded and narrower approaches to clinical expertise, patient's values, and how these should equate with evidence.

Although necessary, mastery of literature skills is not enough for EBP. This attitude created an autistic distortion of the three circle paradigm, with the imperceptions that not stressing the importance of the integration between all parts of the process (evidence, clinical expertise, and patient values) created an anamorphic paradigm, with little chances of functioning properly.

Maybe, at that time, EBP doers were too occupied embracing the task of generating a new attitude toward knowledge production and access on one side and establishing secondary information on the other that they did not have the time to look aside.

It is at least quite astonishing that such pitfall passed unperceived since it was well established that attempts to translate research findings into practice and beating clinical inertia through usual forms were neither efficient or effective [18].

Thus, after two decades, it is time to introspectively find out what has been achieved in this new era.

The "evidence based" expression has unquestionably entered the medical lexicon on an everyday basis in conferences, papers, or any clinical issue. However, the reality is that most of the time, there is a clear misinterpretation of the principles and purposes of EBP, which are most often confounded with practice based on evidence.

To simply underscore each clinical affirmation with literature reference or even with an orphan and out of context evidence level is not, as unfortunately many times people think, EBP. In fact, it stretches no further that any unsystematic statement, which usually characterizes the practice of traditional medicine. EBP is an intricate and complex matrix of occurrences aiming at generating a consensus statement that summarizes the outcome of a process of comprehensive and systematic evaluation of all the literature. The statement provides ipso facto scientific validation of the best available evidence thus generated from all of the available research and of the clinical decision-making process [19].

It underscores the fact that research in the health sciences is advancing at such a fast pace that the body of evidence must be systematically evaluated and synthesized for benefit of patients, health providers, and society [19].

Albeit not easy to assess, some recent indirect evidence seems to point to the fact that clinicians still continue to rely mainly on expert colleagues and continuing education presentations for clinical decisions, and there is limited adherence to evidence-based guidelines despite the strong evidence-based recommendations which characterize such interventions [20–23].

The early assumption that clinicians would, despite their overcrowded schedule, still find time to act as EBP doers in all steps was overoptimistic. Time constraints are a real handicap in EBP dissemination, at least as it was envisaged primarily.

Another constraint, which took its toll on EBP implementation, has to do with cultural and economic values, introducing fear and suspicion about the process and contributing to clinical inertia. Culturally, misconceptions about EBP from clinicians arise also from the fact that these are often quite impermeable to impositions, which may be perceived as an unwarranted intrusion into the profession, and in the traditionally isolated environment of dental practice, this may be more the rule than the exception.

On the other hand, economic values can menace the EBP process with different degrees of fairness, and this may acquire an extraordinary importance in dentistry since most of the practice is within the private sector. Sometimes, the evidence may suggest that patients benefit less from or have to comply with increased fees for a determinate clinical procedure compared with another, which is financially less rewarding, and evidence may act on patients' behalf or oppositely; other times,

there will be an obnoxious risk of EBP hijacking from private business partners which will be threatening to doctors and patients.

EBP is an intricate matrix of occurrences and players, which all have to do their part in order for the system to truly function. A high degree of coordination is mandatory, and this is one of the biggest challenges EBP faces in the future. Albeit nothing but a part of the process, evidence still holds as a cornerstone, and good quality primary research is the first step without which the old aphorism "Garbage in, garbage out" will inevitably apply.

In dentistry, this may be more of a rule than an exception. In a recent study in which the results of 80 Cochrane database systematic reviews were looked upon, it was concluded that 35 (44%) failed to answer the initial clinical question due to lack of evidence, 32 (40%) managed to answer the question, but from those, 10 (12%) found no difference between the interventions considered, and 22 (28%) found an effectiveness in the intervention under study.

Finally, the answers to the 13 remaining questions were strongly affected by evidence quality which was rated as weak, or lacking, meaning that a clinical recommendation could be established only to a minor set of questions [20, 24]. This has a devastating effect on EBP credibility and gives its detractors an unavoidable yet demagogic ground for criticism. Moreover, one of EBP pitfalls, which occurs but must be avoided at any cost, is the tempting and fallacious conversion of the absence of evidence for an effect to the evidence of absence of the same effect [19, 20].

The fact is that "good quality research" is quite a broad term requiring multistep appraisal ranging from study structure (its anatomy and physiology) to study objectives, purposes, and context. The trade-off between studies' internal and external validity is always a delicate equilibrium and hard to achieve specially when considering that research usefulness addresses to patients, clinicians, private payers, policy makers, and public stakeholders.

However, and albeit a great gap between evidence and health care execution still has to be bridged, high-quality clinical research remains the major aim yet to be achieved in the complex paradigm of EBP.

## 4.3 Pragmatic and Explanatory Trials

### 4.3.1 The Basis for Understanding Clinical Effectiveness Research

The major constraints in translating the evidence-based paradigm to effective clinical practice have been now identified. In the USA, the Institute of Medicine (IOM) has estimated that less than half of all treatments delivered today are supported by high-quality evidence [25].

In fact, many supposedly high-leveled evidence summaries meant for direct clinical advising like guidelines or systematic reviews often lack quality research as their basis, and when they do are seldom translated by practitioners into clinical practice.

Dentistry has several particularities when compared with other medical fields. Apart from preventive or prognostic interventions in dentistry, cariology, or oral

oncology which are managed mainly at a community- or hospital-based level, dental care by the individual clinician, at his practice directed for managing single patients, is almost and exclusively one of intervention and therapeutic nature. Thus, the randomized clinical trial (RCT) or the systematic review/meta-analysis of RCTs should be considered as the highest primary and secondary sources of clinical evidence on interventions, respectively.

Systems of appraisal for RCTs like the critical appraisal skills programme (CASP) or editorial support systems for authors like the CONSORT which aim at helping in the reporting of this type of research have been developed with the aim of asserting and enhancing trial quality. However, the problem may run even deeper, as this type of highly qualified research output has been recently and increasingly challenged, addressing the relation between its structure and its purposes.

In 1967, two French statisticians published a paper questioning the adequacy of most therapeutic RCTs' formulation from the very beginning of their inception [26].

In their view, there was a profound discrepancy between the use clinicians make of trials, namely to get information about therapy decisions, and the design of those very same trials most often best suited for testing causal hypothesis [26].

Consequently, they proposed a classification of RCTs based on their purpose and upon which their structure and design should be adapted. Trials in which the main objective is to explain a biological process in response to an exposure to a particular treatment or, in other words, can this intervention possibly work and why, are to be named explanatory trials by opposition to those RCTs whose main goal is simply to directly inform real world decisions among alternative treatments, or does this intervention work in normal practice under usual clinical conditions.

The explanatory and pragmatic framework represents opposite attitudes toward study designing, resulting in a differential weighing of the delicate trade-off between internal and external validity in RCTs.

In explanatory trials, patients' inclusion criteria, treatment, control, and settings are highly restricted, maximizing the detection of treatment effect differences between study groups and thus emphasizing internal validity, whereas in the pragmatic attitude, recruitment criteria are widened except for the clinical condition of interest, greater flexibility is drawn upon the intervention which should mimic standard practice, the outcome is directly related to patient needs, and the setting should be maintained as realistic as possible, therefore enhancing external validity with the purpose of better informing real world decisions among alternative health care interventions.

In the 1967 Schwartz and Lellouch paper, the two French statisticians proposed that the trial's purpose should be clearly stated and the design properly adequate in order to avoid mismatches since information from an explanatory trial hardly will provide an adjusted answer to a pragmatic question.

Discrepancies between trial purposes and designs lead to pernicious voids affecting practitioners and health care policy makers who are left without direct applicable clinical evidence on which to base patient care decisions or define sound funding strategies.

Recent studies suggest that inadequacies between trial design and purposes may be far more common than suspected [25]. In the USA alone, from 250,000 trials

listed by the national library of medicine, the proportion of pragmatic trial scores under 0.04%, suggesting that an overwhelming majority of trial designs are explanatory in nature and therefore possibly unsuitable for direct health decision-making. This only confirms Lellouch and Schwartz's worst suspicions that even at the time they wrote their paper, from all the explanatory trials to their knowledge, the pragmatic approach would have been far more often justifiable [27].

Why is it so? Three major causes have been considered.

The major force driving RCT implementation is licensing regulations by awarding bodies. In developed countries like the USA or across Europe, licensing regulations from the FDA and agencies alike are extremely demanding, whereas efficacy of the interventions is considered. Historical reasons like the thalidomide tragedy are behind this philosophy, and safety concerns push the licensing demand onto extensive animal studies and human efficacy and documentation.

Thus, regulating policies stream steeply against the pragmatic concept, which is often viewed as lack of scientific rigor. Active control studies are faced with mischief and disbelief and are discouraged, and the use of placebos for comparison is envisaged as necessary to enhance treatment effect sizes and classified as parameters of excellence. Reality-oriented clinical settings, mixed populations, and active controls are cited as potential threats, bias, and compared with poor standards in health care.

Therefore, industry costs with licensing are enormous, explanatory incentivized, and efficacy oriented. These costs take an unavoidable toll on the pragmatic approach since after such spending, corporations have strong reserve and unwillingness in funding research with less than conventional ideal settings, unequivocal effect sizes brought up by the use of placebos, and highly selected and compliant patients [27].

Moreover, research funding agencies like the National Institutes of Health in the USA, or others around the world strongly prefer to support trials which aim at elucidating clear physiologic hypotheses, thus striving for explanatory purposes in supported projects. Some say that research pertains to efficacy while health quality assessment is linked with effectiveness [28]. As a result, the pragmatic approach is much diminished when compared to the explanatory types of studies.

However, great concerns from stakeholders, policy makers, and even clinicians are arising related to treatment cost benefits and risks in the real world. In the past few years, an active debate has been going on whether to elucidate if the pragmatic attitude would in fact lead to better informed decision-making [27, 29].

It seems to be the case as there is presently an increasing call for pragmatic trials which are believed to be better providers of direct information on clinical decision-making related to doctors, patients, and third party funders.

Thus, the design frames of a study meant for information on a research decision about the benefit of a new treatment are likely to be more explanatory (reflecting ideal conditions). Those for a later trial of the same procedure intended to inform practical decisions by clinicians or policy makers are likely to be more pragmatic (reflecting usual conditions) [27, 29].

Publishers and journal editors understand that they must be an important part of this process, since recently, an extension of the Consolidated Standards of Reporting Trials (CONSORT) has been created with specific recommendation for reporting

pragmatic trials. This may be an important tool for helping clinicians and others to judge the applicability of trials to their own settings.

However, this is not an easy task. Increasing trial pragmatism and widening external validity without loss of the study rigor is extremely challenging. Explanatory and pragmatic are at the opposite extremes of a continuum multidimensional framework. Pure explanatory or exclusive pragmatic trials are almost academic concepts, which are extremely rare to find in real life.

When planning their study, researchers have to choose whether a trial's design matches the needs of those who will use the results. A tool to locate trial design choices within the pragmatic–explanatory continuum could facilitate these design decisions, help to ensure that the choices that are made reflect the intended purpose of the trial, and help others to appraise the extent to which a trial is appropriately designed for its intended purpose.

Such a tool could, for example, expose potential inconsistencies, such as the use of intensive adherence monitoring and intervention (explanatory tactics) in a trial being designed to answer a more pragmatic question. Alternatively, a trial might include a wide range of participants and meaningfully assess the impact (pragmatic tactics) but evaluate an intervention that is enforced or tightly monitored (explanatory tactics) and thus not widely feasible.

By supporting the identification of potential inconsistencies such as these, a pragmatic–explanatory indicator could improve the extent to which trial designs are fit for purpose by highlighting design choices that do not support the needs of the intended users of the trial's results [27–30].

Recently, a group of international researchers has developed and proposed a new tool to assess and display the position of any given trial within the pragmatic–explanatory continuum, the Pragmatic–Explanatory Continuum Indicator Summary (PRECIS). Its major aim is to help researchers assess the degree to which design decisions align with the trial's stated purpose (decision-making versus explanation) [29].

The following ten domains have been identified that should be taken into consideration by researchers when stating the purpose of the trial within the pragmatic–explanatory framework [29]:

1. Participants' eligibility criteria
2. Flexibility of the experimental intervention
3. Experimental intervention – practitioner expertise
4. Flexibility of the comparison intervention
5. Comparison intervention – practitioner expertise
6. Follow-up intensity
7. Primary trial outcome
8. Participant compliance with prescribed intervention
9. Practitioner adherence to study protocol
10. Analysis of primary outcome

If a trial purpose is clearly shifted to one of the ends of the explanatory–pragmatic continuum, identification of the trial characteristics within each domain should not be hard to assess. However, this is most of the time a highly hypothetic scenario as

purpose decisions within each domain are expected to vary considerably between the explanatory pragmatic continuums and also between each domain.

Therefore, a systematic approach in assessing whether design decisions are fit for purpose is to be taken in four steps [29]:

1. Declare whether the purpose of the trial is pragmatic or explanatory.
2. Specify the settings or conditions for which the trial is intended to be applicable.
3. Specify the design options at the pragmatic and explanatory extremes of each domain.
4. Decide how pragmatic or explanatory a trial is in relation to those extremes for each domain.

The authors propose that the results of this assessment should be displayed in a plot where each domain is arranged like the spoke of a wheel with the explanatory pole near the hub and the pragmatic end near the rim. Researchers are expected to put a thick on each spoke between the explanatory and pragmatic ends, and joining each thick will complete the display.

This type of plot is pointed by the authors as having three advantages: quick and efficient suggestion of the explanatory pragmatic shift, detection of inconsistencies within the domain framework, and last but not least a quick and useful way of communication interpretation and decision for trial users.

Pragmatic and/or cluster randomized trials may provide a critical link between the highly regulated "efficacy studies" that dominate current research and the "effectiveness" studies that are critical to clinical practice. The establishment and validation of personalized medicine require further innovation in these areas.

## 4.4 Comparative Effectiveness Research

### 4.4.1 A Description of Comparative Effectiveness Research

Presently, and despite the fact that in the past decades a tremendous evolution has occurred in medical knowledge and practice, there is a generally accepted perception that large gaps remain in the quality and outcomes of health care. The need for better clinical evidence, which translates from and applies to real world conditions, is a must if clinical intervention disparities are to be diminished and cost-effectiveness of health interventions improved.

Health care practitioners need clinical evidence, which not only reports to populations but also provides information about individual patients in realistic settings. The increased knowledge in proteomics, genomics, and other biological fields is generating the potential for more targeted therapies, fuelling the urgent need for more evidence about interventions that describe research outcomes at subgroup levels besides main representative populations.

On the other hand, patients also need more accurate information on the population-level effects of health interventions, patient-level predictors of both positive and negative outcomes, and their preferences in making choices, since they are

being progressively called upon participation in self-related decisions, and health insurance plans are shifting greater shares of expenses to them [30].

Moreover, public and private health care expenditures are at their highest peak and expected to rise continuously in the next years. However, there is an established lack of association between health spending, costly interventions, and improved outcomes. Achieving health funding and allocation of resources while maintaining economic sustainability of public and private co-participation welfare is thus increasingly challenging.

Therefore, policy makers, health employers, private insurers and financing programs, and all of those who bear financing responsibilities in health also need clinical evidence which measure real-life outcomes and the benefits and harms of an intervention in ordinary settings and broader populations.

The type of clinical evidence needed to help answering these former issues has to rely on a widened degree of pragmatism or effectiveness, oppositely to the traditional and more implemented explanatory or efficacy approach.

Comparative effectiveness research (CER) is an emerging paradigm which aims precisely at generating evidence comparing the effectiveness of at least two active interventions, in real clinical settings, including subpopulation analysis within an economic cost-benefit frame.

The IOM definition of CER goes by: "Comparative Effectiveness Research (CER) is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels" [25].

However, different definitions for CER have been established, all including its mandatory characteristics, namely:

1. CER has the objective of directly informing a specific clinical decision from the patient perspective or a health policy decision from the population perspective.
2. CER compares at least two alternative interventions, each with the potential to be "best practice."
3. CER describes results at the population and subgroup levels.
4. CER measures outcomes – both benefits and harms – that are important to patients.
5. CER employs methods and data sources appropriate for the decision of interest.
6. CER is conducted in settings that are similar to those in which the intervention will be used in practice.

There are various research techniques for performing CER including generation of primary data, systematic reviews, electronic health records, patient registries, and other observational datasets [25].

Primary data arises from individual clinical studies, preferably RCTs, namely head to head trials; observational studies like cohort studies also may be performed, but increasing pragmatism in non-randomized studies will dramatically increase chance of bias, which may threaten validity, and therefore extreme caution should be employed.

Secondary studies like systematic reviews or meta-analysis may be used with the advantage of pooling and enhancing results from individual studies. CER is a potential means to standardize quality, improve outcomes, and control medication costs [31].

The methodology of CER is closely linked with EBP at its various steps, from PICO criteria clinical question formulation to clinical implementation of generated or synthesized evidence. Moreover, EBP and CER have similar aims as EBP intends to answer focused clinical questions with recommendations derived from high-quality evidence, which will help not only clinicians but also patient's preferences, enhancing their participation in health decision process with preserved freedom of choice. CER does exactly the same, emphasizing patient individual specificity and financial implications of compared interventions.

Thus, CER and EBP have the potential to work synergistically for the patient's and society's best interests [32].

However, CER is still at an early phase, and considerable investment measures have to be undertaken in order to achieve its goal of more informed decisions and better outcomes.

There are still many gaps to be bridged for CER to become an implemented reality, but considerable efforts are being made in the USA to identify CER needs and priority areas. In the current US public policy environment, the 111th Congress made a $1.1 billion investment in CER and has created a Federal Coordinating Council to coordinate CER within the Department of Health and Human Services (HHS) [25].

Findings are that the incentives for doing primary CER are still uneven, the infrastructure for supporting the development of new evidence is in an early stage of development, and a wide gap exists between CER results and their translation into consistent clinical practice and health policy [25].

These menaces and drawbacks are curiously intertwined with EBP since its major problems are also related with the quality and adequacy of research and its dissemination to clinical settings. This is of critical importance especially in the dentistry field.

As formerly mentioned, RCT with the potential for minimizing bias in patient selection, operator inclination, and other confounding factors have been recognized as the gold standard in dental clinical research.

However, dentistry has quite distinct features when compared with traditional medicine. Different outcomes are considered in dental studies, which are strongly influenced by the technical and surgical aspects of dentistry [33]. Typical RCT randomizes patients to one of two or more intervention groups. Although conventional RCTs are widely recognized as the most reliable method to evaluate pharmacological interventions, skepticism about their role in non-pharmacological interventions (such as surgery or dentistry) remains [34].

An alternative trial design, the expertise-based randomized controlled trial, randomizes participants to clinicians with expertise in intervention A or clinicians with expertise in intervention B, and the clinicians perform only the procedure they are expert in [34].

There is evidence to support the argument that increased use of the expertise-based design would enhance the validity, applicability, feasibility, and ethical

integrity of randomized controlled trials in surgery, as well as in other areas such as dentistry. However, the problem may run deeper as expertise-based trials at least where dentistry is concerned may not reflect the usual standard of care practice which is desirable to mimic in the view of more pragmatic research.

Most of dental practice is within the private domain. The usual standard of care setting is the single or multiple dentist private office, and dentists involved in practice have traditionally little or no contact at all with research activities. In fact, most, not to say all, of the dental research production has been performed within academic centers, in which investigators are specially trained and top experienced clinicians.

Clinical studies performed at academic settings have usually and inherently narrow degree of pragmatism with highly restricted requirements for patient selection, type of interventions, calibration of interventions, outcomes considered, and follow-up schedules. Moreover, in academic environment, there are little time constraints for clinical performance compared with the frequently packed and stressful patient lineup of a typical day at the private office [35].

Frequent criticisms have been pointed at this type of research as underpinning the disparity between these artificial academic settings and the environment of a full-time clinical practice, which for some may be one of the leading reasons for translational gap emergence between research and common practice. Recently, conflicting findings on longevity of restorations from academic-based clinical trials and practice-based cross-sectional studies have been reported, and the Agency for Healthcare Research and Quality (part of the US Department of Health and Human Services) indicated that the time from introduction of a new concept in health care to its use in practice may be as much as two decades [36, 37].

As formerly stated in this chapter, integration of research and practice is not easy because it often requires new kinds of relationships, conceptual frameworks, and even languages for clinicians, patients, researchers, academic institutions, and funding agencies [28].

For CER to become a reality in the dental field, new forms and initiatives linking research with clinical practice have to be implemented. Firstly in the UK and more recently in the USA, several NHS and NIH funding initiatives with the objective of excelling technology transfer processes by encouraging clinical research to be conducted in the practices of physicians and dentists so that the results may be seen as directly applicable – and, thereby, have a greater and quicker effect on clinical practice – have been launched.

Consequently, practice based research networks (PBRN) are flourishing and seem a promising paradigm for clinical translational research enhancement. They vary markedly in approach to the research, covering anything from product testing to educational networks, with emphasis on continuing and advanced education.

In PBRN, private practice clinicians are trained in different forms of clinical research, data registry, and reporting, enabling large clinical studies in real world settings to be conducted. However and once again, considering the technical specificities of the dental profession, caution must be taken for PBRN to represent more than just a mere change of setting. Critics argue that in PBRN, the training of

practitioners and vertical imposition of clinical problems to be studied will unlikely generate outcomes any different than from those within academic settings.

Proposals are that practice-based research should focus on the identification and frequency of recurring clinical problems. A clinical problem that cannot be identified is unlikely to be resolved. The criteria used by clinicians in practice should be the same as those used for the treatment of patients.

Nevertheless, PBRN is expanding fast, and this new paradigm can become a major driving force for development of CER in the future [35].

Finally, merely generating better evidence is not enough to meet the decision-making needs of consumers, patients, health care providers, and purchasers. To maximize its impact on the quality and value of health care, these parties must use evidence when making clinical and policy decisions.

Disseminating evidence into clinical practice must be accompanied by ongoing evaluation and feedback to decision-makers, the key characteristic of a true learning health care system. Once the CER studies have been completed and analyzed, the results must be evaluated for their usefulness to improve clinical decision-making.

Getting the information to the patients and their providers where they need it, when they need it, and in a format that is efficient and user friendly must be part of the effort to improve care. This critical integration step is accomplished by clinical decision support (CDS) systems that have the ability to work seamlessly with practice aids, electronic records, and devices [38, 39].

Integrating CDS into the workflow will make the information derived from CER both useful and important, two necessary components needed for adoption of CER by health care providers. The promise of comparative effectiveness research (CER) is that it will provide evidence that is better focused on the decisions of daily medical practice compared with existing evidence and therefore will help patients, caregivers, providers, payers, and policy makers to make informed decisions about health care [31].

## 4.5 Conclusion

Ultimately, health care practice is about patients, their health, and quality of life improvement within the possibilities of modern science. Treating patients is about answering questions and problem solving. It is a scientific act within the traditional *questio-disputatio* framework.

Questions arise from individual patient's focused problems, and answers derive from findings of objective experimentation in clinical science. Basic science is important in knowledge expansion of biological processes, which underpin physiology and pathology, but stretches no further where direct clinical interventions are concerned.

Clinical evidence, distilled from sound clinical research, is the mandatory information clinicians should rely upon where health care practice is concerned. We now have the technological means to rapidly access clinical information and appraise and synthesize it in systematic scrutinized clinically useful recommendations. EBP has been doing it for the last two decades, but despite several drawbacks and a translational gap

between clinical evidence and practice still lying to be bridged, the truth is that there is no possible credible alternative way for clinical practice in the years to come.

Therefore, it can be expected that EBP will continue to slope an increasing momentum in the years to come, as systematic reviews and high-quality-evidence-based guidelines will increase in number and importance. However, the quality and adequacy of clinical research has to be improved, especially where dentistry is concerned.

Clinical studies of efficacy are extremely important in clinical research as a departing proof of principle, which cannot be underestimated. However, efficacy studies are extremely focused, aiming maximization of internal validity at external validity expenses, and therefore of limited application to variable populations which exist in real clinical settings.

Thus, more pragmatic studies of effectiveness are needed as the only way to learn what works, how well, for what groups of patients, and in what specific circumstances. In this context, CER, along with EBP, is the emerging paradigm with the best potential to standardize quality, improve outcomes, and control medication costs which every health care related professional can no longer do without.

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# Endodontic Therapy Versus Dental Implant Therapy: Deciding the Best Approach

5

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**Core Message**

The purpose of this chapter is to discuss important considerations when deciding between two potential clinical treatment alternatives – endodontic treatment of a compromised tooth or its extraction and replacement with a dental implant. We will appraise the quality of available clinical evidence of the long-term success of dental implants compared with endodontic treatment.

## 5.1 Introduction

### 5.1.1 Decision Making by Doctor and Patient

Dental practitioners may face a common clinical situation in their daily practice – whether or not to preserve a tooth needing endodontic treatment. This decision might be facilitated when evidence suggests tooth survival prognosis is good [1]. For example, clinicians can easily decide to keep a tooth when periodontal conditions are optimum (with no bone loss) and the tooth has no decay.

The worse the tooth prognosis becomes, however, the more difficult it is to make easy clinical decisions. Clinicians face a dilemma when a tooth is, for example, severely periodontally affected or when periapical inflammation is present. In these circumstances, the dental practitioner is normally asked by the patient whether the strategy to preserve the tooth is worth trying. Nevertheless, patients

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have the right to receive all pertinent information to make, with their dentists, a shared decision on the best treatment option.

We will, therefore, describe in this chapter some important considerations when deciding between endodontic treatment and further restorative treatment or dental implants. We will organize the topics taking into account three main considerations that will serve as the basis for the decision-making – scientific evidence, patient perception, and cost-effectiveness of treatment. Finally, the quality of evidence will be appraised and a decision-tree-like scheme will be constructed.

## 5.1.2 Evidence of Efficacy

### 5.1.2.1 Periodontal Treatment and the Role of Risk Factors

There is a body of evidence of improvement of periodontal conditions and consequent tooth maintenance after active therapy [2–6]. The function of most teeth suffering from periodontitis may be preserved for a long time by use of noninvasive forms of treatment only [3, 4, 7–10]. Remaining supporting bone is one factor that can affect the long-term survival of the tooth [6, 10].

On average, the more severe the bone loss at baseline, the worse is the prognosis of an affected tooth. Furthermore, when more variables are taken into account when determining tooth prognosis, the long-term chance of survival of an affected tooth can drop substantially [6].

For example, posterior teeth with two or more roots may respond differently from anterior teeth to periodontal treatment, with, on average, a worse prognosis [6, 10, 11]. Systemic conditions, for example uncontrollable diabetes or patient behavior (for example smoking habits), may also reduce tooth prognosis after periodontal treatment.

### 5.1.2.2 Endodontic Treatment and the Role of Risk Factors

The pooled probability of tooth survival from 2 to 10 years after endodontic treatment ranges from 86% to 93% [12]. There are, however, factors that can increase or reduce the chance of survival. There is evidence that vital teeth have a better prognosis than necrotic teeth or teeth needing endodontic retreatment.

A study reported that, after 10 years, survival of vital teeth, measured at baseline, was 0.81 (i.e., proportion surviving 81%) compared with 0.68 (proportion surviving 68%) for non-vital teeth measured at baseline [13]. A recent retrospective study [14] also revealed that long-term survival and success of single crowns on teeth with vital pulp was better than for endodontically treated teeth.

Furthermore, teeth without periapical lesion are associated with a better chance of survival than those with periapical lesion at baseline [12]. Regarding baseline periapical status, a meta-analysis of three studies [12] revealed a significantly greater chance of survival of teeth without periapical lesion (odds ratio (OR) = 2.40; 95% confidence interval (CI) 1.11–5.18).[1]

[1]Confidence intervals taken, as customary, at 95%.

Some data suggest that the type of restoration after endodontic treatment can also interfere with long-term tooth survival. A recent systematic review [15] revealed that 10-year survival for crowned teeth was 18% higher than for teeth treated with direct restorations (81% (*SD* = 12%) and 63% (*SD* = 15%), respectively).

Although the literature has identified these risk factors for tooth survival after endodontic treatment, most data are based on studies with retrospective design [12], and, therefore, results should be interpreted with caution. Nevertheless, evidence presented here should be graded and weighted together with other factors to furnish guidance for clinical decisions.

#### 5.1.2.3 Dental Implant Survival and Its Risk Factors

The cumulative survival of dental implants supporting fixed partial dentures and single crowns is over 90% for follow-up ranging between 1 and 8 years [16]. Another meta-analysis showed survival of implants supporting single crowns of 96.8% (95% confidence interval (CI): 95.9–97.6%) after 5 years [17].

Despite this high survival, however, several systemic and local factors can negatively affect implant survival. Smoking habits and uncontrolled diabetes seem to increase implant failure compared with that for nonsmokers and systemically healthy patients [18–20].

Two other studies [21, 22] have revealed that smoking seems to be more associated with loss of implants in the early, rather than later, phases of the osseointegration process, although one systematic review with meta-analysis [23] also revealed more risk of implant failure for smokers even after 5 years.

Data regarding history of periodontitis and implant loss are contradictory. Some studies suggest that a history of periodontitis might be associated with lower implant survival [19, 24]. In contrast, other authors suggest there is no significant difference between implant survival in healthy and periodontal patients, but patients with more aggressive periodontitis might suffer more implant loss than those with other forms of periodontal disease [20].

Local factors can also be important in the survival of implants. One retrospective study [22] revealed that the probability of failure of implants located in the posterior maxilla was greater than that for implants placed in the anterior region of the mandible (*OR* = 6.83; 95% CI 2.65–17.57).

Survival is lower for implants placed in previously failed sites than for implants in pristine sites [25]. In an assessment of a large sample of implants (*N* = 6946), local risk factors were identified for early implant failure (up to abutment connection) [21]. For example, it was demonstrated that length of implant seems to play a role in implant survival.

Implants with a wide neck and less than 10 mm long were 71% more likely to be lost than implants more than 10 mm long (*OR* = 1.71; 95% CI 1.11–2.64). These implants were normally used as "rescue" implants (i.e., implants placed in compromised sites), however, which may explain the higher failure rate.

Peri-implantitis is also a frequent complication after implant therapy [26] because bone loss around the implant can, in severe cases, cause implant exfoliation. There is no standard therapy for peri-implantitis, and most data are based solely on surrogate endpoints, for example clinical attachment level and pocket probing depth [27].

A final remark on the survival of implants supporting single crowns concerns the scarceness of long-term data. Most long-term data involve other types of fixed prosthetic reconstruction, for example fixed bridges involving more than one implant or tooth and implant [28, 29].

## 5.2 Patient Perception of Endodontic and Implant Treatment

### 5.2.1 Information Transfer from Doctor to Patient

The patient's perspective regarding clinical decision making is pivotal in a shared decision. For ethical reasons, patients must receive detailed information on the prognosis of a tooth requiring endodontic treatment. Information such as quality of available evidence supporting the treatment planned and the strength of the clinical recommendation (i.e., how much the proposed treatment will affect the patient's life) is a condition sine qua non in current clinical practice [30].

The literature on patient perception after single-crown implant therapy is scarce or even nonexistent. Most literature on patient perception of implant treatment is based on prosthetic removable constructions with implants, in the form of overdentures [31, 32].

One report [33] concludes that at least 80% of patients receiving implant-supported single crowns were satisfied or extremely satisfied after therapy. In the same way, a study assessing the quality of life of patients after endodontic treatment [34] demonstrated that factors in the OHIP questionnaire improved after treatment. Although studies have assessed patient satisfaction with both endodontic and dental implant treatment, no study has directly compared both therapies.

### 5.2.2 Comparative Cost-Effectiveness of Endodontic and Dental Implant Treatment

It is increasingly being recognized that cost-effectiveness is an important aspect of decision making in dentistry [35–38]. By way of illustration, imagine a patient who has a compromised upper molar. Intuitively, a clinician will ask about the clinical prognosis for each of the treatment options available. At best, the corresponding information process will rely on an evidence-based approach and will tell us which of the interventions has the best clinical prospects.

If we were to live in a world without budget restrictions, we would always go for the treatment which is most effective. Most often, however, we face monetary limitations. In the end, we need to decide whether the higher effectiveness of a treatment is worth the additional costs in comparison with the next best alternative.

In other words, we must identify the most cost-effective, i.e., most efficient, treatment strategy. Generally speaking, health economic evaluation seeks to maximize the benefits which can be obtained from limited resources for health care or, vice versa, to minimize the costs in order to achieve a specific health outcome [39].

Again, referring to our example of a compromised upper molar, the first treatment decision to make is whether preservation/replacement of the tooth is even desirable. When there is no clinical need for tooth preservation/replacement (no positive effect on tooth function attributable to treatment) and the patient does not perceive any detrimental effect of having a missing tooth, the choice of not providing treatment can clearly be identified as the most cost-effective approach because its lower cost results in no reduction in health outcome.

When there is an additional health benefit of having a tooth or a replacement (compared with having a missing element in the row of teeth), however, three general treatment alternatives can be considered for a compromised upper molar.

### 5.2.3 Endodontic Treatment

First, endodontic techniques seek to preserve the tooth. Here, orthograde root canal treatment can be regarded as the mainstay of therapy. The procedure involves:

- Trepanation of the tooth
- Preparation of the canals
- Irrigation
- Application of an anti-inflammatory drug
- Definitive root filling, when the tooth is completely asymptomatic

Note that, over time, re-treatment may become necessary when this first-line endodontic treatment can no longer be considered adequate. If, moreover, the periapical lesion is very large, the first-line endodontic treatment described may no longer be sufficient.

An apicectomy could then serve as the last resort for preservation of the compromised tooth. Also, when preserving the tooth, it may at some stage become necessary to improve its prognosis by means of periodontal treatment. Overall, endodontic treatment of a compromised tooth seems to entail huge cost differentials depending on the specific conditions under which treatment is performed.

#### 5.2.3.1 Fixed Partial Dentures

Second, bridgework could be placed, implying that the compromised tooth is extracted and adjacent teeth are incorporated into the restoration. Although replacement of the missing tooth, per se, can be regarded as beneficial to the patient, bridgework may have the negative side effect of sacrificing tooth material of an adjacent tooth which may previously have been pristine.

Note that over time, and depending on the survival of the bridge, renewal might become necessary. Different dental materials (e.g., gold compared with full ceramic restorations) may be of different esthetic benefit to the patient but will also contribute to substantially different treatment costs.

#### 5.2.3.2 Implantology

Third, a missing tooth can be replaced with an implant. Despite a steadily increasing body of clinical evidence which suggests that dental implants can be regarded as a

reliable treatment alternative, long-term evidence on implant survival and associated complications is still sparse.

One such uncertainty in predicting the long-term effectiveness of dental implants can be seen in the case of peri-implantitis, for which the treatment outcome is not yet entirely conclusive [40].

Moreover when identifying the costs of implant treatment, it is essential to take into account potential costs of bone and tissue augmentation techniques in advance of, or after, implant insertion. Specifically, for posterior teeth in the maxilla, it may be necessary to take account of the costs of sinus floor elevation in cases where initial vertical bone height is reduced [41]. Finally, costs relating to prosthetic complications such as failure of single crowns placed on implants should be also taken into account.

Despite some complexity in identifying the precise costs and health outcomes of these four general treatment options, decision analytical modeling is further complicated by the fact that the above treatment options cannot be regarded as mutually exclusive.

In other words, one treatment option could follow another and, considering the whole life of a patient, a vast variety of potential treatment pathways emerges. One way of managing such a complex decision analytical framework is provided by Markov modeling [42].

This assumes a patient occupies one of a series of defined health states at a given time. Each health state is associated with a specific cost and health outcome and, as time elapses, transitions from one health state to another occur with preset probabilities.

Within this framework, the expected costs and health outcomes can be calculated when weighting the averaged time duration in each health state by the associated costs and health outcomes. In this sense, a recent paper [43] has established a Markov model for comparing the lifetime cost-effectiveness of endodontic and implant approaches for treatment of an irreversibly pulpitic maxillary incisor.

The results obtained suggest that orthograde root canal treatment is highly cost-effective as a first-line intervention whereas surgical re-treatment is not. Moreover, implant placement is suggested as a third-line intervention in cases where endodontic re-treatment fails. Research is needed to adapt Markov modeling to the more complex setting of a compromised upper molar (as described above).

## 5.3 Quality of Evidence and Strength of Recommendation

### 5.3.1 Different Types of Recommendation and Their Effects

A system that deals with evidence of different quality and weighs the available evidence together with other factors is necessary for shared decision making in clinical dentistry. The Grading of Recommendations Assessment, Development, and Evaluation (GRADE) system assesses important variables used to rate the quality of evidence and the strength of a recommendation [44].

The quality of evidence is first rated on the basis of study design. For example, randomized controlled trials (RCTs) generate the highest level of evidence on a specific topic [45]. However, other factors such as study limitations or reporting bias can downgrade our confidence in the body of evidence.

Therefore, an RCT with limitations can be rated as moderate evidence. In contrast, studies at a lower level in the hierarchy of evidence, for example case series, might be upgraded from low to moderate level of evidence when, for example, the magnitude of the effect of treatment is very large.

Strength of recommendation reflects how much we can be confident that treatment effects outweigh the side or undesirable effects of proposed therapy. The GRADE system classifies the strength of recommendation as strong or weak [30]. A strong recommendation means that most patients would probably choose the proposed clinical option taking into consideration the four factors that affect the strength of recommendation.

A weak recommendation means there is a trade-off between desirable and undesirable treatment effects, and patient choice will vary, depending on their values and preferences.

#### 5.3.1.1 Quality of Evidence in Studies of Endodontic Treatment

Most studies of endodontic therapy were retrospective, and therefore the quality of evidence is low. There is, however, a body of evidence suggesting that conservative endodontic therapy is effective, and it is able to maintain the long-term function of a tooth; we can, therefore, consider this body of evidence as of moderate quality.

#### 5.3.1.2 Quality of Evidence in Studies of Dental Implant Treatment

In the same way, most long-term studies of implant treatment with the objective of substitution of an extracted tooth by a dental implant and single crown are retrospective in design. Following the same criteria as in studies on endodontic therapy, the whole body of evidence regarding dental implant treatment is indicative of good prognosis for unitary implants with single crowns; we can, therefore, consider this body of evidence as being of moderate quality.

### 5.3.2 Decision-Tree-Like Scheme

We have constructed a scheme based on factors that determine the strength of a recommendation: quality of evidence, balance between treatment effects and side effects, variability in values and preferences, and cost-effectiveness of the therapy. The decision-tree-like scheme would suggest that a smoker patient suffering from periodontitis will probably gain more value from conservative endodontic treatment because risk factors may substantially affect the long-term survival of implants. In contrast, a systemically healthy and nonsmoker patient with no money concerns (i.e., there is no restriction of the cost of therapy) might decide on implant therapy as a strong recommendation. The same patient with money concerns might accept endodontic treatment as a strong recommendation.

## 5.4 Conclusions

The decision to keep an endodontically affected tooth or to replace it with an implant and single crown may be challenging. Various systemic and local factors must be taken into consideration if a reasonable clinical decision is to be made. The decision will also depend on another important factor – the number of teeth available might imply a need for more complex prosthetic restorations. In some cases, strategic extraction of a questionable tooth is necessary, even when long-term maintenance is still possible.

The GRADE system may be a valuable tool enabling clinicians to improve and achieve transparency in a shared decision-making process.

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# The Translation Evidence Mechanism

6

Janet G. Bauer and Francesco Chiappelli

**Core Message**

Currently, best evidence is a concentrated effort by researchers. Researchers produce information and expect that clinicians will implement their advances in improving patient care. However, difficulties exist in maximizing cooperation and coordination between the producers, facilitators, and users (patients) of best evidence outcomes. The Translational Evidence Mechanism model is introduced to overcome these difficulties by forming a compact between researcher, clinician, and patient. With this compact, best evidence may become an integral part of private practice when uncertainties arise in patient health status, treatments, and therapies. The mechanism is composed of an organization, central database, and decision algorithm.

## 6.1 Introduction: Evidence-Based Research

### 6.1.1 Evidence-Based Research and Comparative Effectiveness Research

Advances have been made in reasoning the value and application of best evidence to patients and society. Current mechanisms include comparative effectiveness research (CER) and evidence-based research (EBR). CER may be independent or synergistic with EBR. CER both conducts studies and uses systematic review analyses to compare similar treatments or procedures in maximizing the choice of the

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most effective cost/benefit option within the context of new evidence. EBR uses similar analyses; however, the result is to determine best evidence in maximizing best outcomes, not costs.

Since the agenda for health care reform has become a major stimulus for political action, CER is being used by governing health, budget, and funding agencies to maximize effective, efficacious, and cost containment service coverage plans to assure the best health care system for all within the dynamics of change in knowledge and treatments [1, 2]. Clinicians, on the other hand, use these advances to promote health and its maintenance as well as disease control in individuals, their patients.

While CER and EBD assist in reasoning individual health and treatment choices during shared decision-making with service providers, a mechanism to maximize cooperation and coordination between those who produce and those who apply evidence is lacking. Currently, best evidence is a concentrated effort of researchers who produce or analyze existing evidence for publication in the professional literature.

Once published, researchers expect clinicians will implement their advances in knowledge and technology in improving patient care. This is rather presumptuous for two reasons. Published evidence is based on the merit of the methodology and the applicability of results in advancing health care for the "average patient."

The evidence is rarely structured for use by the clinician for shared decision-making during individual patient encounters within the scope of local factors and population characteristics. Secondly, researcher expectations disallow the acknowledgement of practice demands that must be met by service providers in providing health care services.

Clinicians are expected to search over numerous resources and media, organize evidence, analyze that evidence, and produce decision, utility, and cost outcomes, then apply these outcomes in customizing individual treatment and therapy choices. For clinicians, these expectations give salience to the time and demand constraints that providers experience when servicing patients, particularly in times of economic conservatism. Unfortunately, clinicians want to utilize best evidence in improving patient care for accomplishing practice goals and improving patient care – a conundrum waiting for creative solutions.

The Translational Evidence Mechanism (TEM) model was developed to facilitate and maximize the production and application of best evidence for use in clinical practice and improving individual patient care. TEM explains the development of data, its transformation into best evidence, clinical relevance, and meaning in practice.

This mechanism proposes to understand, define, and characterize the underlying process involved in clinical decision-making for CER and EBD. For health care, this mechanism defines the compact between researcher (research synthesis), clinician (clinical expertise, local long-term monitoring and implementation of evidence), and patients (patient choice and compliance) in providing the essential components of the biological, behavioral, and social interventions involved in clinical decision-making related to health care delivery. With this compact, best evidence may become an integral part of clinical practice when uncertainties arise in patient health status, treatments, and therapies.

### 6.1.2 Translational Evidence Mechanism

The Translational Evidence Mechanism (Fig. 6.1) explains the dynamic by which best evidence is transformed into consumer choices; in the case of health care, providing needed analyses of differing health care and service options for patients in deciding the best clinical decision. However, the mechanism is non-disciplinary specific; in other words, it may be applied to any discipline that services clients or patients, researchers, and policy-makers.

For any discipline-centered evidence-based decision-making, this mechanism consists of three components: Translational Evidence Organization, central database, and decision-making algorithm. The outcome of this mechanism is to provide end users – patients or clients – with current, best evidence to make informed and optimal decisions regarding life choices, choices specific to any discipline in which consumer-based wants, needs, and desires are expressed.

The product of such a system is typically called a guideline that takes into account the probability (or odds ratio) of an event or events, the human preferences (expressed as benefits or trade-offs) attributed to these events, and their costs. Similarly, researchers and policy-makers may be granted access to evidence for future research or policy needs.

Evidence regarding the "average patient" is helpful to researchers investigating current issues of discovery, to decision-makers in developing policy of sociopolitical and socioeconomic importance, monitoring health care systems including private practice, and determining care and cost-effective therapies and treatments. Best evidence assists by protecting society in reducing health inequalities and potentials for harm.

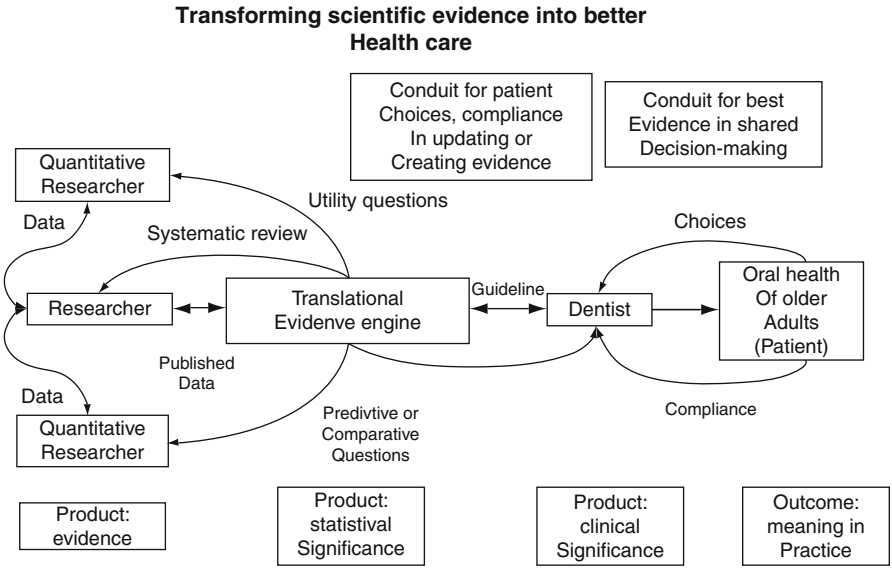


**Fig. 6.1** Translational Evidence Mechanism: transforming scientific evidence into better consumer choices

### 6.1.3 Translational Evidence Organization

The Translational Evidence Organization develops, verifies, maintains, and updates current, best evidence for end users in their consultations with service providers. This organization (Fig. 6.2) requires a workforce and input process that initiates an inquiry regarding uncertainties in consumer decision-making.

This inquiry drives this organization to provide in real-time, effective, and efficient decision-making; in other words, in a manner that demands current best evidence for immediate decision-making in determining the optimal cost/benefit choice for the consumer. If best evidence does not exist currently, the organization initiates an internal inquiry to develop new evidence-based on consumer-based wants, needs, and desires.

The primary purpose of the Translational Evidence Organization is to arbitrate published evidence and, in its absence, identify research that needs to be conducted by organizational affiliates or the research community in producing needed data. The Research Administrator and Evidence-Based Dentistry Team are responsible for this purpose.

The Comparative and Translational Researcher (Translational Researcher) coordinates with IT Research and Development administrator to vest a central database with evidence defined by templates of inputs and outputs that may be stratified by geographic, discipline, population, and/or other categorical needs. Outputs consist of arbitrated decision data produced by the quantitative researchers, including related utility (attitudes, beliefs, and preferences of both providers and consumers), produced by qualitative researchers, and cost data.

Arbitrated best evidence meets the rigorous standards of statistical significance, the soundness and generalization of information, or whether findings can be applied to similar consumers in similar settings. Outputs are stated in terms of validity and reliability of study design to express confidence in providing best evidence.

For health care, all are displayed in clinical practice guidelines (CPGs). The CPG is the vehicle, or professional standard, that manages data for use in clinical practice.

Additional post arbitrators of best evidence include the provider (clinician) and consumer (patient). The clinician is concerned with clinical significance – whether research findings can be applied to individual patients. Dental clinicians, dentists, make judgments that may weight best evidence differently from researchers. Initially, clinical significance of decision data is coordinated with dentists in developing nationally, regionally, or locally relevant best evidence.

The dentist is provided a CPG with decision and utility best evidence and locally provided cost schedules. The dentist provides an assessment of the clinical significance of the CPG based on practice and local factors. This assessment is developed from applying knowledge logically based on concepts learned during training and implicitly in rendering health services based on experience and patient characteristics of well-being.

During care delivery, dentists perform assessments, evaluate services needed, and develop plans for treatments and therapies. In providing dental care services, the dentists may contribute to the understanding of the “when, where, and how”

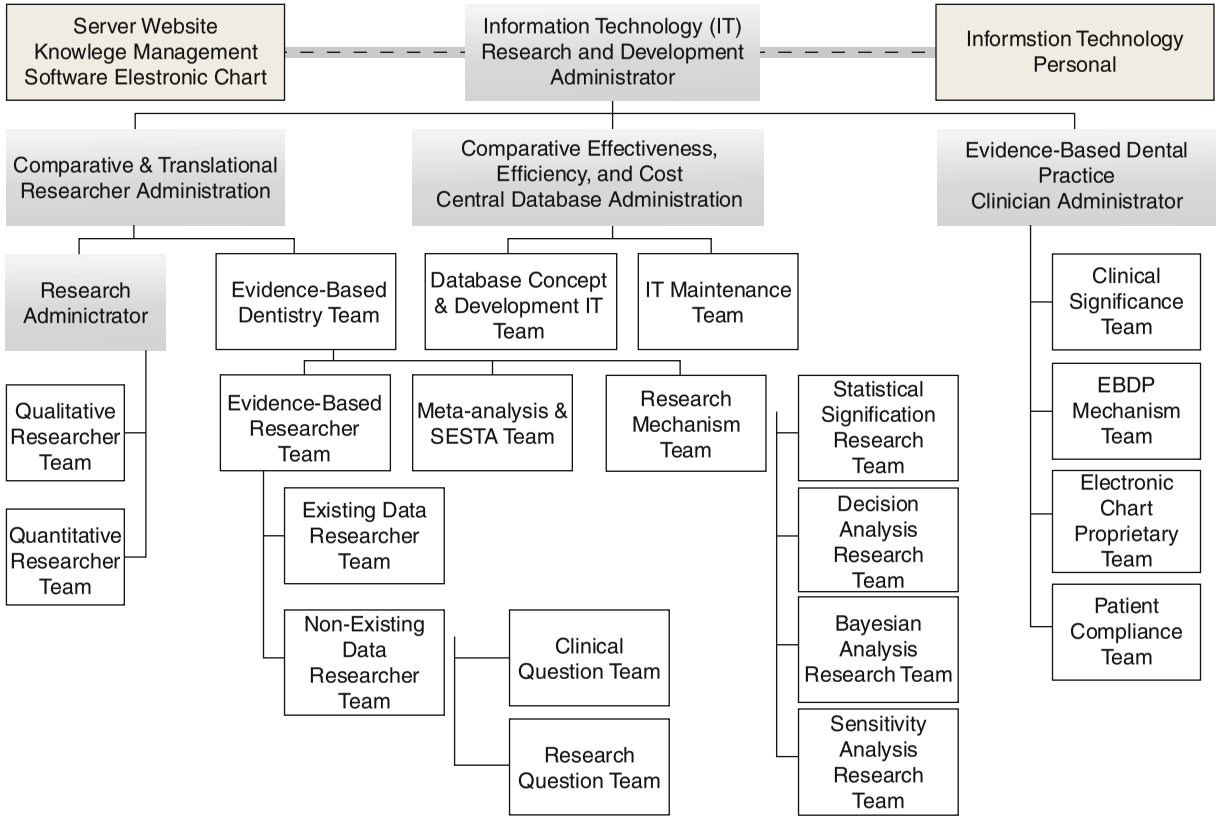


**Fig. 6.2** Translational evidence organization and manpower

of knowledge. The dentist's evaluation of clinical significance is used by the translational researcher to reject or modify the clinical practice guideline or to re-identify and conduct investigations that produce other clinically relevant data.

Personal and professional experiences, values and preferences, and appropriate practices, as well as patient well-being, quality of life (QoL) issues, and costs, weigh heavily on whether best evidence is used in clinical decisions. Thus, dentists are able to predict clinical outcomes in weighing risks against benefits and costs for individual patients. Dentists may also make relevant standards of care in their local practice and for specific patient population characteristics. These clinical validations are necessary to translate research data into clinically useful data for patient care.

Clinical significance also addresses the importance of the evidence that takes into consideration the long-term multifaceted monitoring of evidence in the context of human behavior (patients). However, clinical significance may vary between dentists and between patients. This difference results because dentists, as well as patients, make judgments that weigh differently personal and professional experiences, values and preferences, and appropriate practices.

In other words, judgments of risk and benefits vary because of differences in weights given to values and preferences that also include costs. All is important for patients in accepting best evidence in their acquiring the highest level of cost-effective services, either through fee-for-service or as a defined benefit of their dental insurance plan.

### 6.1.4 Patients as Consumers

Patients are typically categorized as the consumers of products and services and not the developers or guarantors of knowledge. Patients become the "conceptual subject" to which best evidence is applied and quantitative and qualitative outcomes are measured.

However, patients may be advocates or adversaries of evidence. Patients may also exert demands on evidence to meet specific, personal needs. They may exert influence on the development and application of knowledge that does not necessarily meet acceptance criteria of researchers and dentists, but serves a personal need.

They may also exert pressure to deny the development and application of knowledge that is contrary to their philosophical beliefs. Even in the profession's best efforts of informing patients with best evidence and using clinician expertise to communicate individualized, effective treatments, patients ultimately decide if treatment regimens are adhered to or rejected outright. Patient adherence, modification, or rejection of best evidence in treatment scenarios provides the translational researcher with its meaning in practice.

The translational researcher uses these evaluations in updating clinically relevant data. These updates are processed using decision, Bayesian, and sensitivity analyses within the central database.

Thus, the translational researcher is the final arbiter of evidence. The translational researcher functions, simplistically, to translate basic research language into

the language of the clinician and patient. In other words, basic research is assessed, evaluated, and disseminated to the clinician in a means usable for shared decision-making.

The product of evidence-based dentistry is the published clinical practice guideline. While there is no requirement that the published clinical practice guideline be implemented in private practice, dentists could use best evidence to offer their patients best practices in a state-of-the-art organization and facility. This requires that dentists review and effect personal, behavioral changes to accomplish care and service advances.

## 6.2 Central Database

### 6.2.1 Technology in Evidence-Based Dentistry

The central database is the repository of evidence, organized and stored utilizing evidence-based research knowledge management software (EBRsoftware). EBRsoftware [3] is maintained by information technologists and integrated within the electronic patient chart. Both health information technology and the Internet, as a vehicle for information technology, has become the most cost-effective mode of information transfer [4, 5].

It is this inclusive and reciprocal participation of all decision-makers that accomplishes improvements in clinical care. The structure of the central database consists of a primary network. This primary network contains all vested evidence. The network may then be partitioned into subordinate databases. Information is then filtered and segregated per subspecialty information.

Subspecialty information is that best evidence used in specific populations, geographic locales – national, regional, and specialty – and local secondary networks, specialty procedural treatments, quality assurance, costs, and care delivery, including facility or equipment improvements. These subspecialty databases may facilitate access based on frequency of usage.

### 6.2.2 Initiating the Mechanism

During the examination appointment (initial or recall), the dentist interviews and assesses the patient for his/her health and oral status. The information is inputted into the electronic chart. Once assessments have been completed and the evaluation process of needed services or maintenance regimens begins, the dentist discusses with the patient treatment options in arriving at a treatment plan.

If presenting conditions, patient characteristics, and treatment outcomes are uncertain in their benefits and harms, then the dentist participates with the patient in shared decision-making to provide informed consent, assisting the patient in making the most optimal clinical decision that meets their needs and choice in accomplishing oral health care and wellness.

Using EBR software, the dentist accesses a clinical input form through the electronic chart to assist in forming a clinical question regarding the treatment options and their outcomes. The input template is based on the PICO question, a standardized format for initiating evidence-based research. The PICO input form (Fig. 6.3) is completed by the dentist based on patient responses.

When submitted, the clinical input form will interact with the central database. Because this form is being sent through the electronic patient chart, patient histories, assessments, evaluations, and practice cost schedule are collected and all information is encrypted prior to integrating with the central database (Fig. 6.4). This encryption uses technologies to insure security, privacy, and uphold the requirements of HIPAA.

Based on the query, the EBR software manages relevant data to return decision and utility best evidence coordinated with the practice cost schedule in a clinical practice guideline template (Fig. 6.5). The CPG is returned dated and specified as to expiration date of the evidence provided, the functional status (functionally independent, frail, or functionally dependent) of the patient to which the evidence applies, and, if available, the significance (statistical and clinical), utility ranking, and meaning in practice of the evidence.

Other visual aids include links to "customize," changing the components of the CPG to accommodate different choices or correct mistakes, and "learn more," to exhibit the documentation that produced the CPG and its relevant bibliography. The returned CPG is interactive in that it provides stratified risk scenarios for high, equipoise, or low risk takers that may be accessed and "gamed" through a drop-down menu.

Basically, the CPG provides a minimum of two treatment scenarios: treatment and no treatment with their respective outcome (best evidence) probabilities; if more have been requested through the clinical input form, then those will be displayed in their entirety. Along with this decision data, utilities of each outcome are provided. These utilities are based on a scale from 0 to 9, a standard scale used in utility research.

With the patient's involvement, returned quantitative and qualitative best evidence is organized into decision, utility, and cost data for each possible option/outcome to assist in shared decision-making. If utility rankings, based on the "average patient," are not acceptable to the patient, these may be changed through drop-down menus, reflecting personal preferences, values, and beliefs regarding the presented outcomes.

Thus, the patient may change, or "game," rankings to compare and test different treatment scenarios in optimizing the clinical decision or provide assessments to compare personal trade-offs between options with the "average patient." If so desired, the dentist may manipulate outcome reimbursements to provide different cost outcomes. All changes are immediately recalculated to demonstrate different treatment scenarios for enhanced informed consents.

The objective of patient "gaming" is to optimize the clinical decision. Accompanying the CPG exhibit is the decision analysis statement. This statement provides a written interpretation or meaning of the treatment scenarios and their calculations (Fig. 6.6a–c).

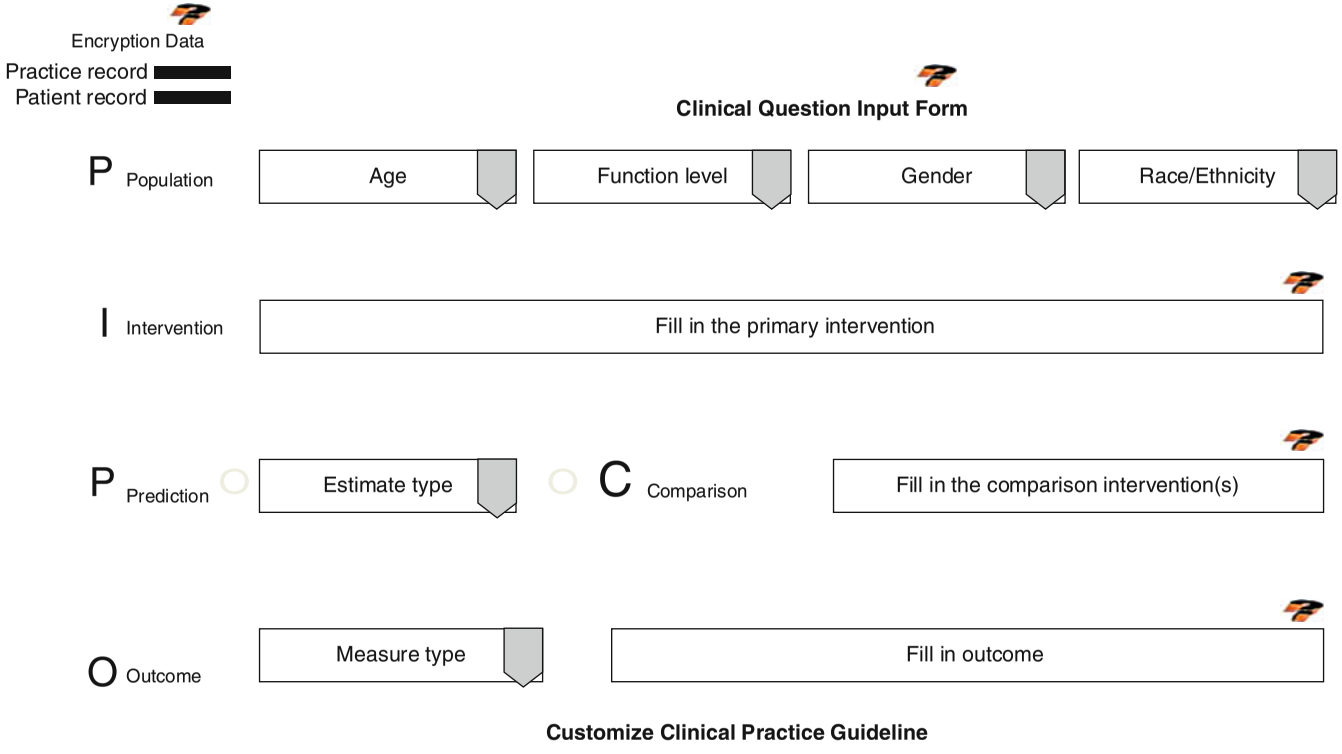


**Fig. 6.3** Clinical question input template

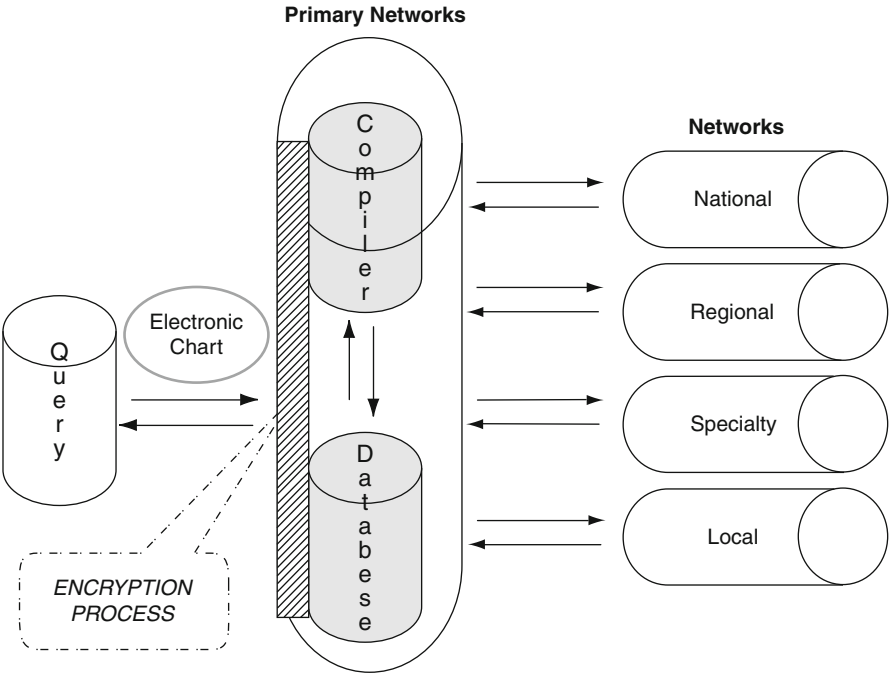


**Fig. 6.4** Central database structure

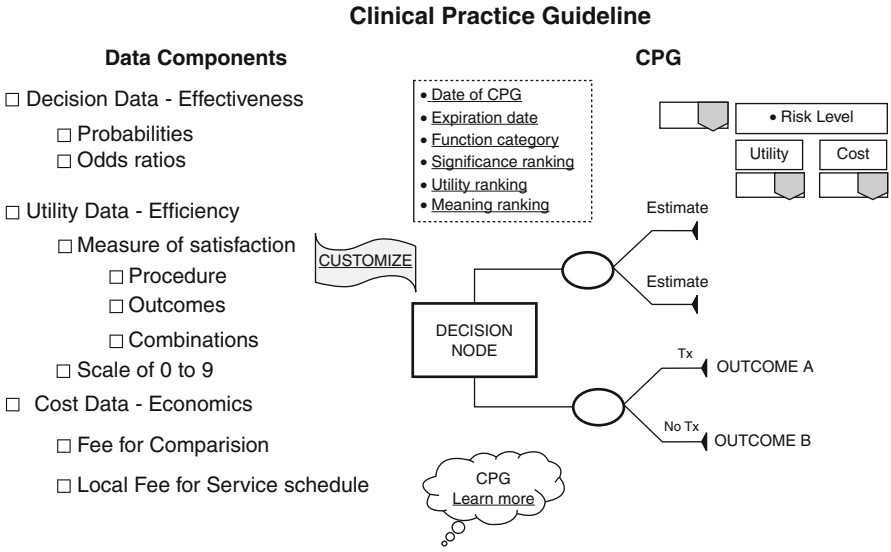


**Fig. 6.5** Clinical practice guideline

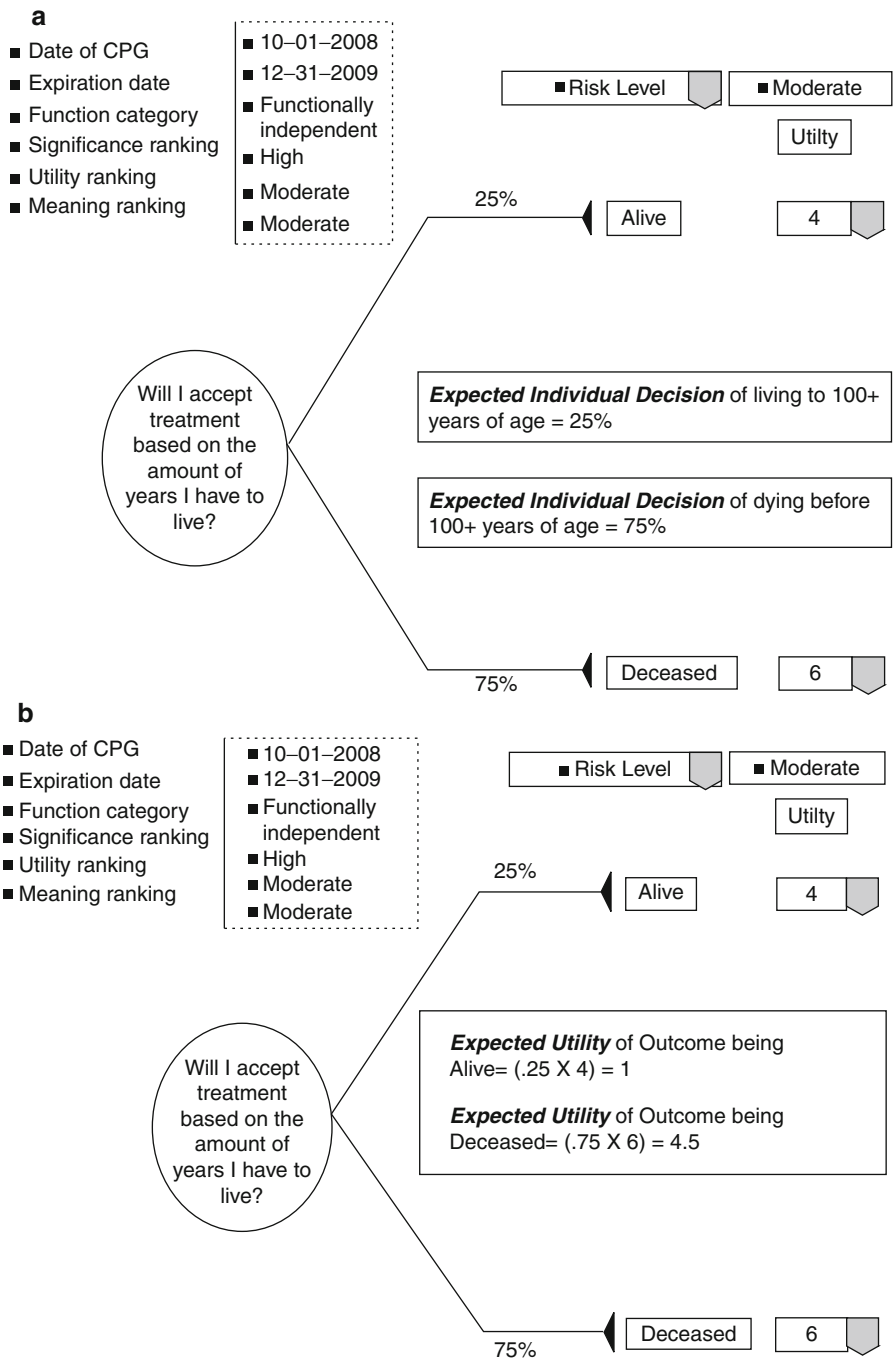


**Fig. 6.6** (**a**) Example of decision analysis using decision data. (**b**) Example of decision analysis using utility data. (**c**) Example of decision analysis interpreting the CPG using decision and utility data

**c** INTERPRETATION BASED ON TARGET CATEGORY

**DECISION ANALYSIS FOR PATIENT-MODERATE RISK TAKER**

**Meaning of Analysis**
**Determining the Optimal Clinical Decision**

From the analysis, best evidence regarding the decision to be made is presented. The data is combined in a way that the value of the decision may be shown. The contex of the decision is presented with respect to like decision or trade-offs made by others in the patient's average age cohort. Using this comparison contex, the patient may make h/her own Optimal Clinical Decision. For the patient, then:

While the probability of living to 100+ years of age is 25%, the benifit of that life expectancy is 1 out of a scale of 0 to 9.

While the probability of being deceased before reaching 100+ years of age is 75%, the benefit of dying sooner is 4.5.

Thus, I the patient can see that while I have a fair chance of living to 100+ years of age, the quality of life of those additional yesrs is minimal. So, while not pleasant to consider, I would be better off if I died sooner than later. Am I comfortatable with that decision or I want to differ from my average age cohort decision?

**Fig. 6.6** (continued)

The decision analysis of the CPG is done:

- Firstly, by interpreting for the patient the probability of the outcome
- Secondly, multiplying utility data by the probability of the outcome in interpreting the service benefit to the patient
- Thirdly, multiplying the probability of the outcome by the treatment cost in determining the economic choice difference between outcomes (not applicable in Fig. 6.6)

With these analyses, patients may determine their optimal treatment option. The example decision analysis in Fig. 6.6 may appear absurd as a stand-alone decision. However, on reflection, the information may be helpful in determining a patient's access to dental services. The type of clinical question raised in Fig. 6.6 becomes relevant when one considers that access to dental care by the elderly remains low. This trend increases with age and is almost two and half times greater than that for younger adult age groups [6].

The resultant patient analysis and optimal clinical decision is returned to the central database to be retrieved by translational researchers to develop new research questions or modify existing evidence. Having an efficient decision-making process has an additional benefit of involving private practice as a unit in clinical research without disrupting normal patient flow or care. Conversely, clinicians may compare local-based practice norms, patient characteristics, and standards to those derived on a regional and national basis.

Each derived option may be logged by its local- or cultural-based probabilities for each reporting research unit. Once processed, the results may be transferred to

the central repository. Within the central database, probabilities are updated using Bayesian analyses. Sensitivity analysis determines which components of the CPG have the greatest impact on the clinical decision.

This analysis may also determine the effects of changes in one of the components or two or more of the components of the CPG. For the researcher, sensitivity analyses are helpful in quantifying and qualifying trends evidenced by data retrieved from private practice and compiled on a local, regional, or national basis. In this way, policy and areas of public concern in health care along with the analysis of current and future trends may be discovered.

For the dentist, sensitivity analyses are used to confer a sense of understanding regarding patient choices and shared decision-making. Updates may then be sent back to the dentist in learning how patient decisions differ from the "average patient" in the practice locale or change over time. This reciprocation of evidence is done through the electronic chart. It is facilitated with alerts or flags to disseminate improvements in understanding as they relate to the patients, treatments, and care delivery.

If best evidence is not available, then CPG forms are provided to the dentist to work with the translational researcher in initiating needed research. In this process, new research is based on clinical questions, or questions arising from real situations that are important to health care and its delivery.

## 6.3 Meaning in Practice of the Evidence

### 6.3.1 Quality and Quantity of the Meaning of the Evidence

Quantifying and qualifying the meaning of evidence begins at follow-up assessment visits, periodic dental examinations. For these assessment visits, the patient provides updates to decision data through patient compliance and outcomes dependent on healthy lifestyles and wellness.

Utility data is updated by patient preferences and value of the services rendered or recommended maintenance regimens. With input from the dentist, the translational researcher uses these periodic assessments to update the CPG in identifying new areas of research or improving its usefulness in private practice through sensitivity analyses.

Thus, the dentist acts as a conduit for the researcher in providing local data regarding patient choices, compliance, and treatment outcomes in updating or producing new evidence.

### 6.3.2 Health Information Technology (HIT)

The Translational Evidence Mechanism is dependent on information technologists who are integral to the development, maintenance, and improvement of the central database that is the repository of best evidence. Technologists supervise and participate in web development, installation, service, modifications or upgrade improvements, and computer, mainframe network, and communication equipment repair.

Technologists problem-solve system failures or dysfunctions through a central help desk, along with assessing user needs and training users in effective use of applications through prepared documentations and assistance. Technologists are knowledgeable in the basic concepts of digital data communications and the principles of electronic data processing, especially in the areas of digital electronic logic and computer time-sharing operations.

Information technologists plan, develop, research, and maintain operating systems and software. They also work with program or product developers and end users in software development, maintenance, and upgrade improvement. Information technologists serve as a technical resource to resolve problems (problem-solving and trouble shooting), make repairs, and assist end users in the use of computer or software functions.

### 6.3.3 Decision-making Algorithm

A decision-making algorithm assists decision-makers in managing a decision process. Similar to evidence-based research, an algorithm provides a systematic understanding of decision strategy management. The basic structure of the algorithm is the decision tree (Fig. 6.7).

A decision tree is a visual representation of instructions to approach and manage a complex decision process. For health care algorithms, clinical practice guidelines become decision tree termini. An example of a health care algorithm for older adults is the Clinical Decision Tree of Oral Health [7].

The Clinical Decision Tree of Oral Health is used to assist decision-makers in making effective and efficacious decisions regarding treatment options that best optimize treatment selection, personal utilities, and goals in improving oral health. Likewise, a decision-making algorithm structures the organization of evidence in the central database registry.

It is by the decision-making algorithm that evidence is managed in storing and providing data when an inquiry is received. It is this structure that constitutes the ordered progression of human decision-making concerning outcomes, their comparisons, and associated information to make inferences regarding preferences (values, benefits, or trade-offs) and affected costs.

## 6.4 Conclusion

The Translational Evidence Mechanism requires three components for effective production, validation, administration, and continuous monitoring of new knowledge and technology. Since best evidence is used in informed consents regarding optimal treatment decisions, it must stand to scientific rigor, be clinically relevant, and produce changes in practitioner and patient behavior.

The Translational Evidence Mechanism accomplishes this in a team approach to best evidence used in decision-making for assisting consumers in meeting their

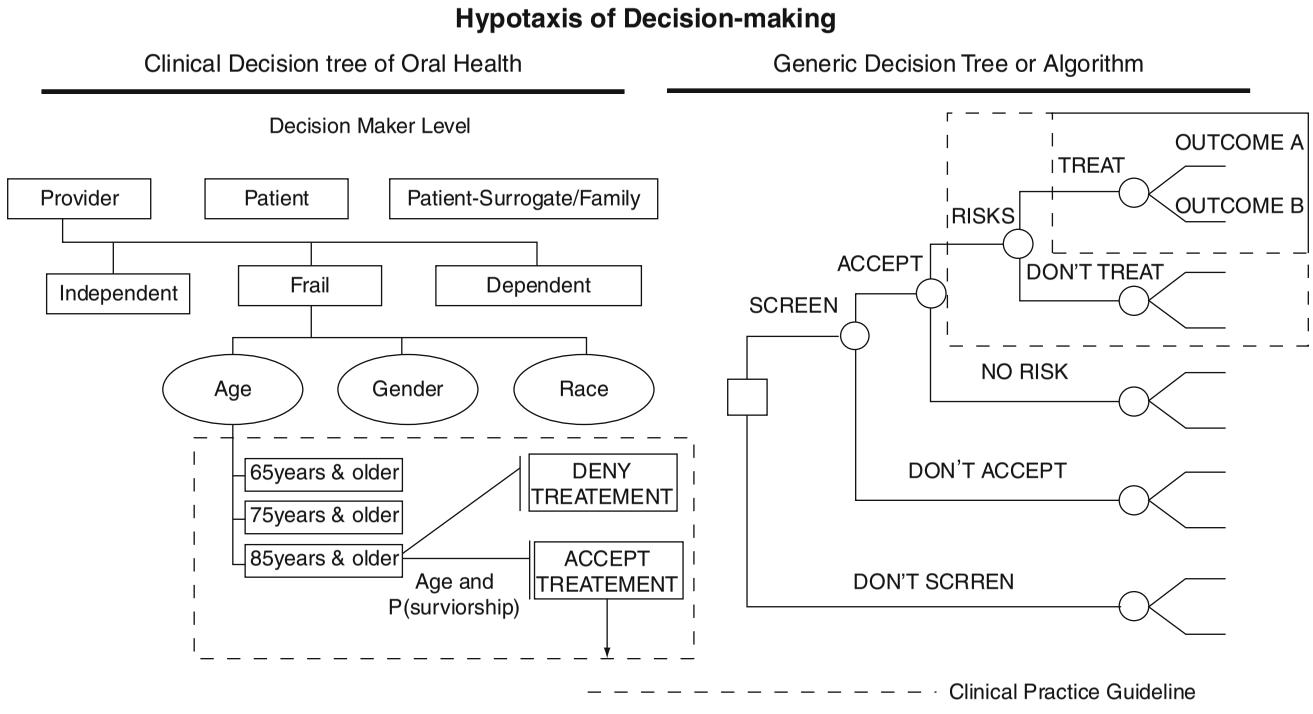


**Fig. 6.7** Hypotaxis of decision-making

needs, wants, and desires. Using evidence-based dental practice as an example, this mechanism includes an organizational structure, a central database, and decision-making algorithm.

While the translational researchers arbitrate best evidence, quantitative and qualitative researchers, dentists, and patients participate in this arbitration. The interdependence of roles is necessary to develop, assess, implement, analyze, and upgrade or revise clinical practice guidelines in translating research into patient care.

Thus, the Translational Evidence Mechanism uses evidence-based research methodologies in optimizing interdisciplinary resources for promoting oral health and preventing disease in individual patients by integrating best evidence, clinical experience, and expertise, and patients in clinical decision-making, best evidence validation, and practical implementation.

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# Part III

# Evidence-Based Nursing

# Evidence-Based Treatment and Management of Asthma and Pressure Ulcers

7

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**Core Message**

Asthma and pressure ulcers are chronic illnesses affecting millions of people and much more so with the aging population. Effective management and treatment of asthma and pressure ulcers are major expectations as resources are limited in today's health-care environment. Evidence shows that prevention, symptom control, and patient/family/staff education are necessary elements to improve health-care outcomes.

## 7.1 Introduction: Chronic Illness

### 7.1.1 Asthma and Pressure Ulcers

The prevalence of chronic illness is increasing worldwide, hence it is imperative that we understand the risk factors, evaluate the effectiveness of nursing care, and determine the best available evidence-based strategies to manage and treat them. Chronic illness accounts for billions of dollars in health-care cost per year. By the year 2030, it is projected that 150 million Americans will have one or more chronic illness [1]. Asthma and pressure ulcers will be reviewed and discussed in this chapter.

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Chronic lung diseases (including asthma) cause $154 billion in health-care costs each year [2]. Asthma alone is responsible for roughly two million emergency department visits, nearly 500,000 hospital admissions, and close to 4,000 deaths [3, 4]. The number of people diagnosed with asthma grew by 4.3 million from 2001 to 2009. Asthma costs in the US grew from about $53 billion in 2002 to about $ 56 billion in 2007, about a 6% increase [5]. The American Lung Association ranks asthma as the seventh chronic health condition in the USA and the leading chronic illness in children.

Pressure ulcer is another major chronic health problem in the USA today. As asthma costs a considerable amount of health-care dollars, so do managing and treating pressure ulcers. The treatment and management of pressure ulcers has been estimated to be between 5 and 8.5 billion dollars annually. This overall annual cost includes the cost of hospital-acquired pressure ulcers, which is between 2.2 and 3.6 billion dollars [6]. To reiterate the cost, to heal a single full-thickness pressure ulcer requires as high as $70,000.00. Pressure ulcer is a chronic disease that can be community or hospital acquired.

The costs in dollars for these chronic illnesses do not take into account the added pain and discomfort, as well as the diminished quality of life, incurred by patients. Evidence-based review of the literature indicates that aggressive management of pressure ulcer and asthma improves outcomes for patients, lowers overall treatment costs for patients, lowers overall treatment costs for payers, and reduces the indirect costs to society.

## 7.2 Overview of Asthma

### 7.2.1 Asthma as an Illness

Asthma is a chronic disease with acute exacerbations characterized by hypersensitivity reactions in the individual's airways. The body of susceptible individuals overreacts to a stimulus; the stimulus is referred to an antigen or trigger. There is no clear single cause of asthma. A genetic inclination to produce immunoglobulin E is associated with the cause of asthma [7].

Predisposing factors include environmental factors such as exposure to indoor and outdoor allergens. Indoor allergens are domestic mites, furred animals (dogs, cats, and mice), cockroaches, fungi, mold, and yeast. Outdoor allergens are pollens, fungi, molds, and yeast. Other factors such as viral infections, exposure to occupational sensitizers, passive and active tobacco smoke, indoor/outdoor air pollutants, and western diet can all be contributing factors to asthma.

Asthma is characterized by recurrent episodes of reversible airflow obstruction, bronchial hyperresponsiveness, and airway inflammation [8]. Immunoglobulin E is largely responsible for the cascade of events in asthma. Patients with disorders of asthma, allergic rhinitis, and atopic dermatitis produce an overabundance of antigen-specific antibody; exposure to the specific allergen initiates inflammatory reactions. The release of inflammatory mediators is responsible for producing the

early-phase response of asthma (acute bronchoconstriction), which peaks about 30 min after exposure to the allergen [9, 10].

With continued exposure to inflammatory mediators, further inflammation results in the late-phase response characterized by decreased airway flow 4–8 h after allergen exposure, with significant airway hyperresponsiveness and bronchospasm [11, 12]. Asthma eventually leads to irreversible remodeling of the airway, making the management of asthma symptoms and disease control increasingly difficult.

### 7.2.2 Diagnosis of Asthma

The diagnosis of asthma is based on characteristic signs and symptoms in the presence of airflow obstruction or hyperresponsiveness [8, 33]. The signs and symptoms are wheezing, a history of chronic cough that is worse at night, and recurrent shortness of breath, and/or chest tightness. Exercise, viral infections, inhalation of allergens or irritants, weather changes, stress, and sometimes menstrual cycles can contribute to worsening symptoms.

The patient's ability to exhale air is assessed to identify the level of possible constriction in the bronchioles using a peak expiratory flow rate, which is measured by spirometry to establish a definitive diagnosis. Spirometry is a simple office-based test that can reveal airflow obstruction, as well as its severity and responsiveness to bronchodilator treatment. An increase in forced expiratory volume in 1 s (FEV1) of greater than 200 ml and 12% from baseline after treatment with a short-acting beta-agonist (SABA) are signs of improvement. Spirometry is performed at the initial patient assessment and then again after treatment initiation [8].

It is vital to classify asthma based on symptoms after evaluating impairment (through symptom assessment and spirometry) and exacerbation risk. Severity is measured in part by how difficult it is to control the disease with treatment [8]. Current evidence-based guidelines, which outline preferred and alternative therapies in a stepwise manner, enable clinicians to select appropriate initial therapy for patients on the basis of disease severity.

The Global Initiative for Asthma [7] identified and defined categories based on severity of symptoms as the following:

1. Intermittent: Nocturnal symptoms occur not more than twice a month with normal lung function between periods.
2. Mild persistent: Nocturnal symptoms occur more than twice a month but less than once a week with normal lung function between episodes. Symptoms occur less than once a week.
3. Moderate persistent: Nocturnal symptoms occur at least once a week with $60\% < \text{PEF1} < 80\%$ predicted or $60\% < \text{PEF} < 80\%$ of personal best. Symptoms occur more than once a week but less than once a day.
4. Severe persistent: Symptoms occur daily characterized by frequent exacerbations of nocturnal symptoms with $\text{FEV1} < 60\%$ predicted or $\text{PEF} < 60\%$ of personal best.

## 7.3 Evidence-Based Treatment and Management of Asthma

### 7.3.1 Asthma Education and Prevention Program

In 2007, the National Asthma Education and Prevention Program (NAEPP) updated its guidelines for the diagnosis and management of asthma [13]. The Expert Panel Report encompasses four essential components: assessment and monitoring, patient education, control on factors contributing to asthma severity, and pharmacotherapy.

According to the NAEPP guidelines, urgent or emergent management of asthma exacerbation involves providing the patient with supplemental oxygen, administering repetitive or continuous short-acting beta-agonist (SABA), administering oral corticosteroids with failure of short-acting beta-agonist treatment, and monitoring the response to therapy with serial peak flow assessments or spirometry.

It also recommended adjuvant treatment with magnesium sulfate or heliox for exacerbations unresponsive to short-acting beta-agonist and corticosteroids. It is essential to educate patients to prevent relapses and recurrences of exacerbations by encouraging them to contact their primary care provider within 3–5 days after discharge from the hospital and to schedule follow-up care within 1–4 weeks. Initiating inhaled corticosteroids therapy upon discharge also should be considered if the patient is not already on it. Exacerbation prevention plans and review of inhaler technique must also be included in the patient discharge education and planning [8].

All patients presenting in the emergency room with a reported asthma exacerbation must be evaluated and triaged immediately based on vital signs and overall physical assessment. The first step is to do an initial assessment including a brief history and physical examination (use of accessory muscles, heart rate, and respiratory rate).

Obtain peak expiratory flow (PEF) or forced expiratory volume 1 (FEV1), oxygen saturation, and other tests as indicated. Treatment should immediately follow the recognition of moderate, severe, or life-threatening exacerbation by assessment of signs and symptoms and lung function. Take a more detailed history (including a list of all medications – especially asthma medications and the time the last dose was taken), complete physical examination, and perform laboratory studies only after initial therapy has been completed [13].

### 7.3.2 NAEPP Recommended Treatment and Management [8]

1. Mild to moderate: (FEV1 or PEF ≥ 40%)
   - Give oxygen to achieve oxygen saturation (Sao2) ≥90%
   - Inhaled SABA by nebulizer or metered-dose inhaler (MDI) with a valved holding chamber, up to three doses in the first hour
   - Oral systemic corticosteroids, if no immediate response or if patient recently took systemic corticosteroids

2. Severe (FEV1 or PEF ≤ 40%)
   - Oxygen to achieve $SaO_2 \geq 90\%$
3. Mild to moderate exacerbation
   - Inhaled SABA every 60 min
   - Oral systemic corticosteroids
   - Continue treatment 1–3 h; provided there is improvement, make admission decision in <4 h
4. Severe exacerbation (FEV1 or PEF ≤ 40% predicted/personal best)
   - High-dose inhaled SABA plus ipratropium by nebulizer or MD1 plus valved holding chamber every 20 min or continuously for 1 h
   - Oral systemic corticosteroids
5. Impending or actual respiratory arrest
   - Intubation and mechanical ventilation with 100% oxygen
   - Nebulized SABA and ipratropium
   - Intravenous corticosteroids
   - Consider adjunct therapies

Oxygen is recommended for most patients with asthma. The repetitive or continuous administration of short-acting beta-agonist is the most effective means of reversing airflow obstruction. In the ER, it can be given safely for three consecutive times spaced every 20–30 min as initial therapy. Adding multiple high doses of ipratropium bromide produces additional bronchodilation resulting in fewer hospital admissions.

In mild or moderate exacerbations, equivalent bronchodilation can be achieved either by high doses (4–12 puffs) of a SABA by MDI with a valved holding chamber or by nebulizer. The onset of action is less than 5 min; repetitive administration produces incremental bronchodilation. Response to the initial three doses in the ED will be sufficient to discharge them, and most patients will have a significant response after the first dose.

In general, the treatment involves oxygen, aerosolized short-acting beta-agonist, and systemic corticosteroids, and perhaps adjunct therapies. Management of asthma should also include frequent clinical assessment of respiratory status for distress and fatigue as well as objective measurement of airflow. Other adjunct therapies include intravenous beta2-agonists, intravenous leukotriene receptor antagonists, and noninvasive ventilation. Immediate intubation of patients presenting with apnea or coma is highly recommended by the expert [8].

### 7.3.3 Response to Treatment and Action Plan

It is equally important to monitor response after treatment and management in order to establish if treatment is effective or not. The following outlines the response to treatment with corresponding action plans [13]:

1. Good response (FEV 1 or PEF > 70%)
   - Response sustained 6 min after last treatment
   - No respiratory distress
   - Normal physical examination

Action/plan:

- Discharge patient home and continue treatment with inhaled SABA
- Continue course of oral systemic corticosteroid, consider initiation of inhaled corticosteroids
- For those not on long-term control therapy, consider inhalation of corticosteroids
- Provide patient education (review medications, including inhaler technique and environmental control measures)
- Review/initiate action plan
- Close medical follow-up
- Before discharge, schedule follow-up appointment with primary care provider and/or asthma specialist in 1–4 weeks

2. Incomplete response (FEV 1 or PEF 40–60%)
   - Patient still manifests mild to moderate symptoms

Action/plan:

- Admit to hospital unit
- Oxygen therapy
- Continue inhaled SABA
- Systemic oral or intravenous corticosteroid
- Consider and adjunct therapies, monitor vital signs, FEV1 or PEF, and $SaO_2$.

3. Poor response (FEV1 or PEF < 40%)
   - $PCO_2$ > 42 mmHg
   - Severe, drowsiness, confusion

Action/plan:

- Admit to hospital intensive care
- Oxygen therapy
- Administer inhaled SABA and/or intravenous corticosteroid
- Consider adjunct therapies, possible intubation/mechanical ventilation

### 7.3.4 Discharging a Patient

Regular care in an outpatient setting should be emphasized when discharging a patient to home. Patient should be referred for follow-up of asthma care appointment either to a primary care provider or to an asthma specialist within 1–4 weeks. If appropriate, refer to an asthma self-management education program. It is also necessary that discharge medications are reviewed including the dose, frequency, indications, and side effects of these medications with the patient. The patient should be given instructions regarding the use of inhaler, how to measure and record PEF, and when and who to contact for any worsening symptoms of asthma. Preventive measures against asthma should also be reviewed before discharging the patient to home.

## 7.4 Prevention and Control of Asthma

### 7.4.1 How to Prevent and Control

Asthma exacerbations may be caused by a variety of factors, sometimes referred to as triggers, including allergens, viral infections, pollutants, and drugs. Evidence suggests that reducing a patient's exposure to some of these categories of risk factors such as smoking cessation, reducing exposure to occupational agents known to cause symptoms, and avoiding foods/additives/drugs known to cause symptoms improves the control of asthma and reduces medication needs.

Asthma control is the degree to which signs and symptoms, risk of future episodes, and loss of lung function are reduced through treatment and environmental control. Because both patients and health-care providers tend to under recognize and underreport asthma symptoms [14, 15], control should be assessed at each visit using a validated tool such as the Asthma Therapy Assessment Questionnaire, the Asthma Control Questionnaire, or the Asthma Control Test [8]. These questionnaires are easily accessed online and can be administered quickly.

Periodic lung function measurement with spirometry and patient monitoring of peak flow and symptoms can further help clinicians to determine control and adjust medication accordingly. Cochrane review of asthma therapy has made a number of important contributions to the evidence base for decision-making in asthma. Evidence for decision making for asthma management includes maintenance therapy with regular inhaled steroids, introduction of long-acting beta-agonists, leukotriene receptor antagonists, and new therapies such as anti-immunoglobulin E.

Proper outpatient treatment may reduce the incidence or exacerbations of asthma requiring hospitalizations. The use of clinical pathways for inpatient asthma patients decreased the length of stay. Nebulized short-acting beta-agonist therapy decreased acute asthma encounters through 2 weeks after patient is discharged according to a study [37].

In addition, the use of inhaled corticosteroids in the treatment of asthma showed a decrease in the need for oral corticosteroids, as well as a decrease in airway reactivity and reduction in the frequency of acute exacerbations and the need for concurrent medications [17, 18]. Other complementary therapy may also be helpful like stress management and relaxation therapy in the asthma management.

Cochrane reviews highlighted the importance of education and self-management of these asthma patients. Individualized written action plans based on personal peak expiratory flow, using 2–4 action points, and recommending both inhaled and oral corticosteroids for the treatment of exacerbations consistently improve asthma health outcomes. Asthma education plays a role in improving patient perceptions of their symptoms. Self-management education reduced hospitalizations, emergency room visits, unscheduled visits to the doctor, days off work or school, nocturnal asthma, and quality of life [40].

### 7.4.2 Managing Asthma: Evidence-Based Nursing

Improved methods of healthcare delivery for patients with asthma are needed to prevent readmissions [34]. A study done on 96 adult subjects (predominantly young African American women) hospitalized with an asthma exacerbation, who had a history of frequent healthcare use, were randomized to an asthma nurse specialist intervention (intervention group $n=50$; control group $n=46$) for 6 months [36].

This Nurse-Directed Inpatient Asthma Intervention (a hospital-based program for adults) used asthma nurse specialists to help high-risk patients reduce hospital readmission rates within 6 months of hospital discharge, reduce cost, and improve health-related quality of life. As a part of the intervention, individual treatment plans were revised to be consistent with the National Asthma Education Prevention Program Guidelines.

The treatment plan included a daily asthma care flow sheet to enhance communication between the nurse specialist and the physician. Nurses educated patient participants about asthma and help them develop an individualized asthma self-management plan. Asthma education included medication dosing, inhaler technique, and peak flow monitoring. Outpatient care is facilitated through telephone follow-ups.

The results showed a 60% reduction in total hospitalizations following the program in the intervention group when compared to a regular-care control group (31 vs. 71 readmissions, $p=0.04$). Readmissions due to asthma decreased by 54% (21 vs. 42 readmissions, $p=0.04$). Intervention participants had markedly fewer lost work or school days (246 vs. 1,040 days) and substantially reduced direct and indirect healthcare costs ($5,726 vs. $12,188) with a saving of $6,462 per patient ($p=0.03$).

This study provided evidence that a brief intervention program focusing on high health-care users with asthma can result in improved asthma control and reduced hospital use with substantial cost savings. In addition, it is worth mentioning, that this study well established the role of advanced practice nurses in the management of asthma [36].

A study done in 2009 by Borgmeyer et al. to measure the effectiveness of a pediatric nurse practitioner showed the effectiveness of pediatric nurse practitioner in inpatient asthma care. Pediatric nurse practitioner lowered the costs of care. The nurse practitioner model of care where asthma clinical pathway was utilized brought consistency to the care of asthma patients [38].

The results also indicated that community physicians and university-based physicians perceived the role of inpatient nurse practitioners as effective in the areas of direct patient care, communication, consultation, and education. Staff nurses agreed that nurse practitioners managed patients appropriately and facilitated nursing care. The families of hospitalized children, in this study, also agreed that their child was given individualized attention and was well cared for by the nurse practitioners [39].

In another study, 115 asthmatic patients participated to assess the effect of a nurse-run asthma clinic on practice workload and the morbidity of patients. Comparisons were made between the 12 months prior to the introduction of the clinic and the first 12 months after the clinic started.

Morbidity was measured in terms of the number of courses of oral steroids, the number of emergency salbuterol nebulizations, and the number of days lost from work or school. The number of consultations with the general practitioners was recorded.

The repeat prescribing register was also monitored throughout the study to examine the effect of the clinic on prescribing in the practice. Consultations with general practitioners fell from a total of 818 to 414 during the study period ($p<0.001$) [41].

This was offset by 496 consultations with the nurse in the first 12 months of the clinic. As a result of attending the nurse clinic, there were significant reductions ($p<0.01$) found in the patients' requirements for courses of oral steroids, acute nebulizations, and days lost from work or school. The results for the 46 children were similar to those for the 69 adults, confirming that the asthma clinic was effective for all ages.

The clinic coincided with an increase in the number of patients receiving regular bronchodilator therapy and prophylactic medication. Eighty percent of patients had their medication modified as a result of attending the clinic. The cost of prescribing remained remarkably stable [43].

Evidence-based strategies used by nurse practitioners in primary care or within a hospital setting improve patient health outcomes and contribute to decreasing cost of health-care dollars. Examples of evidence-based strategies include but not limited to nursing interventions for tobacco cessation in the acute care settings and asthma home management training program [43]. These strategies significantly reduce asthma exacerbations [31, 32].

## 7.5 Overview of Pressure Ulcer (PU)

### 7.5.1 What Is a Pressure Ulcer?

Pressure ulcer or pressure sore is a localized injury to the skin and/or underlying tissue usually over a bony prominence as a result of pressure or pressure in combination with shear [6]. A pressure is a force applied perpendicular to the skin, while a shear is a layer of the skin sliding horizontally disrupting the blood flow and deforming the muscle and adipose tissue (Fig. 7.1).

A pressure ulcer is a serious complication of multimorbidity and immobility. To better understand what a pressure ulcer is, we will review the different stages of pressure ulcer. The following are the different stages [6]:

- Stage 1: intact skin with blanchable erythema
- Stage 2: open skin with a partial loss of thickness from epidermis to dermis with a pink wound bed
- Stage 3: full-thickness loss of skin with extension through the underlying tissue but not to the fascia
- Stage 4: full-thickness loss of skin extending to the bone, muscle, tendon or joint capsule, and fascia
- Unstageable: full-thickness loss of skin with the base of the ulcer of slough or eschar such that the full depth of the wound cannot be appreciated. The depth of the pressure ulcer can only be evaluated and correctly staged when the slough or eschar is removed. Deeper tissues can manifest a wider area of injury or necrosis that is apparent from the surface, so the "true stage" of a pressure sore may not be evident until the wound is debrided and all necrotic material is removed [23].

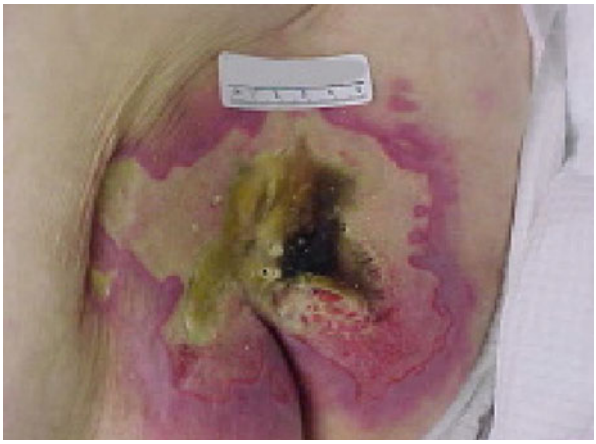

**Fig. 7.1** Pressure ulcers

Although pressure ulcer is not always preventable or curable, we should always attempt to prevent and treat using the best available evidence in its treatment and management. Physical, cognitive, and physiological factors such as impaired perfusion increase the risk of pressure ulcer [24].

### 7.5.2 Evidence-Based Prevention and Treatment Strategies

Prevention and treatment of pressure ulcer require multifaceted approach and strategies. These strategies include physiological and physical care, optimal nutrition, mobility, psychological and emotional support, and knowledgeable group of practitioners.

#### 7.5.2.1 Nutrition

From the Cochrane reviews, nutrition is a major contributing factor to wound healing as evidenced by multiple randomized controlled trials from the Cochrane reviews [46]. Studies were done to compare enteral vs. parenteral nutrition, dietary supplement in addition to regular diet vs. regular diet alone, and different types of dietary supplements. Subgroup analyses were done on the characteristics of the setting (e.g., hospital inpatients vs. outpatients), method of feeding (e.g., enteral vs. parenteral feeding), and characteristics of patients (e.g., people with existing malnutrition vs. people without malnutrition).

Among the prevention studies, Delmi in 1990 examined 59 elderly patients in two hospitals who sustained femoral neck fractures after accidental falls. The two groups either received a standard hospital diet alone or one nutritional supplement daily in addition to their hospital diet. The results noted that the number of pressure ulcers (PU) in the control group was 9% ($n = 3/32$) compared to 7% of the treatment

group ($n=2/27$) with a statistically nonsignificant relative risk (RR) for pressure ulcers of 0.79 (95% confidence interval (CI) of 0.14–4.39, $p=0.8$).

In another hospital wherein the same study was done, the number of PU was 0 in the treatment group ($n=9$) and three in the control group ($n=15$, 20%) with a not statistically significant RR of 0.23 (CI of 0.01–3.98, $p=0.3$). Wound healing had an RR = 0.79 in the control group which is basically the same wound healing number of PU at 6 months.

There was no PU in the treatment group ($n=25$) at 6 months compared to a 7.4%, and two PU in the control group with a not statistically significant RR of 0.22 (CI of 95% 0.01–4.28, $p=0.3$).

Hartgrink in 1998 followed 140 patients recovering from fractured hip for two weeks. Treatment group received standard hospital diet with nasogastric tube feeding compared to the control group receiving standard hospital diet. The study showed that 52% (25/48) in the treatment group vs. 56% (30/53) in the control group had grade 2 or more PU. There is no significant difference in a per protocol analysis (RR = 0.92, CI of 95% 0.64–1.32, $p=0.6$).

After 2 weeks, the treatment group who received one nutritional supplement with the hospital diet had 55% (27.51) of a stage I–II PU whereas the control group who received hospital diet with noncaloric water-based placebo had 59% (39/52), respectively with an RR of 0.92 (95% CI of 0.65–1.3). The incidence of stage II PU was 18% in the treatment group compared to 28% in the control group, which is not statistically significant odds ratio (OR) of 0.6 (95% CI of 0.3–1.6).

In summary, of the three groups who received mixed nutritional supplements, all reported a lower PU incidence in the supplement group; however, the sample is far too small to detect clinical significance.

Several studies were also done on the treatment pressure ulcers. Taylor in 1974 used ascorbic acid 500 mg twice daily on 20 surgical patients with pressure ulcers for a month. The results showed a statistically significant reduction of the size of pressure ulcers by 84% in the intervention group as compared to 42% reduction in the control group.

Complete healing of the pressure ulcers occurred in six patients in the intervention group with a mean healing rate of 2.47 $cm^2$/week vs. three patients with healing rate of 1.45 $cm^2$/week in the control group. Another study was done in 1995 by Riet in multicenter nursing homes using 500 mg ascorbic acid twice daily (healing rate of 0.21 $cm^2$/week) in the treatment group ($n=43$) compared with the control group ($n=45$) of a healing rate of 0.27 $cm^2$/week.

Chernoff in 1990 used high-protein diet for pressure ulcer size ranging from 1.6 to 63.8 $cm^2$ and very high-protein diet for pressure ulcer size ranging from 1.0 to 46.4 $cm^2$. On both diets, the pressure ulcers decreased in size but more so in the very high-protein diet. Pressure ulcer size decreased by 42% with high-protein diet, while 72% decrease was noted with patients who received very high-protein diet.

As optimal nutrition is undoubtedly extremely important in the prevention and treatment of pressure ulcer, nutritional status should always be evaluated to ensure adequate intake of calories, proteins, and vitamins. Protein intake of one to two

grams/kg/day is recommended with supplemental vitamin C and zinc. Malnutrition is one of the few reversible contributing factors to pressure ulcers. Establishing adequate caloric intake has been shown to improve healing of pressure ulcers.

#### 7.5.2.2 Body Surface Mattress

Although pressure ulcers are sometimes unavoidable as announced at the conference in John Hopkins last February 2010 by National Pressure Ulcer Advisory Panel (NPUAP) [26], it is still an expectation that every prevention strategy should be implemented. One of the strategies is the use of body surface mattress. There are different body surface mattresses available to prevent or heal pressure ulcers.

Foam alternatives to standard hospital foam mattresses reduce the incidence of pressure ulcers in people at risk. Pressure-relieving overlays on the operating table reduce postoperative pressure ulcer incidence, although two studies indicated that foam overlays caused adverse skin changes. Meta-analysis of three trials indicated that Australian standard medical sheepskins prevent pressure ulcers. The relative merits of higher-specification constant, low-pressure, and alternating-pressure support surfaces for preventing pressure ulcers are unclear, but alternating-pressure mattresses may be more cost-effective than alternating-pressure overlays in a UK context. Medical grade sheepskins are associated with a decrease in pressure ulcer development [45].

#### 7.5.2.3 Psychological/Emotional Support

Pressure ulcer can create an emotional and psychological distress from a distorted body image. A qualitative study was done addressing the health-related quality of life on 30 participants aged 22–94 years old with pressure ulcers from an acute and primary setting in England and Northern Ireland from December 2007 to October 2008. There were four domains on the health-related quality of life (HRQL) such as pressure ulcer (PU) specific symptoms, physical functioning, psychological well-being, and social functioning. PU-specific symptoms include pain and discomfort, exudates and odor; physical functioning included mobility, daily activities, general malaise, and sleep; psychological well-being included mood, anxiety, and worry, self-efficacy and dependence, appearance, and self-consciousness; social functioning included social isolation and participation.

The results revealed that PU has a negative effect on HRQL as it restricts mobility and activities, contributes toward pain and emotional problems leading to social isolation [27]. Pain is less reported by the elderly population, but it needs to be considered prior to dressing changes of PU.

#### 7.5.2.4 Staff Education

Although evidence-based guidelines for pressure ulcer prevention are available and recommended, non-adherence is frequently reported. Lack of knowledge about PU prevention and negative attitudes of nurses are also frequently reported [22]. As clinicians (nurses, physicians, and physical therapists), we should always strive to

continue to seek out the best available evidence in the prevention and management of PU. One example of evidence is the clinical care daily project, the translating research into practice (TRIP) was used to support implementation of a care management solution aimed at preventing pressure ulcers.

Initial success was evidenced by 34% reduction in PU rates and an 86% reduction in missed patient turns 3 months post-implementation of the daily intervention. The TRIP is an extension of the Roger's model, which has proven evidence-based nursing practice intervention for PU. The Roger's diffusion innovation model provides a framework for identifying expected patterns of human behavior when an innovation or change is introduced [28].

#### 7.5.2.5 Physical and Physiological Care

In order to prevent pressure ulcers, skin should be kept moist and well hydrated. Pressure ulcers and its surrounding areas should be dry and clean, free from urine and feces. Passive mobility of patient should be done routinely for those who are unable to move or who are very weak to move on their own. For those who have enough strength to move, they should be encouraged to do so.

Although immobility is a contributing factor for developing pressure ulcer, there is no clear evidence that repositioning every 2 h or every 4 h prevents pressure ulcers. However, pressure is reduced to some degree as the patients are moved by turning and positioning. Adequate pain control to ensure pain-free mobility and pain-free wound debridement. Any physiologic deficiency like anemia and dehydration should also be corrected.

#### 7.5.2.6 Wound Debridement

Wound debridement is done for devitalized tissue to prevent bacterial decontamination. Debridement is accomplished either by surgical removal of devitalized tissues or by a chemical enzymatic agent. Chemical enzymatic agent may be applied for stage 1–111 pressure ulcers.

Amputation of the extremity may be done for reconstructive surgery of a nonhealing wound. Approximately 7–8% of patients with paraplegia die with pressure ulcer [4].

## 7.6 Clinical and Financial Implications

### 7.6.1 Pressure Ulcer Assessment

Pressure ulcer assessment on admission is the key to early management [30, 47]. It is very important to design and implement interventions that are consistent with individual needs, goals, and recognized standards of practice. Furthermore, monitor/evaluate the impact of the interventions and revise the approaches as appropriate [26]. Documentation and communication of pressure ulcers between units must be facilitated [42]. Inconsistencies in documentation can penalize the institution for hospital-acquired pressure ulcers.

The Agency for Healthcare Research and Quality (2007) reported a nearly 80% increase in hospital stays with PUs from 1992 to 2006 resulting in an annual costs of $11 billion [18]. In 2008, the Centers for Medicare & Medicaid Services (CMS) implemented a new policy under the inpatient prospective payment system that the costs of hospital-acquired conditions or complications are no longer reimbursed.

The types of complications included in the first stage of the policy include several different hospital-acquired infections (HAIs), stage two and three pressure ulcers, falls, mediastinitis, air embolism, blood incompatibilities, and foreign objects retained after surgery. The changing health-care environment moves employers to use their market power to promote quality and value of health-care services through a principle called value-based purchasing.

Value-based purchasing is a general philosophy toward quality improvement (QI) and cost-efficiency in health care that focuses payment on quality and shifts resources from lower- to higher-performing organizations in the process [10].

Value-based purchasing is another way in which CMS rewards hospitals who are meeting the national benchmark or better on established national measures on quality of care, safety, and patient satisfaction; on the other hand, it is a way to penalize those hospitals who are not meeting the national benchmark on established measures of performance as previously mentioned. Payments or penalty depending on performance will start to be rewarded in October 2012.

## 7.7 Conclusion

Early detection and identification of a pressure ulcer will prevent complications including but not limited to the progression of pressure ulcer to the next stage. Identification of the risk factors of the patients such as immobility, elderly, cognitively impaired, iatrogenic or nosocomial complication, and vascular compromise (such as a code or septic shock) is essential to proper management of pressure ulcers.

Every care provider must have adequate knowledge and skill in the prevention, early detection, and treatment of pressure ulcers. The utilization of evidence-based practice leads to better clinical decisions and patient outcomes. Effective and efficient treatment and management of any illness including asthma and pressure ulcers is not a choice rather a must do in order to continue to transform our current health-care system [35] in the provision of cost-effective, yet high-quality care. The survival of health-care organizations depends on accountable clinicians mindful of the current health-care focus on cost-effective, safe, and high quality care.

Asthma is one of chronic diseases that can be controlled when managed effectively. The active participation of the patient with asthma in her/his care provides a positive base for asthma prevention and symptom control.

The use of evidence-based strategies on asthma such as timely assessment and medical management, patient education on prevention, symptom control, medication adherence, and appropriate follow-up visits with health-care providers are essential to achieve positive patient health outcomes and quality of care, improve patient's quality of life, and decrease health-care costs.

There are multiple studies and evidence-based strategies in the literature that can provide clinicians guidelines in the care and management of asthma. Every clinician is expected to practice based on evidence as health-care ventures to an expectation of low cost, yet high-quality care.

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# Evidence-Based Care for Patients with Acute Coronary Syndrome in New York City

8

Mary Brennan and Janet Johnson

**Core Message**

With a state-of-the-art cardiac catheterization center, cardiologists, partnering with nurse practitioners, collaborate to utilize the best available evidence to assess, diagnose, and treat patients with acute coronary syndrome. Evidence-based emergent assessment and treatment as well as primary and secondary prevention of acute coronary syndrome are necessary to improve safety and quality of patient care and outcomes, and cardiovascular outcomes for individuals negatively impacted by racial, social and economic disparities.

## 8.1 Introduction: Evidence-Based Emergency Cardiac Care

The upper east side of New York City is a geographic area rich with ethnic diversity, but economically impoverished, with almost 40% of residents living below the poverty line, a rate almost three times greater than the national average [1]. The health disadvantages associated with poverty, including high rates of uninsured inviduals, and a significant number of residents without primary care providers, contribute to an increase in the prevalence of comorbidities such as obesity, hypertension, and diabetes, culminating in a community with the second highest incidence of premature cardiovascular disease [1, 2]. While city-wide efforts to reduce cardiovascular risk factors have resulted in a decline of deaths, this geographic area continues to experience disproportionate rates of cardiac disease, increased years of potential life lost (YPLL), and early mortality [1].

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To address the socioeconomic, racial, and ethnic inequities in cardiovascular care, the cardiology service of a large academic medical center located in this area has developed an innovative model of emergency cardiac care with cardiologists, interventionists, and nurse practitioners, who together, by working in collaborative practice, provide evidence-based emergency treatment of patients with acute coronary syndrome. The medical center's cardiology service has the highest volume of cardiac catheterizations in New York State. Skillful interventionists perform over 18,000 invasive coronary and vascular procedures per year, of which 5,700 procedures involve percutaneous coronary interventions for patients with acute coronary syndrome. Nurse practitioners, performing within an advanced role, participate in the early assessment, acute monitoring, management, and discharge of over 6,000 cardiac patients per year. Together, the team has contributed to an improved safety record, a record that has surpassed other centers in the area since reporting began in 1994. Personal Communication Janet johnson, January 21, 2011. The team members incorporate evidence from the high-quality guidelines, systematic reviews, meta-analyses, randomized controlled trials, and cohort studies to inform and guide their clinical decision making. Treatment recommendations from the American College of Cardiology and American Heart Association Guidelines are ranked according to the risk-benefit profile and range from a Class I Recommendation, when the benefit outweighs the risk, to a Class III when the risk surpasses the benefit. Additionally, recommendations are classified according to the strength, quality, and precision of evidence and begin with Level of Evidence (LOE) A, high-quality evidence, to Level of Evidence (LOE) C, indicating limited evidence [3, 5–6]. While intensified efforts aimed at primary prevention are needed to treat and prevent the development of risk factors, accessible emergency care is urgently required to address the existing cardiovascular death rate [15]. The provision of expert, evidence-based emergency care in a tertiary care center, led by highly skilled cardiologists, cardiac catheterization interventionists, and advanced nurse practitioners, informed by the latest evidence, constitutes an innovative approach to address the health disparities and disadvantages associated with poverty and the pervasive, premature cardiovascular death rates in this community.

## 8.2 Evidence-Based Treatment Goals

### 8.2.1 Evidence-Based Goal #1: Reperfusion of the Occluded Coronary Artery

Achieving reperfusion of the occluded coronary artery is the primary goal for patients who present to the emergency room with chest pain and acute coronary syndrome (ACS). Acute coronary syndrome represents a spectrum of cardiac conditions, characterized as an obstruction to coronary blood flow, with associated and varying degrees of impaired blood supply and subsequent damage to the myocardium. Unstable angina (UA) represents a temporary decrease in blood supply to the

myocardium, most frequently, occurring from a clot in one or more coronary arteries that intermittently obstruct blood flow, without causing myocardial necrosis. Non-ST-elevation myocardial infarction (non-STEMI), thought to be related to a platelet-rich clot that arises from a ruptured plaque, obstructs blood flow sufficiently enough to inflict subendocardial myocardial damage, as evidenced by elevated troponins, and ST and T waves changes on the EKG. ST-elevation myocardial infarction (STEMI) develops from disruption of a plaque with a fully developed clot that completely impedes blood flow resulting in significant myocardial necrosis to the myocardium. An understanding of the underlying pathophysiology informs the initial assessment and treatment approaches and assists providers in comprehending the complex array of targeted evidence-based therapies necessary to minimize the period of ischemia and preserve the myocardium.

### 8.2.2 Goal #2: Risk Stratification of All Patients Presenting with Chest Pain

Owing to the diverse and high-risk population of patients managed by the cardiology service, risk stratification helps to guide treatment decisions. The most importantly decision is whether to pursue an early invasive strategy with a percutaneous coronary intervention (PCI), or an initial conservative approach, relying solely on medical pharmacotherapeutic modalities, including fibrinolysis, antithrombotics, and antiplatelets. Patients are initially assessed and classified according to the likelihood of ACS ranging from a high likelihood of acute coronary syndrome to an intermediate or low likelihood based on presenting features reflective of acute coronary syndrome [3]. Clinical features associated with the highest risk category include a history of protracted, characteristic chest pain, ST-segment changes of.5 mm or more, or a new bundle branch block on the EKG, and elevated biomarkers on serial testing [3]. Intermediate risk patients do not display high-risk features, but may have a history of a coronary artery disease, with extended ischemic pain that may have resolved upon admission, and may or may not have T-wave changes on EKG accompanied by slightly elevated biomarkers. Individuals who describe an episode of anginal pain that occurred greater than 2 weeks prior to admission, in the absence of EKG changes and normal biomarkers, constitute the lowest-risk group. In general, it is important to remember that the individual's greatest risk of death and coronary vascular events is at the time of presentation with the risk diminishing over the ensuing hours [3].

The NP who is performing the history and physical uses the admission data to assess the patient's short term risk of cardiovascular as well as the long-term risk of morbidity and mortality. A number of risk assessment tools have been developed to ascertain the risk of death and ischemic events in patients presenting with UA/NSTEMI, providing a foundation for therapeutic decision making. The Thrombolysis in Myocardial Infarction (TIMI) risk score is an instrument that quantifies risk according to seven different variables:

(a) Age 65 years or older
(b) At least three risk factors for CAD; prior coronary stenosis of 50% or more

(c) ST-segment deviation on EKG presentation
(d) At least two anginal events in the prior 24 h
(e) Use of Aspirin in the prior 7 days
(f) Elevated serum cardiac markers [4]

Patients with a score of 2 of 7 have a predicted risk of 8.3% of all-cause mortality, recurrent myocardial infarction, or recurrent angina within a 2-week period following the acute myocardial infarction. Individuals with a higher risk, as indicated by a score of 6–7 of 7, experience a 40% risk of recurrent angina and myocardial infarction[4, 14].

The initial assessment, diagnosis, and treatment of patients with suspected acute coronary syndrome are based upon the guidelines published by the American College of Cardiology/American Heart Association for patients with unstable angina/non-ST-elevation myocardial infarction and the American College of Cardiology/American Heart Association Guidelines for patients with ST-elevation myocardial infarction [3, 5–7]. Determination of a working diagnosis is made within the initial 10-min assessment period. Individuals with high-risk clinical features, including characteristic chest pain and accompanying ST elevations of 1 mm or more in two or more continuous EKG leads, or with a new left bundle branch block on the EKG, are immediately diagnosed with an ST-elevation myocardial infarction (STEMI), and, in the absence of contraindications, are emergently transferred to the cardiac catheterization lab for an urgent diagnostic cardiac catheterization and possible stent placement of the affected occluded coronary artery, known as a percutaneous coronary intervention. Multiple studies have demonstrated the superiority of percutaneous coronary interventions compared with fibrinolysis and reveal a short-term mortality benefit, decreased incidence of reinfarction, and reduced rates of major hemorrhage [5]. The ACC/AHA Guidelines have classified percutaneous coronary intervention as the preferred strategy, a Class I, Level of Evidence A Recommendation, reflecting the high-quality evidence supporting this intervention. Adherence to a specified protocol by the team ensures that door-to-balloon times do not exceed 90 min, conferring a survival benefit if administered within the prescribed time period [5, 6].

Individuals who are not candidates for a percutaneous coronary intervention are evaluated for possible fibrinolysis within 30 min of admission to the hospital [5, 6]. Again, the importance of adhering to specific criteria to ensure that fibrinolysis is delivered within the prescribed time window of 30 min is essential in minimizing ischemia of the myocardium and attaining a survival benefit. Nurses and cardiologists are skilled in the administration of fibrinolytics, and are attuned to the potential side effects of bleeding.

In high-risk patients with typical chest discomfort and associated ST- or T-wave depression with unstable angina or non-STEMI, percutaneous coronary interventions have been shown to reduce the incidence of intermediate death, refractory angina, and rehospitalization, but increase the risk of procedure-related heart attacks and bleeding complications [3, 8]. As a consequence of the increased risks associated with the performance of percutaneous coronary intervention in patients in UA or non-STEMI, the decision to perform early percutaneous coronary intervention

remains a subject of some debate. The updated ACC/AHA Guidelines suggest that early PCI, performed within 12–24 h, may be superior in those individuals deemed to be at high risk of death or intermediate risk of death [3, 8].

- Patients who demonstrate intermediate risk factors, including ongoing chest discomfort associated with ST depression or T-wave inversion, may benefit from an initial invasive coronary intervention.
- In patients, by contrasts, who are characterized as having a low to intermediate risk, an invasive approach may be delayed at the discretion of the interventional cardiologist. Echocardiographic evaluation is performed in all patients; if significant left ventricular dysfunction is detected, then subsequent stress testing and a possible delayed diagnostic coronary angiography and potential stent placement are considered [3, 8].
- Patients selected for a delayed intervention are continuously monitored during their hospitalization for signs of hemodynamic instability and the reoccurrence of angina pain. If symptoms develop, percutaneous coronary intervention will be performed.

### 8.2.3 Evidence-Based Goal #3: Provide Adjunctive Therapies for Inhibition of Platelet Aggregation and Thrombus Formation

Reperfusion of the occluded, culprit artery is the main goal of treatment for all patients with acute coronary syndrome, and research continues to confirm the central role that platelet inhibition plays in restoring myocardial blood flow and facilitating implantation of stents. Whether an initial invasive strategy or a conservative approach is adopted, adjunctive medical therapy, including the coadministration of antiplatelet agent and antithrombotics, is required to assist in the reperfusion of the affected artery.

- Antiplatelet therapy: Combination antiplatelet therapy is directed at inhibiting further platelet aggregation. Antiplatelet therapy is started immediately with the administration of aspirin 162 mg. Patients are instructed to chew the tablets since buccal absorption is more rapid than gastrointestinal absorption [3, 5–7]. The addition of a thienopyridine, clopidogrel, to aspirin, an antagonist of adenosine diphosphate (ADP) and a potent antiplatelet, provides additional inhibition of platelet accumulation and is classified as a Class I, Level of Evidence C Recommendation [5, 7]. Alternatively, the revised 2009 ACC/AHA Guidelines recommend the option of adding a new thienopyridine, prasugrel, to aspirin therapy for all patients undergoing percutaneous coronary intervention, a Class I, Level of Evidence B Recommendation [7]. The use of prasugrel, while associated with a statistically significant reduction in stent thrombosis, is complicated by a significant risk of both minor and major bleeding events, including hemorrhage, and has translated to a 32% increase in the risk of bleeding [7]. Patients considered for treatment with prasugrel must be carefully vetted for any potential risk of bleeding or existing bleeding disorders.
- GPIIb/IIIa inhibitors prevent the cross-linking of fibrinogen to platelets, and provide an additional mechanistic therapy for suppression of platelet aggregation. Studies have consistently demonstrated that adding adjunctive GP IIb/IIIa

inhibitors to other antiplatelets for coronary interventions in unstable angina and non-STEMI is associated with a decrease in the incidence of myocardial infarction [3]. The use of GPIIb/IIIa inhibitors in the treatment of STEMI patients who undergo coronary interventions is less certain, and continues to be studied as an adjunctive therapy to other antiplatelet agents.

- Antithrombotics, including the use of unfractionated heparins (UFH), low-molecular-weight heparins (LMWH), play an important role in inhibiting enlargement of the existing clot and are required adjunctive therapy with coronary interventions and fibrinolytic therapy. More recently, bivalirudin, a direct-acting thrombin inhibitor, with a Class I, Level of Evidence B Recommendation, has been added to the potential list of antithrombotics based on a large randomized controlled trial that reported a decrease in death rates at 30 days and in 1 year due to the decrease in associated bleeding complications [7]. While a significant increase in stent thrombosis was detected, the guidelines recommend that bivalirudin may be an acceptable alternative to UFH or LMWH [7].

### 8.2.4 Evidence-Based Goal # 4: Laboratory Diagnosis of Myocardial Infarction with Sensitive Biomarkers

Multiple guidelines have identified cardiac troponin I (cTnI) and troponin T (cTNT) as the most sensitive biomarkers for the laboratory detection of myocardial damage. Troponins are cardiac proteins that are released into the circulation with necrosis of cardiac tissue. Levels above.4 indicate significant myocardial necrosis with higher levels of troponin correlated with a greater increase in the risk of cardiovascular death. Even minor elevations of troponin from 0.4 to 0.9 confer a 1.7 relative risk in the incidence of cardiovascular death in the following year [3, 5, 6, 14]. Although sensitive for myocardial damage, elevations of troponins may not appear for a period of up to 8–12 h. Given the sensitivity and predictive likelihood ratios associated with troponins, as well as the delay in circulating blood levels, cTnI is assessed upon admission and every 8 h for the first 24 h in order to the accurately determine the degree of myocardial necrosis.

### 8.2.5 Evidence-Based Goal #5: Assessment of the Patient prior to Percutaneous Coronary Angiography

Consistent with the ACC/AHA Guidelines, the nurse-practitioner obtains a targeted history and physical exam to evaluate patients for the presence of significant comorbidities including hypertension, hyperlipidemia, diabetes, chronic kidney disease, as well as a past history of myocardial infarction, stroke, or coronary artery bypass graft [5]. Additionally, the NP assesses the patient for associated symptoms such as amaurosis fugax, weakness of the extremities, and paresthesias to exclude the possibility of a concomitant stroke. A comprehensive medication history is obtained, including a record of all prescription and non-prescriptive medications, reported

compliance with the medical regimen, and a review of medication and food allergies. All patients who report a medication allergy to contrast dye, or to shellfish, are premedicated prior to a PCI with Benadryl and Solu-Medrol according to a protocol developed by the service.

The addition of an antiplatelet, such as Plavix, or prasugrel, with ASA for 1 year after drug-eluting stent implantation prolongs the patency of the stent and minimizes the incidence of cardiovascular events [8]. Prior to the intervention, and to avoid the risk of in-stent restenosis and/or complications resulting from noncompliance, bleeding, and/or death from inappropriate dosing, patients are evaluated for their ability to tolerate dual antiplatelet therapy. Patients with recent major bleeding diatheses, or recent gastrointestinal bleeding, may be excluded from treatment due to the potential increase risk of hemorrhage. The decision to implant a DES is also affected by the patient's ability to understand the risks and benefits related to combination antiplatelet therapy and to comply with this therapy. If patients are unable to tolerate the combined effects of two antiplatelet agents, the interventionist may opt to implant a bare metal stent as this stent does not require prolonged combination antiplatelet therapy.

Chronic kidney disease (CKD), with or without a history of diabetes mellitus, is a factor for the development and progression of cardiovascular disease and constitutes a risk factor for an array of adverse outcomes after myocardial infarctions and cardiac catheterization, including increased bleeding associated with platelet dysfunction, potential electrolyte abnormalities, and the increased risk of contrast-induced nephropathy [9, 13].

A history of renal insufficiency, or a creatinine in excess of 1.5, poses a greater risk of acute kidney injury following exposure to contrast dye [9]. To minimize the potential risk of contrast-induced nephropathy (CIN), the 2009 ACC/AHA Guidelines recommend the use of an iso-osmolar contrast agent (Class I, Level of Evidence A) or low-osmolar contrast agents (Class I, Level of Evidence B) during PCI [7]. Additionally, a number of studies have examined the benefit of various strategies to minimize the risk of contrast-induced nephropathy prior to a coronary intervention, including the administration of acetylcysteine (Mucomyst), the infusion of sodium bicarbonate, or hydration with normal saline. Recent systematic reviews and meta-analyses suggest there is little difference among the interventions, but data are limited due to significant heterogeneity among the studies. Future multisite studies are needed to answer this question definitively; in the interim, the MD and NP cardiology service has decided to continue the practice of administering Mucomyst pre-intervention to patients with an elevated creatinine due to the tolerability of Mucomyst and the lack of undesirable effects associated with the use, but will continue to examine the ongoing research in this area.

Many cardiovascular drugs are excreted via the kidney, necessitating adjustments of medications according to the individual patient's glomerular filtration rate (GFR). As part of the team approach, the laboratory provides a daily calculated GFR based on the patient's updated BUN/creatinine. All medication and dosages are adjusted according to the daily glomerular filtration rate, reducing the potential for complications and toxicities related to compromised renal function. The pharmacy reviews medications and suggests modifications based on patient condition and renal status.

### 8.2.6 Evidence-Based Goal #6: Provide Effective Anti-ischemic Therapy for Pharmacotherapy Pre-intervention and/or for Conservative Approach

Intensive therapies to treat anginal pain are often required and consist of vast array of pharmacotherapeutics: (a) nitroglycerins, (b) ACE inhibitors, and (c) beta-blockers. Nitroglycerin, a vasodilator, thought to increase perfusion to the affected areas of myocardial tissue, is administered via a number of routes, including sublingual, skin patch, and intravenous, within the first 24 h of admission. A meta-analysis of six trials, all of which were powered to detect a reduction in mortality at 2 and 10 days, reported a significant reduction in all-cause mortality with the use of nitrates [1]. This effect was not observed at 30 days. Upon initial presentation to the hospital, all patients with angina are medicated with sublingual nitroglycerin, and repeated every 5 min, up to three times for relief of angina discomfort. If anginal pain persists, an intravenous nitroglycerin drip is initiated and titrated to achieve relief of pain and hemodynamic stability (Class I, LOE C) [5].

Administration of an ACE inhibitor is recommended as Class I, LOE A treatment in patients with acute coronary syndrome and is initiated within the first 24 h of admission [3, 5]. ACE inhibitors induce vasodilation by blocking the effects of angiotensin II and aldosterone and are postulated to reduce the direct toxic effects of angiotensin on myocardial cells. The latest studies observed a nonstatistically significant reduction in all-cause mortality at 2 and 10 days, but noted a statistically significant lowering of all-cause mortality at 30 days [3, 5].

Within the first 24–48 h, beta-blocker therapy, a Class I, Level of Evidence B Recommendation, is initiated in all patients without evidence of heart failure [6]. Patients with suspected left ventricular dysfunction undergo echocardiographic testing within the first 48 h to assess myocardial contractility, ventricular function, and valvular function.

### 8.2.7 Evidence-Based Goal #7: Post-intervention Assessment of the Patient

Following PCI, patients are transferred to the cardiac step down unit where nurse practitioners, in collaboration with cardiologists and fellows, manage the post-intervention care including hemodynamic assessment, evaluation of the intervention site, assessment of possible complications, and follow-up of diagnostic tests. A specific protocol for frequent monitoring of the patient is implemented in the step down unit, which includes a frequent assessment of pulses in the affected extremity, hemodynamic assessment, EKG monitoring, complete blood count, and troponin evaluation within a 12-h period.

Hyperglycemia has been hypothesized to increase inflammatory markers in patients and contribute to an increased risk of cardiovascular mortality. The recommendation for intensive insulin management has recently been downgraded from a Class I, Level of Evidence A Recommendation, to a Class I, Level of Evidence B Recommendation, owing to an increased risk of hypoglycemia and early death with

intensive insulin regimens. Insulin therapy is recommended to achieve a glucose level of less than 180 mg/dl.

### 8.2.8 Evidence-Based Goal #8: Prescribe Evidence-Based Antiplatelet Therapy Post-intervention

When drug-eluting stents (DES) are implanted, dual antiplatelet therapy is recommended for the first year. Although ASA 162325 mg is recommended for at least 6 months following PCI, the AHA/ACC report suggests that ASA 81 mg may be safely used for the first year of therapy [5]. To ensure patency of the stent, the addition of thienopyridine is recommended for 1 year. Treatment with dual antiplatelet therapy requires targeted education for the patient and family regarding the risks, benefits, and complications, including the potential for bleeding. If an individual is deemed ineligible for this therapy due to lack of compliance and/or contraindications to antiplatelets, the decision is made to implant a bare metal stent at the time of the coronary intervention, eliminating the need for prolonged, dual antiplatelet therapy.

### 8.2.9 Evidence-Based Goal #9: Blood Pressure Reduction to Less Than 130/80

- *Beta-Blockers*: In patients with acute coronary syndrome, specifically those individuals who sustain a myocardial infarction, beta-blockers, as a class of medications, inhibit the effect of catecholamines and the resulting sympathetic activation in the heart and are associated with a reduction in blood pressure, prevention of myocardial remodeling, and a decrease in the reduction of ischemic events. After the first 24- to 48-h window, beta-blockers remain an important level I recommendation for patients with ACS [5]. A meta-analysis involving 36 randomized controlled trials examined the difference in blood pressure with the use of beta-blockers and discovered a 29% reduction in coronary events in patients with a previous history of CHD [10]. Sub-analyses revealed a greater benefit in patients who began beta-blocker therapy during the time of the acute myocardial infarction with a relative risk reduction of 31% [10]. Beta-blocker administration is associated with a significant reduction in both systolic and diastolic blood pressure, heart rate, and all-cause mortality at 30 days [11]. Strong evidence, to date, supports the need for all patients post myocardial infarction to initiate beta-blocker therapy, especially those individuals with a reduced ejection fraction. The recommended approach is to initiate metoprolol, a selective beta-blocker and Class I LOE A Recommendation for the secondary prevention of reinfarction or death in all patients [7]. For those patients with left ventricular dysfunction, beta-blocker therapy is initiated at a low daily dose of 50 mg and is slowly titrated to an optimal dose as tolerated by the patient [5]. Patients are instructed to report significant side effects to their primary providers including a low pulse

rate, lethargy, and sexual dysfunction. An alternative beta-blocker may be selected if side effects become intolerable.

- *ACE Inhibitors:* ACE inhibitors (ACEI) are prescribed for all patients treated for unstable angina, NSTEMI, and STEMI (Class I, LOE A), and are particularly beneficial in those patients with impaired left ventricular dysfunction, diabetes, and hypertension, unless a specific contraindication exists due to impaired renal function with a reduced glomerular filtration rate and/or hyperkalemia [3, 5–7]. To ensure that patients receive an ACE inhibitor upon discharge and comply with this recommendation, all discharge summaries require the NP to confirm the addition an ACE inhibitor to the medication regimen, particularly when the patient has sustained a myocardial infarction or if the left ventricular ejection fraction is less than 40%. If an ACE inhibitor is not prescribed, each provider must document the rationale for excluding this therapy.

### 8.2.10 Goal #10: Prescribe Evidence-Based Risk Factor Reduction Strategies for Secondary Prevention

Aggressive risk factor reduction of modifiable cardiac comorbid conditions is required for all patients with acute coronary syndrome. The NP prescribes therapies to promote effective secondary prevention including statin therapy, dietary counseling and physical activity.

- *Lipid management:* Lipid management is an important component of both primary and secondary CV prevention and is managed by the NP provider. Consistent with the Class I, Level of Evidence A Recommendation from the ACC/AHA, within 24 h of admission, all patients undergo an assessment of a fasting lipid panel and baseline liver function tests [3, 5–7]. If the LDL level exceeds the goal of less than 70 mg/dl, and the liver tests are within the normal range, pharmacotherapy is initiated with a statin. High-level doses of a statin are recommended for patients who have sustained a non-ST-segment elevation and ST-segment myocardial infarction. Follow-up should include assessment for complaints of muscle aches and periodic evaluation of liver function tests.
- *Tobacco cessation:* Tobacco smoking is a significant risk factor for the development of cardiovascular disease. In this Upper East Side Community, with an incidence of premature cardiovascular disease that surpasses most other communities, 27% of residents in East Harlem smoke cigarettes compared with only 17% of New Yorkers [1]. Upon admission to the hospital, all NPs and MDs document the patient's smoking status. During the admission, all patients who smoke are advised to quit smoking. Assessment of the individual's readiness for smoking cessation is ascertained, and, if the patient consents, nicotine replacement therapy is initiated based on a number of high-quality systematic reviews and meta-analyses that have consistently suggested that all forms of nicotine replacement are effective in reducing smoking rates, with some analyses revealing a success rate of 50–70% [16]. Upon discharge, all patients who smoke receive education materials designed to enhance smoking cessation.

- *Healthy eating with the DASH diet:* The American Heart Association has redefined the goals for cardiovascular health and disease reduction for 2020 with the emphasis on preventing the development of risk factors instead of treating risk factors [12]. The group defined the ideal BMI as equal or below 24.9 and described a range of healthy practices to achieve this goal [12]. Among the most important methods to achieve the goal of cardiovascular health is the emphasis on healthy nutrition. The group has endorsed the Dietary Approaches to Stop Hypertension or DASH diet, a diet designed to reduce the development of coronary artery disease [12]. In our unit, all patients are educated on the principles of the DASH diet, including an emphasis on at least 4.5 cups of fruits and vegetables per day, intake of fish two times per week, the need for an increase in fiber and whole grains, restriction of sodium to less than 1,500 mg/day, and the elimination of sugar sweetened beverages. Nurse practitioners, nurses, and dieticians employ a number of different strategies including individual consultations, nutrition classes, and educational pamphlets regarding healthy dietary habits. Admission to the hospital represents an opportunity for the team to teach healthy eating practices to both patients and family members.
- *Physical activity: evidence-based goal: 30 min of aerobic exercise 5 days a week:* All patients receive a prescription for physical activity post-intervention. Patients are instructed to avoid heavy lifting and vigorous exercise for the first 5–7 days. Thereafter, they are advised to walk every day as tolerated to achieve a goal of 30 min of aerobic exercise 5 days a week [12].

## 8.3 Conclusion

Social and economic disparities disproportionately affect multiethnic and multiracial communities. Consistent with studies that indicate that guideline-driven cardiac care reduces racial and ethnic disparities, this innovative model of emergency cardiac care has addressed some of the inequities in emergency cardiac care while, at the same time, improving the overall safety record of the aforementioned medical center [2]. Evidence-based emergent assessment and treatment as well as primary and secondary prevention of acute coronary syndrome are necessary in improving safety and quality of patient care and outcomes.

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# The Patient Centered Medical Home

# 9

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**Core Message**

The Patient Centered Medical Home (PCMH) is an innovative evidence-based model of care delivery that is focused on the core concepts of primary care which include easy access, comprehensive and coordinated care, and the development of relationships over time. The redesigned PCMH practice which emphasizes a team approach rather than a provider approach is facilitated by the use of an electronic medical record (EMR).

## 9.1 Introduction: The Patient Centered Medical Home

### 9.1.1 PCMH a Model of Care Delivery

The Patient Centered Medical Home (PCMH) is a model of care delivery that is defined by an ongoing relationship between a provider and patient where the patient is at the heart of centralized and comprehensive coordinated care. The PCMH is an innovative care model that supports not only the core primary care principles but also relationship-centered patient care, reimbursement reform, new information technology, and the chronic care model.

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This chapter is dedicated to Dr. Patricia Bloom who is always very helpful to the nurses in our practice, and for her never-ending guidance, patience, and support.

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This new health care model emphasizes a practice-wide team approach that focuses on disease prevention as well as provision of accessible high-quality and cost-effective health care for patients with acute and chronic medical problems [1, 2]. The PCMH is thought to be a solution for the US health care crisis [3].

As individuals are living longer with multiple comorbidities, providers end up spending more time and resources coordinating care. The number of primary care (physicians) graduates has been steadily declining since 1998 [4]. In addition, physicians are choosing to become specialists rather than primary care providers. It is predicted that by the year 2025, there will be a 20–27% shortfall of primary care doctors [5].

The PCMH model is considered to be one of the most promising care delivery systems that can improve patient outcomes as well as limit the cost of health care. Because of this, there are numerous demonstration projects underway in multiple states that are being funded by various organizations and health care systems to convert practices to PCMH model of care. Over the next few years, it is predicted that thousands of practices will attempt to convert their sites into a PCMH.

### 9.1.2 History of the PCMH

The first known documentation of the term "medical home" appeared in Standards of Child Health Care, published by the American Academy of Pediatrics (AAP) in 1967. Medical home was originally defined as one central source of a child's pediatric records – a centralized medical records – especially for children with special needs.

It was not until the 1970s when the AAP began to address the policy implications of the term "medical home" that the model became more clearly defined. The PCMH concept evolved from a focus on centralized medical records to one of providing primary care at a community level and recognizing the importance of addressing the needs of the total child and family [6].

The concept changed and developed throughout the years, and finally, in 2007, the American Academy of Family Physicians (AAFP), American Academy of Pediatricians (AAP), the American College of Physicians (ACP), and the American Osteopathic Association (AOA) issued a joint statement defining their vision of the PCMH. This vision includes seven core concepts [7]:

1. Personal Physician: Patient and physician have an ongoing relationship for a continuous and comprehensive care.
2. Physician-Directed Medical Practice: The physician leads a team of individuals at the practice level. The team collectively takes responsibility for the ongoing care of patients.
3. Whole Person Orientation: The physician is responsible for providing for all the patient's health care needs including the responsibility for appropriately arranging care with other qualified professionals. This includes care for all stages of life: acute care, chronic care, preventive services, and end-of-life care.
4. Care Coordination and/or Integration: Care across all elements of the complex health care system (e.g., subspecialty care, hospitals, home health agencies,

nursing homes) and the patient's community (e.g., family, public and private community-based services) is coordinated. Care is facilitated by registries, information technology, health information exchange, and other means to assure that patients get the indicated care when and where they need and want it in a culturally and linguistically appropriate manner.

5. Quality and Safety as Hallmarks of the Medical Home:
   - Practices advocate for their patients to support the attainment of optimal, patient-centered outcomes that are defined by a care planning process, driven by a compassionate, robust partnership between physicians, patients, and the patient's family.
   - Evidence-based medicine and clinical decision-support tools guide decision making.
   - Physicians in the practice accept accountability for continuous quality improvement through voluntary engagement in performance measurement and improvement.
   - Patients actively participate in decision making, and feedback is sought to ensure patients' expectations are being met.
   - Information technology is utilized appropriately to support optimal patient care, performance measurement, patient education, and enhanced communication.
   - Practices go through a voluntary recognition process by an appropriate nongovernmental entity to demonstrate that they have the capabilities to provide patient-centered services consistent with the medical home model.
6. Enhanced Access to Care: Open scheduling, expanded hours, and new options for communication between patients, their personal physician, and practice staff are available throughout the system.
7. Appropriate Payment: Added value provided to patients with patient-centered medical home is recognized. The payment structure should be based on the following framework that:
   - Reflects the value of physician and nonphysician staff patient-centered care management work that falls outside of the face-to-face visit
   - Pays for services associated with coordination of care both within a given practice and between consultants, ancillary providers, and community resources
   - Supports adoption and use of health information technology for quality improvement
   - Supports provision of enhanced communication access such as secure e-mail and telephone consultation
   - Recognizes the value of physician work associated with remote monitoring of clinical data using technology
   - Allows for separate fee-for-service payments for face-to-face visits (Payments for care management services that fall outside of the face-to-face visit, as described above, should not result in a reduction in the payments for face-to-face visits.)
   - Recognizes case-mix differences in the patient population being treated within the practice

## 9.2 Why the PCMH Now?

### 9.2.1 United States Health Care System

The United States Health Care System is facing an increasingly elderly population requiring more chronic, complex, and costly medical care. Fewer medical students are now choosing primary care career paths due in part to the current reimbursement structure and overwhelming workload [8].

The traditional primary care structure is often fragmented, with the physician provider operating separately from the other interdisciplinary team members. This fragmented structure can lead to poor collaboration between the team members resulting in decentralized patient care. In addition, the current payment structure of fee-for-service reimbursement encourages maximum utilization with little or no incentive toward quality. Primary care practices often focus on the number of care visits generated rather than the quality of visits [8].

More than half of the Medicare beneficiaries receive treatment for five or more chronic conditions throughout each year [8]. Many elderly patients see multiple providers for each chronic illness. The lack of coordination and continuity of care leads to poor medical management and often results to negative patient satisfaction and negative health care outcomes. Fragmented, ineffective, inefficient, and impersonal patient visits cause patient dissatisfaction [8].

With PCMH, focus will be on quality rather than quantity, where providers will be able to spend more time with patients. All health care team members work together to improve care coordination as well as to improve effective communication in a timely manner (with the utilization of an EMR).

### 9.2.2 Strengths of the PCMH

The Medical Home Model of Primary Care: Implications for the Healthy Oregon Act (2007) along with other various comprehensive studies discusses the benefits of having a medical home:

- Shi and Baicker noted that states and countries with a strong primary care system have better health outcomes and lower health care costs, while those areas with more specialists have higher health care costs and lower quality of care [9–12]. Backus's study also demonstrated that improved access to primary care (where patients can easily access and receive efficient and timely care from a regular source anytime including weekends and evening times) results in decreased hospitalization [12, 13]. Patients who receive care from a regular source with easy phone access (including on weekend/evening), and efficient on-time visits can receive better care [12].
- Continuity of care which is defined as seeing the same provider over time is consistently associated with decreased ED visits, decreased hospital admission, increased patient satisfaction, and lower costs of care resulted in studies done by Saultz, Lochner, and the Robert Graham Center [12, 14, 15].

- Team-based approach to care, especially in patients with chronic illness, shows that a multidisciplinary team yields higher patient satisfaction, improved quality of life, better health outcomes, and decreased cost of care as demonstrated in the National Diabetes Education Program [14, 16].
- Coordination of care: Patients receive personal-based coordination of health care at the level of their primary provider, rather than coordination based on a specific disease or condition [12].
- Health information systems (EMR) will "form the basis of many quality improvement efforts" [17, p. 10] to help maintain and improve the health of our patients at the primary care level as well as reduce primary care practice costs [12].

### 9.2.3 Barriers to Implementing the PCMH

As stated earlier in this chapter, certain principle requirements must be met before a practice can qualify as a PCMH. Many practices tried to implement these principles separately but did not realize that the principles are interdependent and can impact on workflow in the practice. Due to this, the principles of the PCMH need to be implemented simultaneously.

This often requires a redesign of the practice which can be overwhelming for all team members. Frequently, individual roles, the practice identity, and a shared vision all need to be changed. Historically, practice redesign aims to enhance the physician workflow, whereas in the PCMH model, the practice is redesigned to enhance patient experience. This paradigm shift requires a continuous process of change, not an incremental one [18]. Following are identified as some of the barriers to PCMH implementation:

1. *Technology (use of EMR)*

One requirement for the implementation of the PCMH is the use of an EMR. This new technology (e-prescribing, disease registries, patient portals, etc.) is more difficult to implement and more time-consuming than initially thought. It frequently requires the staff to learn more sophisticated work programs on the computer. This task is made more difficult because workflow processes needed to be defined before implementation of the PCMH rather than after [18].

2. *Transformation of physicians*

Transforming a medical practice into a PCMH requires a different approach to patient care. This is especially challenging for physicians because they need to shift the way they practice from a physician-led model to a team-based approach. It often requires a leader to be a facilitator rather than an authoritative figure [19]. The transformation will impact on the physicians' relationship with other team members as well as their patients. They need to start shifting their focus from one patient at a time to a population-based approach for chronic disease management and preventative services. This can be a substantial barrier to the adaptation of the PCMH, as it requires physicians to change their professional identity as well as the way they deliver patient care [18].

3. *Change fatigue*

Significant changes are required when a practice transitions into the PCMH. Staff may become overwhelmed by the need to make numerous and frequent changes. In order for a practice to successfully transition into a PCMH, the staff must adapt and maintain the change while preparing for the future. If these changes are implemented too quickly, it may lead to staff burnout and staff turnover as well [18]. There were few practices enrolled in the initial NDP that completed the transition within 2 years even with facilitation. It is likely that a practice's successful transition to a PCMH will take more than 2 years and will require the staff and leadership to be highly motivated with significant time and resources and possibly outside facilitation [19].

4. *Cost*

Transforming to a PCMH costs money (as well as time and effort).The current available funding opportunities and reimbursement are often inadequate for the transitional costs which can disenfranchise smaller medical practices. Most practices lack adequate funding to support the advanced technology that is required to transition into the PCMH. Capital, operational, and educational resources are necessary requirements toward a meaningful implementation of the PCMH in primary care practices [19].

## 9.3 PCMH Projects: Evidence-Based Outcomes

### 9.3.1 Evidence-Based Outcomes and PCMH

Since its inception in 1967 in pediatrics, the PCMH model has expanded to include the entire life cycle. Numerous demonstration projects have supported its effectiveness in the delivery of timely, transparent, and collaborative interdisciplinary care for patients with complex medical problems including preventive care and the treatment and management of chronic disease.

Successful transition into a PCMH depends on "adaptive reserve" – flexibility in making structural changes. Structural changes occur through organizational learning, development, and facilitative leadership [20]. Structural changes which do not require significant alterations in current structure, such as same-day patient appointments and electronic prescribing, will be easier to change than those that require fundamental changes [21].

In 2005, AAFP funded a 2-year National Demonstration Project and created TransforMed (see Fig. 9.1), a division of AAFP, to conduct the project. The NDP was launched in 2006 as a 2-year intervention to test the PCMH model in 36 family practices participating in the project. The results of the NDP showed positive outcomes including better prevention and management of chronic diseases [17].

In 2009, the Center for Medicare and Medicaid Services (CMS) began accepting applications from practices to participate in another 2-year National Demonstration Project to determine whether the medical home model produces savings and quality improvements. This project spanned eight states and involved 400 practices, 2,000

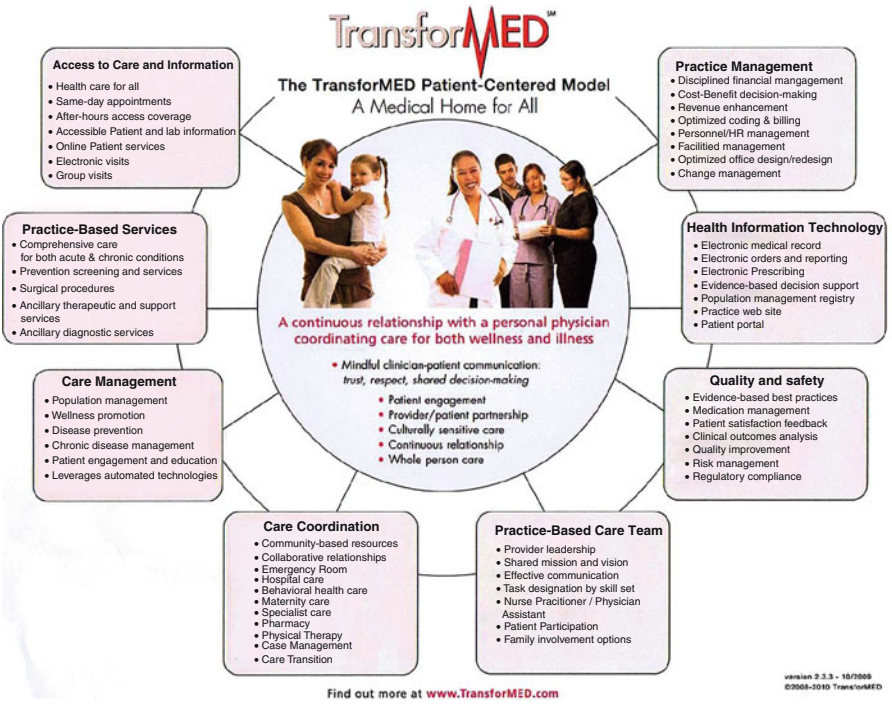


**Fig. 9.1** The TransforMed patient-centered model

physicians, and 400,000 Medicare beneficiaries. The time frame was from January 2010 to December 2010 [22]. Results are still pending.

### 9.3.2 Group Health Cooperative at Puget Sound

In 2006, Group Health Cooperative (a large health care delivery system in Seattle) piloted a practice representing the medical home vision. This project examined the medical home vision's impact on patient experience, cost, clinical quality, and staff burnout. Evaluation was done after 12 months and after 24 months of implementation. The results showed that medical home patients reported better experience at 12 months (80% response rate) in the quality of doctor–patient interaction, shared decision making, access to care, and helpfulness of office staff compared to the control group's experience.

At 24 months (70% of baseline respondents), patient reported better experience in coordination, access, and goal setting with moderate improvement in the quality of doctor–patient interactions. Clinical quality including screening, chronic illness care, and medication monitoring to name a few noted with greater improvement at 12 months and continued to improve at 24 months by 20–30% greater in three out of four composites [23, 24]. The PCMH staff reported a 10% high emotional exhaustion at 12 months compared with 30% in the control group [23, 24].

The cost-benefit analysis of the PCMH intervention demonstrated that for an additional cost of approximately $8.00 per member per month, there was an overall decrease in member cost of $4.00 per month due to a reduction in emergency room/urgent visits and inpatient admissions. This reduction provided a total cost savings of $14.00 per patient per month [23].

The researchers also found that individuals enrolled in the PCMH had fewer in-person primary care visits when compared with patients in other clinics; however, patients had more secure e-mail exchanges and telephone calls with members of their care teams.

In addition, many of the PCMH components were implemented including pre-visit outreach, emergency room follow-up, group visits, and self-management support workshops [24]. There was also improvement in patients' experience on patient involvement in their care, goal setting, and care coordination [24].

### 9.3.3 The Geisinger Medical Home Initiative

Geisinger Health System is a physician-led health care system founded in 1915 with approximately 700 physicians and 200,000 patients. In 2006, Geisinger implemented a medical home project to improve chronic disease management, health promotion, reduce hospitalizations, and decrease length of inpatient stays [25].

The results of the Geisinger pilot study showed a 20% reduction in hospital admissions and 7% savings in medical cost. For chronic disease management, initial results from approximately 20,000 diabetic patients found improvement in glucose and blood pressure control and vaccination rates [26]. The results also noted higher patient and staff (including physicians) satisfaction [27].

### 9.3.4 Community Care of North Carolina (CCNC)

The CCNC program was created in the late 1990s. This program had approximately 1,200 primary care practices in North Carolina that followed 750,000 Medicaid patients. These 1,200 practices represented 50% of North Carolina primary care practices. The CCNC program successfully implemented many features of the PCMH.

The practices provided continuous comprehensive primary care across multiple settings which included referrals to specialist, enhanced access to care, and 24-h on-call coverage. The CCNC model had not adopted practice redesign, and there was limited use of electronic medical records [28].

CCNC showed improved quality of care of chronic diseases such as asthma, diabetes, and hypertension. Asthma care and management was greatly improved. There was a 21% increase in asthma staging and a 112% increase in the numbers of asthmatic patients who received influenza vaccines. Emergency visits of children with asthma decreased by 8% and reduced rate of hospitalization by 34% during the first year of the program.

In addition, these rates of reduction in emergency room visits and hospitalization have been maintained [29]. Results also noted that diabetes control and blood pressure control (in most areas) exceeded the National Committee for Quality Assurance (NCQA) benchmark. The CCNC model implemented many core features of the PCMH including improved chronic disease management, preventive services, and comprehensive primary care [28].

## 9.4 The National Committee on Quality Assurance (NCQA)

### 9.4.1 PPC-PCMH

The National Committee on Quality Assurance (NCQA) in 2007 developed a tool, Physician Practice Connections: Patient-Centered Medical Home (PPC-PCMH), which set the standards for recognition as a medical home. There are nine standards containing 30 requirements that each has a point value.

A practice must accrue a certain number of points in order to achieve a certain level. Each level also has six "must-pass" elements which the practice must meet in order to obtain that level [30].

The nine standards are facilitated by use of an electronic medical record. These standards focus on access and communication, patient tracking and registry function, care management including patient self-management, electronic prescribing, test and referral tracking, and quality improvement and advance electronic communications [30].

The 2011 NCQA standards that a practice must meet to qualify as a PCMH are summarized below. These reflect the table of standards published in Standards and Guidelines for NCQA's PCMH 2011 [30].

1. Provide enhanced access and continuity of care
   - Trained staff providing team-based care.
   - Practice enables patient access to care 24/7 for patients facing barriers of language, culture, or disability.
   - Patients can access their medical records and contact their provider electronically.
   - Patients can select which provider they see.
2. Identify and manage patient populations
   - Practice collects demographic and clinical data to better manage care of its patient population.
   - Practice assesses and documents patient risk factors.
   - Practice provides proactive reminders to providers about health maintenance and follow-up care.
3. Plan and manage care

   Care management is based on:
   - Pre-visit planning
   - Assessing patient's progress toward treatment goals
   - Addressing barriers to achieving treatment goals

- Identifying specific diseases that are prevalent in the patient population
- Promoting medication management and using e-prescribing

4. Provide self-care support and community resources
   - Practice assesses self-management ability of patient and family.
   - Practice develops self-care plan with patient and family and provides appropriate tools and resources, including community resources.
   - Patients counseled on healthy behaviors by practice clinicians.
   - Practice assesses and facilitates treatment for mental health and substance abuse.
5. Track and coordinate patient care
   - Practice tracks, follows-up on and coordinates tests, referrals and care with other health care facilities.
   - Practice manages care transitions from inpatient to outpatient settings and between other health care facilities.
6. Measure and improve performance
   - Practice uses treatment success and patient experience data for ongoing improvement in care.
   - Practice tracks utilization measures such as patients' frequency of hospitalization and visits to the emergency room.
   - Practice identifies vulnerable patient populations.
   - Practice demonstrates performance improvement through patient satisfaction surveys and reduced hospitalizations [30].

### 9.4.2 NPs as Primary Care Providers

The move to utilize the nurse practitioners as primary care providers in the PCMH model stems partly from the critical shortage of physicians as primary care providers. As the nurse practitioner role continues to evolve and expand, so does literature on the quality of NP services.

Over the last 40 years, there have been many studies done on the quality of NP practice in various outpatient settings. Brown and Grimes in 1995 did a meta-analysis of 38 studies comparing a total of 38 patient outcomes and found that NP care was equal to or greater than MD care.

NP-managed patients had higher levels of medication compliance, patient satisfaction, and resolution of pathological conditions than MD-managed patients [31]. In another study done in 2009, Mundinger and colleagues compared patient outcomes in the management of some chronic diseases like hypertension and asthma. The study found that patients with hypertension did better under NP management as compared to that of the physicians. There was no difference, however, between NP and physician management for patients with asthma [32].

In a study done by Shum and colleagues, looking at nurse management of patients with minor illness in general practice, the researchers found that nurse practitioners gave more information to patients and offered more advice on self-care and management [33]. Several studies found that NPs provided care that was equal to the care provided by physicians and in some cases more effective [34, 35].

Sacket and colleagues in 1974 had a sample of 1,598 families, and these families were randomized to receive care from an NP or an MD. Outcomes of the study included mortality, physical function, and emotional and social function. The researchers found that patients had no significant difference in outcomes whether followed by an NP or MD [36]. Studies such as these aforementioned provided support for the NPs as reliable and qualified professionals to take the role of primary care providers (PCP). NPs as PCPs are fundamental to the PCMH.

In 2009, the ACP endorsed the idea of an NP-led PCMH, subject to the same eligibility requirements as physicians [4]. By 2025, the United States could face a shortage of 44,000 primary care physicians because many physicians are choosing not to go into primary care due to reimbursement and workload issues [37]. Facing a shortage of primary care physicians provides an even more reasons for NPs to lead the PCMH. NPs impact on the following concepts of the PCMH:

1. *Personal health care provider instead of personal physician*

NPs have been providing primary care to patients for more than 40 years. They manage acute and/or chronic illness, and emphasize disease prevention and health promotion in their practice leading to better patient outcomes [38]. According to the statistics from the University of Southern California Center for Health Professions, eleven states permit NPs to practice independently, 27 states require NPs to practice in collaboration with an MD, and 10 states require MD supervision for NPs [39]. Forty-four states allow NPs to develop diagnosis and management plan in collaboration with physicians. In addition, 20 states permit NPs to order diagnostic test, and 33 states allow NPs to refer to specialist [39].

2. *Primary care provider–directed practice instead of physician-directed practice*

NPs are authorized to practice and have prescriptive authority in all 50 states including the District of Columbia [38]. There may be some limitations dependent on the state in which the NP is prescribing in.

3. *Whole person orientation*

Nursing is a holistic profession which views individuals within the context of their families and the communities in which they live. NPs provide comprehensive patient care in numerous inpatient and outpatient settings. NPs enjoy partnering with patients so patients can achieve their goals [38].

4. *Coordinated and integrated care*

NPs manage patients throughout their life cycle and coordinate care with other disciplines including but not limited to physician colleagues. They are expert primary care providers. NPs have a long history of coordinating care with numerous specialists across the health care system whether within hospitals, home health agencies, and/or nursing homes. NP practice focuses on family and community-based services [38].

5. *Quality and safety*

Health care providers that provide patient-centered care should advocate for their patients. In the PCMH, the patient's opinion is actively sought. Feedback from the patients leads to continuous quality improvement. This process is facilitated by the use of an electronic medical record and a patient portal system. NPs are patient advocates. Through health promotion and disease prevention, NPs educate patients

on their illness and encourage patients to be proactive. Brown and Grimes (1995) found that NP-managed patients had better outcomes in diastolic blood pressure control and glucose control compared with physician-managed patients. The researchers also found that NP-managed patients were more compliant with taking medications and keeping appointments than physician-managed patients [31].

6. *Enhanced care*

In the PCMH, enhanced care is characterized by open scheduling, expanded hours, and options for communication among patients, their health care providers, and practice staff.

7. *Appropriate payment*

The PCMH model recognizes the need for payment to be equal to the advanced care coordination. With that said, it is important to keep in mind that practices reduce overall cost for the health care system. Cost savings have been documented in NP practices. In 2006, Paez and Allen compared NP and physician management of hypercholesterolemia following revascularization. The researchers found that NP-managed patients were more likely to achieve their goals, comply with prescribed medication, with reduced drug cost [40].

NPs have all the tools necessary to lead the PCMH. Many NPs have large patient panels. These patients have multiple and complex comorbidities. NPs see patients for acute and chronic illness and coordinate care with many different disciplines including social workers, ancillary support staff, primary care providers, and specialists. NPs see patients 7 days a week – 24 h per day.

NPs are always interested in patient feedback to improve care. They encourage their patients to be proactive and manage their medical problems through medication and lifestyle modification. NPs teach patients about their illnesses in one-on-one sessions or in group visits. The PCMH envisions collaborative team care in which the team leader may be an NP. Patients and practice perspectives need to transform into one wherein the physician is part of a team and not even necessarily the team leader [19].

## 9.5 A Geriatric Scenario of the PCMH

### 9.5.1 Senior Patients

Geriatric care is labor and time intensive. Many patients are older, sicker, and frailer. There are limited resources available for geriatric care. Many staff members, such as social workers, medical technicians, and front desk staff, do not receive reimbursement for services provided. In addition, geriatric care is primarily non-procedure-based care, unlike care that is provided in cardiology and gastroenterology.

The core concepts of primary care include a responsibility for patients over time. This comprehensive and coordinated care focuses on meeting the health care needs of the patients in the context of their home environment and relationships with family and friends. It often requires that the provider deal with the patient's multiple medical problems and numerous care providers in many different clinical settings. These concepts apply to both geriatrics and the PCMH [21].

A true facilitative leader, following the NCQA guidelines, would be able to implement the PCMH in a geriatric outpatient practice. Initially, staff members would complete questionnaires focusing on how to change and improve the current patient care delivery system.

The staff would then focus on improving patient care through improved access, patient education, and promotion of self-management techniques in order to avoid hospitalization and reduce the number of urgent visits. Other areas for improvement would include patient and staff satisfaction, collaboration with specialists, and utilization of health information technology.

Implementation of the PCMH should occur over time. Implementation would be facilitated by quarterly retreats, monthly staff meetings, and daily team huddles to discuss patient care issues. A truly facilitative leader would encourage all team members to take ownership of the PCMH. The overall goal of the PCMH would be to improve patient and staff satisfaction while decreasing the number of emergency room visits and inpatient admissions. In order to achieve this goal, the staff would provide comprehensive and centralized care on a daily basis which is best illustrated by the case studies listed below.

### 9.5.2 Case Studies: Geriatric Scenarios

**Case study #1**
Mrs H. is a 77-year-old female with chronic obstructive pulmonary disease, atrial fibrillation, hypertension, urinary frequency, diabetes, and hypercholesterolemia. Her husband died in early December 2010. She was seen in the practice on December 21, 2010 for routine follow-up exam. Prior to this visit, the patient was treated with Bactrim for a urinary tract infection on December 14, 2010 but developed angioedema after taking Bactrim.

The patient was seen in the emergency room and was given Benadryl. Medications included: albuterol inhaler prn, warfarin 4 mg at bedtime, lisinopril 5 mg daily, Spiriva inhaler daily, gabapentin 300 mg at bedtime, metformin 1,000 mg twice a day, and simvastain 40 mg at bedtime. On December 21, 2010, the patient complained of feeling weak, tired, and a "little hazy." She believed that she had urinary infection but denied dysuria, urinary incontinence, and urinary frequency. Patient had no other pertinent review of systems.

Physical examination revealed a well-developed, well-nourished elderly female who was very pleasant and did not appear ill. Blood pressure was 100/60, pulse was 72, and physical exam was significant for a benign abdominal exam, no costovertebral tenderness, and a sad affect with tearfulness.

After discussion with the patient's primary care provider, the NP began a workup for urinary tract infection which included complete blood count with differential, urine culture, urinalysis, and post-void residual with bladder scanner. In addition to

the urinary tract infection workup, a secondary diagnosis was hypertension which was well controlled on current medication. Patient was also referred to the social worker for bereavement counseling.

After completion of visit, patient attempted to get out of chair but felt dizzy. The NP checked patient's vital signs and found lying blood pressure was 100/60 with pulse = 64 and standing blood pressure 80/60 with pulse = 64. After discussion with the primary provider, the NP ordered an intravenous infusion of normal saline at 125 cc/h for 5 h and a basic metabolic panel.

Patient then spent the next 5 h in the practice where she was monitored by a nurse and the NP. Initial laboratory results were reviewed to rule out possible cause of dizziness like anemia, infection, hypo/hyperglycemia, or dehydration to name a few. An intravenous line was placed by a staff nurse, and a medical technician sent the additional blood test.

The patient was moved to another room for intravenous hydration. After completing intravenous hydration, the patient's blood pressure was 100/60 lying and standing and pulse 64 lying and standing. The NP recommended that patient hold the lisinopril and follow up 3 days later in the geriatric practice.

This case study illustrates key concepts of the PCMH and primary care. The patient had access to an urgent appointment, received intravenous therapy and stat labs, and had close follow-up in the geriatric practice. Care was coordinated between all team members, and hospitalization was avoided.

**Case study #2**

The patient is an 87-year-old female with hypertension, chronic obstructive pulmonary disease, and diabetes seen for evaluation of cough on April 29, 2011. The patient's medications include glargine six units subcutaneous daily, diltiazem cd 180 mg daily, albuterol multidose inhaler prn, baby aspirin 81 mg daily, and atrovent inhaler twice a day. According to her daughter, the patient had not been feeling well. She had been coughing and wheezing all night. The cough was productive of white sputum. The daughter had been giving the patient Robitussin (a cough medication) which did not help. The patient stated that she had runny and congested nose. She denied fever, chills, sore throat, shortness of breath, and nasal congestion. No other pertinent review of systems.

Physical examination revealed a thin Hispanic female with dry cough, shortness of breath, and audible wheezing. Physical examination findings were unremarkable except for the following: oropharynx erythematous with no exudates, distant s1 s2: pulmonary exam with inspiratory/expiratory wheezing base to top and posterior to anterior and poor air flow, and anxious appearance.

The NP ordered the following: albuterol nebulizer administered by staff nurse, chest x-ray, Medrol dose pack, azithromycin 5-day course, and complete blood count with differential and follow-up with the NP in 3 days. After the albuterol

nebulizer was administered – lung auscultation was found to be clear with normal breath sounds. Patient's blood pressure was well controlled on current regimen.

Patient went for chest x-ray which revealed a normal size heart; no focal consolidation; a 7-mm nodular density overlying the right eighth posterior rib, likely representing a granuloma; no radiographic evidence of pulmonary edema; no pleural effusion; no pneumothorax; and no focal infiltrate. Initial laboratory results were reviewed by the NP.

Patient was seen 3 days later in geriatric practice. According to the daughter, there was no improvement in shortness of breath, chest tightness, or productive cough. The NP spoke with the patient's attending physician and agreed to send patient to emergency room. The patient was sent to emergency room via escort. The emergency room was notified about the patient, and the inpatient teams received a progress note via EMR.

Both case studies demonstrated care coordination in a geriatric outpatient practice. In the first case study, hospitalization was prevented by intravenous hydration, blood testing, and monitoring by nursing staff. In the second case study, although hospitalization was not prevented despite close follow-up in the practice, it captured in a timely manner the need for an ED referral for more work-up and intervention. The outpatient team communicated with the inpatient team via EMR facilitating an easy transition for the patient. The case studies demonstrated team work, care coordination, and access to care.

## 9.6 Conclusion

Although the PCMH is a new model of care, numerous demonstration projects have already provided support for its effectiveness in a patient-centered care within an interdisciplinary-coordinated care system. It requires the philosophy of a practice shift from a physician-focused approach to a patient-centered approach. This model promotes the core concepts of primary care, namely, easy access to care, comprehensive/coordinated care, and the development of personal relationships over time.

The PCMH utilizes evidence-based medicine for chronic disease management and prevention. It is facilitated by the use of an EMR which provides for population metrics, supports for care programs, and tracks billing issues [19]. There is a reason to believe that the PCMH will improve quality and decrease cost over time. The PCMH payment model will encourage investment in practice changes to promote quality rather than incentives for increased volume.

With the predicted shortage of physicians in primary care, NPs are at the right place to assume a lead role in this innovative care model – the PCMH. NPs have all of the attributes necessary to assume the role of the primary care provider. NPs work well within the structure of a team by working with all team members to coordinate care for patients with multiple comorbidities. Nursing views the patient within the context of family and environment providing more holistic care. Numerous studies as mentioned in this chapter cite NPs providing high-quality and cost-effective care.

The PCMH represents the essentials for better primary care by improving delivery of chronic care. It also supports active partnership with informed patients, synergized by appropriate use of information and communication technology [18]. This model is still evolving. Adequate funding from state, federal, insurance companies, and other health system resources is mandatory for successful implementation across various practice settings. The PCMH is an exemplary evidence-based care delivery system characterized by high-quality, low-cost, collaborative/interdisciplinary care and coordinated across multiple health care systems.[1]

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[1]The concepts discussed in this chapter apply as well to the first part of this book, and specifically to the Patient-Centered Dental Home/Neighborhood [41]

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# Transitional Care

# 10

Maria Tereza Lopez-Cantor

**Core Message**

Transitional care is an innovative evidence-based care delivery system where services are provided collaboratively by the health care team to vulnerable patients as they move from one point of care setting to another. Transitional care is an innovative evidence-based care delivery system where services are provided collaboratively by the health care team to vulnerable patients as they move from one care setting to another.

## 10.1 Introduction: Transitional Care in Health Care

### 10.1.1 Is there a need for Transitional Care in Health Care?

Transitions, regardless of care settings, can be overwhelming to patients, their families, formal and/or informal caregivers, their hospital, and community primary care provider. This is largely because health interventions can significantly impact at any point of these transitions.

Transitioning from hospital to home, for example, where responsibility is transferred from the inpatient provider or hospitalist to the patient and primary care provider can be difficult [1]. Expectations for assuming responsibility for self-care such as understanding instructions on beginning new treatments, discontinuing some medications, and/or changing medication dosages/schedules as they return home from the hospital can add greater challenges to patients and their caregivers [2–4].

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Transitioning care requires planning and coordination of care prior to discharge to prevent patient dissatisfaction, adverse events, and frequent hospital readmissions [5–8]. Tsilimangras et al. [9] identified transitional care as one of the key areas of opportunity to improve post-discharge care. To accomplish this, effective communication is essential. Evidence suggests that deficit in communication by hospital medical providers to primary care providers are a common barrier to a successful discharge [10].

One of the earlier strategies to communicate information from inpatient to outpatient is the patient discharge summary. This form of communication, however, has been imperfect because it is often inadequate in providing important administrative and medical information, such as the primary diagnosis, results of abnormal diagnostics, particulars about the hospital course, follow-up plans, pending laboratory test results, and patient and/or family counseling [11].

For example, when results of laboratory tests do not arrive in a timely manner or are not communicated to the primary care provider (PCP) [10, 12–14], appropriate follow-up treatment, and visits may be delayed or missed. An elderly patient with multiple medications who have cognitive limitations (like dementia), poor social support, and inadequate ancillary services is even more vulnerable to missing follow-up visits and appropriate treatment during transitions.

These challenges result in increased emergency department and hospital utilization; health care costs; medical errors and adverse outcomes; and patient, caregiver, and provider dissatisfaction [5, 6, 15–18].

Innovative interventions, such as involving an advance practice nurse (APN) in the transition, may surmount some of the intrinsic difficulties this vulnerable group face [19]. Utilization of advanced practice nurses with patients with congestive heart failure, for example, have been shown to result in decreased readmission rates after 90 days of discharge from the hospital, and reduced health care costs [20].

Similar results have also been seen in the geriatric patients with a variety of diagnoses using advanced practice nurses to bridge the vulnerable period of discharge, or by interventions to improve the ability of family caregivers to handle the challenge of transition [3, 21, 22].

### 10.1.2 Transitional Care Model (TCM)

The Transitional Care Model, developed by Dr. Mary Naylor at the University of Pennsylvania School of Nursing in 1981, is an interdisciplinary model of care delivered by advanced practice nurses. The master's prepared nurse practitioner (NP), a type of advanced practice nurse, provides primary care and is responsible in the clinical management of the patient's medical problems.

The key attributes to this model include increasing access to health care, leveraging long-term relationships, and facilitating comprehensive and coordinated care. The nurse practitioner promotes key principles of patient-centered care, care integration, coordination, and improved access [23].

Transitional Care Model (TCM) generally refers to the care and services that promote the safe and timely transfer of patients from one level of care to another and from one type of setting to another [24]. Its original intention was to enable earlier discharge of vulnerable patients by substituting a portion of their hospital care to transitional home follow-up. Unique to this program is the initiation of services in the hospital by the same advanced practice nurse provider who coordinates and visits the patient in their home.

This model was further expanded by Neff et al. [25] by using advanced practice nurses to direct the program and the home health care registered nurses and licensed practical nurses to provide the nursing services.

### 10.1.3 Principles for Transition between Care Settings

The American College of Physicians (ACP), Society of Hospital Medicine (SHM), Society of General Internal Medicine (SGIM), American Geriatric Society (AGS), American College of Emergency Physicians (ACEP) and the Society for Academic Emergency Medicine (SAEM) developed consensus standards to address the quality gaps in the transitions between inpatient and outpatient settings [26]. The following principles were established:

- Accountability
- Clear and direct communication of treatment plans and follow-up expectations
- Timely feedback and feed forward of information
- Involvement of the patient and family member in all steps when inappropriate
- Respecting the hub of coordination of care
- Identification of medical home or coordinating clinician/provider by all patients and their family/caregivers
- Necessity of knowing who is responsible for their care and who to contact and how by patient and/or their family/caregivers every point along the transition
- Adoption and implementation of national standards for transitions in care at the national and community levels through public health institutions, national accreditation bodies, medical societies, medical institutions, etc., in order to improve patient outcomes and patient safety
- Monitoring and improving transitions with standardized metrics for continuous quality improvement and accountability

Based on these principles, standards describing necessary components for implementation were developed, namely: coordinating clinicians, care plans/transition record, communication infrastructure, standard communication formats, transition responsibility, timeliness, community standards, and measurement [26].

### 10.1.4 Transitioning from the Emergency Department

The emergency department represents a unique subset of transitions of care [26]. The potential transition can generally be described as outpatient to outpatient or outpatient to inpatient depending on whether or not the patient is admitted to the

hospital. The outpatient-to-outpatient transition can also encompass a number of potential variations.

Patients with a medical home may be referred in to the emergency department by the medical home, or they may self-refer themselves. A significant number of patients who do not have a physician refer themselves to the emergency department. The disposition from the emergency department, either outpatient to outpatient or outpatient to inpatient is similarly represented by a number of variables.

Discharged patients may have a medical home, may need a specialist and may require urgent (<24 h) follow-up. Admitted patients may have a medical home and may require specialty care. This variety of variables rules out a single approach to emergency department transitions of care coordination. This intensely hectic setting may distract the patient's attention from instruction and education.

A recent study at the Georgetown University Hospital [27] suggested that more than 75% of patients did not understand important aspects of their medical condition when they leave the emergency department. Nearly one-third of patients failed to understand what to do at home after discharge from the hospital and virtually a quarter did not know the symptoms that should prompt a return to the emergency department.

Perhaps most striking, the same study found that 80% of patients, who did not understand aspects of their care and the instructions about what to do next, believed they understood everything [27]. In the case of heart failure, lack of compliance with medications, failure to follow a salt-restricted diet, and delays in seeking medical attention are among the primary reasons for the high rate of rehospitalization [28].

## 10.2 Preventing Hospital Readmission

### 10.2.1 How Readmission Happens?

Most patients do not want to return to the hospital shortly after discharge. But for a good number of patients, they find themselves readmitted back to the hospital within 30 days of discharge. Some readmissions are planned, others as expected course of treatment for specific illness. Increasingly, however, more avoidable readmissions are thought of as "indicators of poor care or missed opportunities for better coordinated care" [29].

These hospitalizations are expensive, accounting for 31% of the gross health care expenditures [30]. Inpatient care accounts for 37% of Medicare expenditure [29] and these includes readmissions of which 18% occur within 30 days of discharge amounting to $17.4 billion [31].

Poor transitions between different providers and care settings were identified as one of the multiple factors that contribute to these avoidable hospital readmissions. Others include: poor quality of care, premature discharge, poor education, inadequate patient information, and patients' inability to process and utilize discharge information.

A lack of system factors, such as coordinated care, seamless communication, and information exchange between inpatient and outpatient or community-based providers may also lead to unnecessary and unplanned readmissions. Reducing avoidable hospital readmissions presents a unique opportunity for policymakers, payers, and providers to reduce health care costs while increasing quality of patient care.

The passing of the Health Reform Act forced hospitals to keep readmissions rates down otherwise be penalized. The House of Representatives' Health-Overhaul bill called for cutting Medicare reimbursement to hospitals with high readmissions rates. A recent Medicare payment proposal states that hospitals with high readmission rates will have 20% of the original admission payment withheld if the patient is readmitted within 7 days, and 10% withheld if patient is readmitted in 15 days [32].

President Obama's proposed 2010 budget blueprint also cited penalizing hospitals with the highest quartile of readmissions as an $8.4 billion cost-saver over 10 years, a measure that could be used to finance Obama's plan to establish a $634 billion health reserve fund.

The chairman of the Senate Finance Committee also called for similar penalties, although they recently announced support in rewarding hospitals rather than punishing them, for reducing their readmission rates [33]. Hospitals are now under more pressure, especially with Medicare calling for published 30-day readmission rates for heart attack, pneumonia, and heart failure from every hospital in the country.

Heart failure, the leading cause for rehospitalizations, affects about 5.8 million people in the United States, with 550,000 new patients diagnosed each year [34]. Usually patients with heart failure also have a high incidence of comorbidities such as chronic obstructive pulmonary disease, dementia, diabetes mellitus, renal failure, and hypertension. Over one million is admitted to hospitals each year and 27% of Medicare patients are readmitted within 30 days [35].

Pneumonia is among the top 10 causes of death in the United States and is a significant cause of outpatient visits and hospitalizations, about 1.1 million US hospitalizations are due to pneumonia [36]. Coronary heart disease caused 425,425 deaths in 2006 and is the single leading cause of death in America today. 17,600,000 people alive today have a history of heart attack, angina pectoris (chest pain), or both. This is about 9,200,000 males and 8,400,000 females.

This year an estimated 1.26 million Americans will have a new or recurrent heart attack. There are about 295,000 EMS-assessed (Emergency Medical Services) out-of-hospital cardiac arrests annually in the United States [37]. The national average for readmissions for all reporting hospitals in the US is 24.7% for heart failure, 18.3% for pneumonia, and 19.9% for heart attack [38].

This economic challenge is being faced head on by health care systems, hospitals, and agencies through varied, unique, and innovative approaches in discharge planning, medication reconciliation, transitional care, and home health care [21, 39, 40]. Project RED (re-engineered discharge) at the University of Boston utilized a modified discharge planning approach using nurse discharge advocates, after-hospital care plan (AHCP) tool, and follow-up telephone call by pharmacist, showed decreased hospital utilization within 30 days of discharge by about 30% among patients in the general medicine service [41].

A unique and a more proactive approach to addressing the spiraling health care cost by decreasing emergency department and hospital utilization that can be adapted in transitional care was conceptualized by Dr. Gawande through identifying "hot spots," – areas in the community having the most number of people with the most number of hospital admissions and emergency department utilization, and improving patient care at these sites.

Improved patient care and implementation of preventative care for these "high utilizers" resulted in significant impact to their lives and remarkable decline in health care costs [42]. A systematic review of research literature undertaken by Naylor et al. [43] summarized 21 randomized clinical trials of transitional care interventions targeting chronically ill adults.

They identified nine interventions that exhibited positive outcomes on total all-cause readmissions, time to first readmission, or length-of-readmission stay. With the Affordable Health Act of 2010 serving as a stimulus, transitional care has been identified as one of the quick means to achieving one of the foci of health reform – reduction of preventable hospital readmissions.

## 10.3 The Essentials of Transitional Model of Care

### 10.3.1 Communication

Several indispensable factors are necessary for the success of a transitional model of care program: communication, teamwork, home visits, and information technology and medication reconciliation. The finer points are discussed in this section.

Effective open communication is a key to the success of any endeavor.

Changes in physical location of care and handoff communication between caregivers create the potential for error and loss of information. Communicating plans to patient and caregivers is extremely essential in the preparation for the transitions. Instructions are provided on medication reconciliation, symptoms and signs of worsening condition, and an explicit follow-up plan for tests and appointments improve the patient's chances for success [15].

Printable health and discharge instructions, medication lists, clinic follow-up, and specialist appointments increase patient adherence and prevent instruction errors. Instructions in their respective language that patients and caregivers can review once they are back home, alone, with their family members, visiting nurse, and home health aide or home attendant. Regardless of the mode – electronic, telephone, face-to-face, or written – communication is the key to transitional care.

### 10.3.2 Teamwork for Success

No healthcare organization today can be complacent about clinical, operational, or financial performance. The stakes are just too high. Providing care in the different points of patient's transitions involves individuals, teams, internal and external

groups, or organizations, at different levels that sustaining an effective and open communication becomes a challenge.

The "right" culture nurtures patient-focused care, organized and multidisciplinary teamwork, formal and informal learning, and effective communication at and among all levels [44].

Ongoing consultation with the patient care team and reassessment of the patient's changing medical condition, social, and cognitive capabilities, assures that the comprehensive needs of the patient are addressed. Patients and families are apprised of the appropriate community resources available and encouraged to participate in all phases of the transitional care planning process.

Referral mechanisms with community providers occur in a timely, systematic fashion in order for the patient to gain access to identified resources. The process concludes with the coordination and implementation of services and transition to the least restrictive level of care in keeping with the individual's wishes.

Inpatient hospital discharge is a complex process involving a team of health providers such as physicians, nurses, dietitians, physical therapists, social workers, and care coordinators. Patients or family members may be experiencing physical and emotional discomfort. They may be eager to leave and thus, less interested in the instructions.

Moreover, a significant number of patients have low literacy and/or health literacy levels. Education of patients at discharge promotes self-care, reduces readmissions, and helps patients identify problems early, increasing the chances for intervention and improved outcomes [45].

### 10.3.3 Home Visit Programs

Home visits are essential to the success of transitional care. A systematic review of nurse home visit programs for the elderly undertaken by Marek and Baker [46] had shown that although there are several variables that influence the effectiveness of home visit programs, programs structured to be condition-specific appear to be far more effective than those designed to treat more general older populations.

Four home visit program processes were identified: hospital-to-home, early hospital discharge, disease-specific protocols and transitional care. Hospital-to-home program is referred to as the "substitution of care" phenomenon, where care is provided by the most appropriate professional at the lowest cost level [47]. This generally utilizes multidisciplinary teams, and although nurses deliver the majority of home visits, physician visits are common. [48–53].

These programs also utilized intense home care services in lieu of acute care hospitalization. In 2004, Tibaldi et al. [53] found that the group that received home based services had less agitation and had better outcomes compared to the hospitalized subjects. Early hospital discharge is where part of the hospital care is substituted by nurse home visits instead of staying the full course of hospitalization.

A common characteristic is a visit in the acute care setting by a nurse to begin discharge planning and follow up by the same nurse at home once discharged

from the hospital [54–56]. Disease-specific protocols address specific clinical conditions such as chronic obstructive pulmonary disease and heart failure as the most common foci for home visits. Marek and Baker [46] listed these protocols as:

- Medication management (i.e., medication for congestive heart failure management, monitoring for medication compliance)
- Counseling (i.e., related to mental health, and specific clinical conditions such as depression, myocardial infarction, and post breast cancer)
- Telehealth (i.e., use of technology for monitoring vital signs, weights)
- Rehabilitation (i.e., chronic obstructive pulmonary disease program that consists of education, inspiratory muscle training, exercise training and relaxation exercises)
- Exercise (similar to rehabilitation programs and including exercise tolerance activities beyond basic activities of daily living)
- Diet (usually instructions on disease-specific protocols for congestive heart failure, hypertension, diabetes, and adherence to recommendations)
- Self care (i.e., interventions related to promoting and supporting activities of daily living in disease-specific conditions)
- Preventive monitoring, with the goal for early identification of problems, referrals, or provision of timely and appropriate care

### 10.3.4 Electronic Technology in Transitions of Care

Health IT (information technology) was one of the important system changes necessary to improve quality in health services as determined by the Commission for Health in its June 2003 report submitted to Congress [57].

This determination was supported by the Institute of Medicine as one of four critical forces to improve health care quality and safety [58]. The Institute Of Medicine had released a report in 2000 estimating that 44,000–98,000 people die in the United States hospitals yearly due to medical errors.

Many of these errors involve medications. In response to these reports, the Leapfrog Group, a group of large employers committed to patient safety improvements, made hospitals adopt CPOE – computerized provider order entry [59]. In their research, Bates et al. [60] had determined the serious medication errors were reduced by 55% using the computerized provider order entry.

At least one study showed improved quality and safety by reducing medication errors, including adverse drug events, decreasing dosage errors, prescribing certain medications more precisely, or prescribing with improved accuracy [61].

Electronic technology does not only benefit the health providers and ancillary services that have access to the data, but also, patients and their designated health representatives. An online survey of 4,282 members of the Geisinger Health System registered users of an application (MyChart) was undertaken.

The application allowed patients to communicate electronically with their providers and view selected portions of their electronic health record (EHR). The results had shown an overwhelmingly positive attitude with the experience and

overall satisfaction. It also showed that confidentiality and privacy of their information were a mild concern [62].

### 10.3.5 Medication Reconciliation

The Joint Commission requires accredited facilities to "accurately and completely reconcile medications across the continuum of care," to facilitate safer and more effective transition [63]. To stress the need for this, a study undertaken at a large urban academic medical center showed nearly 49% of hospitalized patients experience at least one medical error in medication continuity, diagnostic work-up, or test follow-up following hospital discharge [17].

About 19–23% of patients suffered adverse events, most common result from adverse drug events [40]. Forster et al. found that of most adverse events during hospitalization and after discharge, a significant number were preventable and ameliorable [16, 64, 65]. Most of these errors or adverse drug events resulted from breakdown in communication between the hospital team and the patient and primary care provider [64].

To compound this problem, a study found that on average, patients omitted 6.8 hospital medications; 44% of patients believed they were receiving at least one hospital medication that was not actually prescribed; patients <65 years old omitted 60% of their "as needed" medications, whereas patients >or=65 years old omitted 88% ($p=.01$); only 28% reported having seen their hospital medication list; and, 81% reported receiving their discharge medication list would improve their satisfaction with hospital care [66].

A very important component of transitional care is medication reconciliation. Wong et al. [67] expounded that medication reconciliation at hospital admission and at time of discharge are two very distinct processes. Admission medication reconciliation requires a straightforward comparison of a comprehensive list of a patient's preadmission medications with admission orders.

Discharge medication reconciliation requires multiple comparisons between different pieces of information, including medications on the best possible medication history, medications prescribed in the hospital (adjusted, new, discontinued), unchanged home medications, and medications to be started at discharge, which makes this process complex.

The process of discharge reconciliation on a general medicine service is especially critical given the complex needs of the patients involved (e.g., multiple comorbidities and medications). Patients are at a high risk of medication discrepancies as they transition from hospital to home.

These discrepancies are important, as they may contribute to drug-related problems, medication errors, and adverse drug events that can bring the patient back to the emergency department. Wong's study supported the need for the multidisciplinary practice of discharge medication reconciliation, and a transitional care program to follow up can significantly decrease potential return to the emergency department.

## 10.4 Supporting Evidence

A frequently referenced transitional care intervention delivered by advanced practice nurses was implemented to elders hospitalized with heart failure. This randomized, controlled trial with follow-up through 52 weeks post-index hospital discharge was undertaken in six Philadelphia academic and community hospitals with 239 eligible patients aged 65 and older [18].

The 3-month advanced practice nurse-directed discharge planning and home follow-up protocol demonstrated improved clinical and economic outcomes in the intervention group ($n=118$) over the control ($n=121$) reflected by fewer hospital days (588 vs 970, $p=.071$), and decreased readmission (104 vs 162, $p=.047$), deaths at 52 weeks (56 vs 74, adjusted $p=.01$), and lowered healthcare costs ($7,636 vs $12,481, $p=.002$).

Patient satisfaction assessed at 2 and 6 weeks was found to be significant ($p<.001$) with quality of life (QOL) improved at 12 weeks ($p<.05$). The process involved a comprehensive patient assessment using valid and reliable instruments that addressed the following:

- Patients' and caregivers' goals
- Nature, duration, and severity of heart failure and comorbid conditions
- Physical, cognitive, and emotional health status
- General health behaviors and skills
- Availability and adequacy of social support

A major focus of advanced practice nurses' intervention during the hospitalization phase was collaboration with physicians and other providers to optimize the patient's health status at discharge, design the safe discharge plan, and arrange for needed home care services. Special emphasis was placed on preventing functional decline and streamlining medication regimens.

Advanced practice nurses were able to provide input to the nursing staff regarding the discharge needs of patients and caregivers, thus maximizing the time staff nurses devoted to these areas. They worked with discharge planners to prevent duplication of post discharge services and coordinate the ordering of essential medical supplies. After patients were discharged to their homes, advanced practice nurses conducted targeted assessments to identify changes in patients' health status.

Their involvement throughout the transition from hospital to home provided a safety net designed to prevent medication and other medical errors and assure accurate transfer of information. Face-to-face interactions with the patient's physician during the hospitalization and initial follow-up visit (aimed at promoting continuity of care) helped to foster collaborative relationships.

Their expertise in management of heart failure and common comorbid conditions, coupled with their ability to coordinate care, nurtured these relationships and provided patients with increased access to symptom management tools. At the conclusion of the intervention, advanced practice nurses provided patients, caregivers, physicians, and other providers with summaries of goal progression, unresolved issues, and recommendations.

Another study examined the effectiveness of transitional home care delivery by an advanced practice nurse who directed and supervised a pulmonary disease

management team caring for patients with chronic obstructive pulmonary disease compared with a control group of patients who received routine home care [25]. The study was a prospective quasi-intervention involving one intervention group ($n=41$) cared for by advanced practice nurse-directed and -supervised pulmonary disease management team, and a control group ($n=39$) cared for by nurses providing routine home care.

The study was conducted at a large multidisciplinary agency that served Medicare patients in four counties in Ohio. The advanced practice nurse-directed pulmonary disease management team served patients in the metropolitan area while usual home care teams served patients in the rural and outlying areas. Patients with chronic obstructive pulmonary disease in the intervention group received services from pulmonary care registered nurse/licensed practical nurse who were supervised and directed by a cardiopulmonary care advanced practice nurse specialist.

Care for these patients included home visits, telephone contacts, and a nurse specialist available by phone 24 h a day. The advanced practice nurse supervised the registered nurse/licensed practical nurse care and acted as an education resource for the nurses at the agency office. The advanced practice nurse also provided clinical consultation for patients identified as high risk and, as needed, made home visits for teaching, assessment of complex care needs, and to provide assistance with patient/family issues.

They found that the intervention group had shorter length of stay (24.4 vs 32.2 days, $t=2.0$, $p<.05$), decreased rehospitalization (4 vs 11, $x^2=4.471$, $p<.05$) and acute care visits (5 vs 16, $x^2=5.61$, $p<.05$); and patients had stayed home longer (34 vs 20, $x^2=9.07$, $p<.05$) prior to next hospitalization. This study pointed to the effectiveness of the advanced practice nurse-directed and nurse-supervised transition of care model.

A randomized clinical trial to determine the safety, efficacy, and cost savings of early hospital discharge infants with low birth weight (less than or equal to 1,500 g) was undertaken [68]. Infants in the control group ($n=40$) were discharged according to routine nursery criteria, which included a weight of about 2,200 g, and those in the early-discharge group ($n=39$) were discharged before they reached this weight if they met a standard set of conditions.

Instruction, counseling, home visits, and daily on-call availability of a hospital-based nurse specialist for 18 months were provided for families of infants in the early-discharge group. Infants in the early-discharge group were discharged a mean of 11 days earlier and were 2 weeks younger at discharge than control infants.

The study showed that the intervention was cost-effective ($47,520 vs $64,940, $p<.01$), with the mean physician's charge being 22% less ($5,933 vs $7,649, $p<.01$). Although the two groups did not differ in the numbers of rehospitalizations and acute care visits, or in measures of physical and mental growth, but overall safe and economically sound.

Transitional care model has been adapted in multiple clinical trials and studies in vulnerable populations and has shown significant outcomes in hospital length or stay, readmission rates, emergency department utilization, patient and provider satisfaction, and health care costs. These include:

- Patients after myocardial infarction [69]
- Patients with progressive lung cancer [70]

- Women who have undergone unplanned caesarian section [71]
- Elders with cardiac medical and surgical diagnosis related groups [22]
- Women with high-risk pregnancies [72]
- Elders with common diagnosis related groups at risk for poor outcomes following hospital discharge [21]
- Women after abdominal hysterectomies [73]
- An international study on the follow-up of psychiatric patients returning to the community from the psychiatric wards [74]
- Chronically ill homebound patients [75]

Although there were several successful studies on transitional care as a model of health care delivery, some studies on its utilization did not produce positive outcomes. Several factors contributed to failure in transitional care studies.

These studies did not produce significant differences in outcomes in terms of clinical health status at a designated point in time, use of health care services, cost/expenditures on health care services, and client satisfaction or perception of quality of health care delivered.

Two Canadian older adult visit programs were unsuccessful in identifying differences in outcomes. Authors of both studies identified insufficient statistical power as a limitation [76, 77]. Lack of power or authority to control use of other services was also identified as a limitation to these programs.

Low intervention dose is another possible reason for insignificant finding as demonstrated by one study that consisted of one nurse visit with follow-up phone calls [78]. It was also suggested that the nurses' lack of authority in the system was one reason for the insignificant results in the study of nurse case management of the frail older adults. Difficulty in reaching the patient's primary care physician led to emergency department visit [77].

A 2-year nurse practitioner-led transitional care study at a large urban medical center in New York following the chronically ill homebound patients showed that although patient satisfaction and hospital reimbursement were greatly improved, there was no significant change in readmission rate or length of stay [75]. Another nurse practitioner-led transitional care program by an urban-skilled nursing service also showed no significant reduction in hospital readmissions [79].

The two studies showed that despite the increased number and frequency of follow-up, it was imperative to define a methodology that can uncover high-risk factors to rehospitalization such as self-management skills, social support, identifying gaps in the medical home, the need for partnerships with community health care agencies, and improved utilization of electronic technology for communication [75, 79].

## 10.5 Conclusion

Transitional care responds to the challenges that hospitals are experiencing in today's health environment namely the challenges in reducing hospital length of stay, decreasing readmissions rates, and improving patient satisfaction and safety. These challenges need a comprehensive, collaborative, and innovative approach.

There are essential components for its success: open communication, whether face-to-face, paper trail, or electronic technology; teamwork and collaboration; careful assessment and insightful planning; effective and efficient implementation; and organizational support.

The transitional care process starts from a feasible, clear-cut safe discharge plan, to earlier and more frequent follow-up visit which are integrated and cohesive from contributions by different health services including but not limited to the patient's primary care provider (i.e., physician or nurse practitioner), social work, pharmacy, physical therapy, skilled nursing and/or home care services, community agencies, etc.

The process of transition can happen within or between the different and challenging care settings such as at home, clinic, and hospital. Cumbler [19] had succinctly stated that transitions continue to be a difficult time for the most vulnerable patients. Intense efforts have improved outcomes in selected populations but have not been broadly applied.

Identification of patients at the highest risk would allow anticipation of difficult transitions and potential utilization of proven interventions, such as advanced practice nurses or follow-up pharmacy contact. Attention to all elements of effective transitions should become part of the growing culture of patient safety and health promotion.

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# Part IV

# Conclusion

# 11 Evidence-Based Standard of Care: Applications for Comparative Effectiveness and Efficacy Research and Analysis for Practice (CEERAP) in Developing Patient-Centered Optimal Treatment Interventions in Health Care for the Next Decade

Francesco Chiappelli and Sohrab Danaei

**Core Message**

The process of CEERAP pertains equally to evidence-based endodontics and evidence-based nursing and to all facets of health care. It is a hypothesis-driven process directed at answering the PICO question, utilizes as its sample of investigation the pertinent bibliome, evaluates it systematically in a research synthesis design by means of validated measures to assess the level and quality of the evidence, and analyzes the findings by means of stringent statistical analyses for acceptability of the reports and for consensus of the best evidence. Evidence-based research requires regular updating as the research literature constantly grows and secures at any one time the best and the latest available research evidence.

This chapter elaborates substantially on ideas first presented in The Nugget [Sacramento, CA, District Dental Society newsletter (October, 2011, pp. 8 & 9; Is there a place for evidence-based dentistry [EBD] in the standard of care, by F. Chiappelli)

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## 11.1 Introduction

As we come at the end of this endeavor, it might be useful to ponder for a minute on the concept of "Standard of Care" – what do we mean by it?

Perhaps a simple all-encompassing definition could sound something like this: Standard of care refers to the specific and timely treatment intervention that is appropriate for a given patient – taking into consideration gender, ethnicity, medical and dental history, and insurance coverage (or socioeconomic level, if a private patient) – for a given condition.

The term "standard of care" is legally defined in the United States and is the basis of many torte proceedings in the courts. It is also taught in the ethics and jurisprudence courses given to physicians, nurses, and dentists.

The specificity of the standard of care rests not only on the patient's profile but on the specifics of the clinical condition we have duly diagnosed. The timeliness of standard of care depends largely upon and is driven by our awareness of the current scientific evidence. The decision-making process in which we engage to determine which, among a choice of possible alternatives, is the optimal intervention we ought to entertain for the patient is often guided by our collaborations, referrals, and consultations with professional colleagues, whom we trust for their expertise and we know involved in, and abreast of the latest novelties in the treatment of this or similar conditions. In short, we could say that standard of care ideally involves a process by which we engage in clinical decision-making about the optimal treatment for a given patient, based on the research evidence, and all other necessary inputs and factors so that we provide the best possible treatment to the patient.

The key here is, for health professionals in the twenty-first century, our reliance on the research evidence. So then, we might ask, what is the big deal about "evidence-based health care" and CEERAP – comparative efficacy and effectiveness research for practice?

Well, and as an example, let us see how the American Dental Association defined evidence-based dentistry (EBD) in 1999. The ADA Center for EBD (ebd.ada.org) states that EBD is "…*an approach to oral healthcare that requires the judicious integration of systematic assessments of (best available) clinically relevant scientific evidence, relating to the patient's oral and medical condition and history, with the dentist's clinical expertise and the patient's treatment needs and preferences… an approach to making clinical decisions, and is just one component used to arrive at the best treatment decision*…."

So, there you have it: The fundamental distinction between the dentistry based on the evidence that is the customary standard of care and evidence-based dentistry (EBD) is the fact that EBD relies not just on a perusal of the research evidence but specifically, stringently, and systematically on the best available evidence. That is the core of EBD: its reliance on the systematic assessment of the best available evidence for clinical decision-making. And the same can be said of evidence-based nursing.

We then could conceptualize evidence-based health care in general in two parts: In part one, we will work to obtain and report the systematic assessment of the best

available evidence; in part two, we must disseminate this information to empower the dentist or the nurse to utilize it, if deemed necessary for the clinical case at hand, in the clinical decision-making process for treatment. Part one of the process is purely a scientific endeavor.

Part two pertains to the exquisite art of clinical care, with which the reader is fully cognizant. Let us, therefore, focus primarily on what part one entails.

Evidence-based research in health care begins with the patient–dentist encounter, whence emerges the clinical question under scrutiny. The question is stated following systematic rules, such as to address issues that pertain to the patient specifically (P), the treatment interventions (I) we are considering (C), and the clinical outcome sought (O). This PICO questions then guides the research in the pursuit of all of the available research evidence, by means of hands searches in libraries, as well as multiple search engines often including the National Library or Medicine, the Cochrane group, other search engine sites, and so forth. The results of the search can be gargantuan, as one can easily imagine considering the rate of growth of the clinical research literature, and must undergo a systematic process of scrutiny with inclusion and exclusion criteria that help focus the obtained sample of literature, which at this stage, takes on the name of "bibliome," to the patient characteristics, the clinical condition, the nature of the interventions, and the desired outcomes. Next, the bibliome is subjected to a systematic process of evaluation for the "level" of the evidence (i.e., what was done) and the "quality" of the evidence (i.e., how it was done). Fully validated instruments help quantify these outcomes, and every report in the bibliome obtains a numerical score, if you will, of the level and quality of the evidence it presents. The scores undergo acceptable sampling analysis, which yields "the best available" evidence. A consensus across the bibliome, obtained qualitatively and quantitatively, informs evidence-based clinical decision-making for optimizing treatment efficacy and effectiveness [1] (Fig. 11.1).

Evidence-based research is disseminated through "systematic review (SR)," for the systematic nature of the process, and the comprehensive nature of the included bibliome. When several SRs are produced and must be pooled, validated instruments (e.g., R-AMSTAR [2], GRADE [3], PRISMA [4]) serve to evaluate the quality of each so that the acceptable sampling process may be applied [5] here as well. Pooled SRs in this manner yield "clinically relevant complex systematic reviews (CRCSRs)." Both SRs and CRCSRs are rather complicated, research jargon-laden, lengthy documents, which may be cumbersome to read. They are not the ideal mode of dissemination of the best available evidence. The field presents critical summaries (evidence reviews) of SRs and CRCSRs. These short reports are informative tools for the dentist and – since they are most often written in lay language – also empower the patients by raising health literacy.

Since all of these materials are produced and reported in paperless format, they are easily integrated in a health information technology protocol of paperless clinical charts and reports. In brief, our traditional view of the standard of care is intimately intertwined with the fundamental tenets of evidence-based health care (Fig. 11.1).

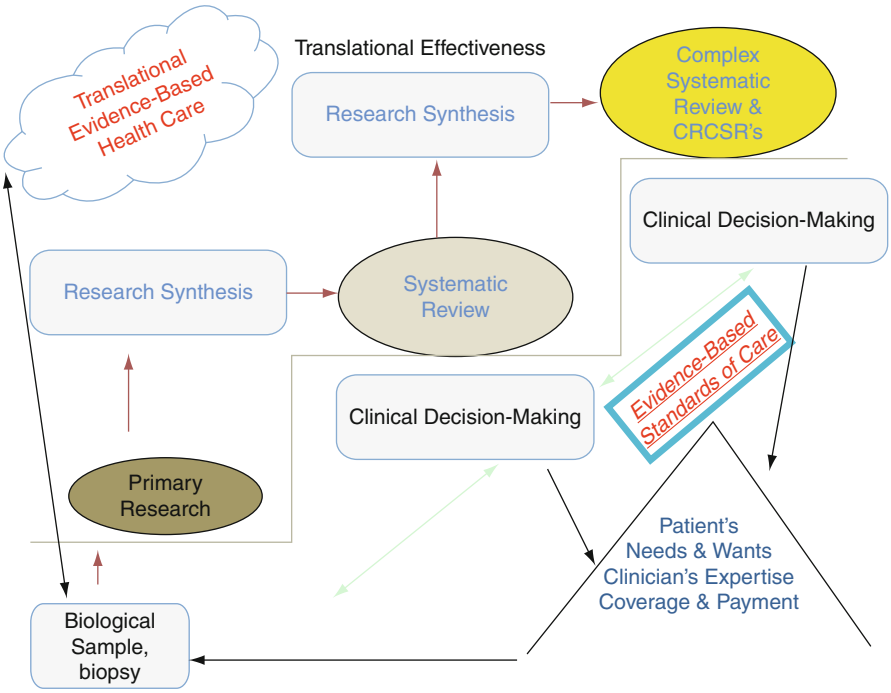


**Fig. 11.1** Evidence-based standards of care and translational effectiveness in CERRAP. The figure conceptualizes the progression of CEERAP from primary research to systematic reviews and onward to complex systematic reviews and clinically relevant complex systematic reviews. Each step involves a higher complexity and stringency of research synthesis. As the patient-clinician encounter establishes the fundamentals of translational research from does clinical setting to biopsy and biological samples of the patient back to the clinical environment, the process of research synthesis, whence emerges the best available evidence of efficacy and effectiveness, also requires "translation" into specific clinical settings (i.e., translational effectiveness). Evidence-based decision-making hence depends and derives from the best available evidence, and responds to the patient's needs and wants, in perfect accord to the clinician's expertise and the payment modalities (e.g., insurance coverage). It is at that stage that standards of care are revised based on the best available evidence and hence become "evidence-based." In the modality summarized in this figure, it becomes evident that evidence-based decision-making, which arises from CEERAP, is intertwined with evidence-based standards of care. This triad – CEERAP<–>evidence-based decision-making<–>evidence-based standards of care – signify and define the overarching concept of translational effectiveness, which has permeated through the various chapters of this compilation of writing, from evidence-based endodontics to evidence-based nursing

Possibly best stated, the following relationship between standard of care and evidence-based health care boils down to the fact that evidence-based health care consists of three important domains:

- The best available scientific evidence
- The dentist's clinical skill and judgment
- The individual patient's needs and preferences

When these domains are given due consideration in individual patient care, then evidence-based health care closely espouses the standard of care because, as the chapters in this book have attempted to demonstrate, evidence-based health care is designed to provide personalized care based on the most current scientific knowledge.

## 11.2 Implications of CEERAP in Endodontics and Nursing: This Book in Perspective

In Part I of the book, it was mentioned by Maida that a community of practice is comprised of individuals who share a common concern for a specific domain of knowledge. This chapter focused on building communities of practice among clinicians and scientists engaged in comparative effectiveness research, an emerging field requiring collaborative partnerships among researchers, practitioners, and consumers for greater transparency in planning and implementing a broad-based and inclusive research agenda. Further, this concept was clarified by Bauer, who argued that a community of practice is comprised of individuals who shared a common concern for a specific domain of knowledge. The chapter discussed building communities of practice among clinicians and scientists engaged in comparative effectiveness research, an emerging field requiring collaborative partnerships among researchers, practitioners, and consumers for greater transparency in planning and implementing a broad-based and inclusive research agenda.

In Part II of this compilation, we discussed the procedural issues associated with the performance, analysis, and interpretation of complex systematic reviews, specifically in the context of clinical relevance. Complex systematic reviews are instruments that derived from the combinatorial process of several homogeneous systematic reviews and, in that respect, pertain to a higher level of research synthesis. These concepts were further developed by Mata and Carneiro, who discussed the importance for the dentist to take the best decision for the patient and the relevance of this decision in the context of evidence-based dentistry. Specifically in the context of endodontic therapy vs. dental implant therapy, Faggion and collaborators argued the important considerations when deciding between two potential clinical treatment alternatives – endodontic treatment of a compromised tooth or its extraction and replacement with a dental implant, a process that must rest upon a stringent appraisal of the quality of available clinical evidence of the long-term success of dental implants compared with endodontic treatment. Taken together the chapters of this section of the book converged to presenting the relevance of comparative effectiveness and efficacy research for the practice of endodontics.

Part III of this work focused on evidence-based nursing; Begonia and Jose examined CEERAP in the context of treatment interventions for asthma and pressure ulcers, two chronic illnesses affecting millions of people, and especially the aging and aged population. Effective management and treatment is improved and ensured prevention, symptom control, and patient/family/staff education in order to improve health care outcomes.

The last two chapters in this section of the book conclude not only the part on evidence-based nursing but more generally are timely and critical to all domains of evidence-based health care. Sundel et al. discussed the patient-centered medical home (PCMH) as an innovative evidence-based model of care delivery that is focused on the core concepts of primary care which include easy access, comprehensive and coordinated care, and the development of relationships over time. Lopez-Cantor examined about transitional care, a novel evidence-based care delivery system where services are provided collaboratively by the health-care team to patients as they move from one point of care setting to another. Transitional model of care and PCMH are innovative evidenced-based approaches to patient care that are geared toward providing services and coordinated best available care to patients in nursing, as well as in dentistry and other health care fields. They rely on the concerted and collaborative effort of all health-care team members [6].

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