Health span or life span: the role of patient reported outcomes in informing health policy

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Abstract

Objectives: Population ageing and the increasing burden of chronic conditions challenge traditional metrics of assessing the efficacy of health care interventions and as a consequence policy and planning. Using chronic heart failure (CHF) as an exemplar this manuscript seeks to describe the importance of patient reported outcomes to inform policy decisions.

Methods: The method of an integrative review has been used to identify patient reported outcomes (PROs) in assessing CHF outcomes. Using the Innovative Care for Chronic Conditions the case for developing a metric to incorporate PROs in policy planning, implementation and evaluation is made.

Results: In spite of the increasing use of PROs in assessing CHF outcomes, their incorporation in the policy domain is limited.

Conclusions: Effective policy and planning of health care services is dependent on the impact on the individual and their families. Epidemiological transitions and evolving treatment paradigms challenge traditional metrics of morbidity and mortality underscoring the importance of assessing PROs.
Introduction

Aging and the increasing burden of non-communicable diseases is influencing strategic policy initiatives in both developed and developing countries. [1] These factors also challenge clinicians and policy makers to consider health and social outcomes beyond traditional concepts of morbidity and mortality. Rapidly growing disciplines, such as health economics strive to balance parameters of demands, costs, and benefits relative to patient outcomes and treatment allocation. [2, 3] Clinicians and policy makers are more aware of the complex interplay of social, economic, physiological and policy factors in determining health outcomes. [4-6] The dilemmas confronting contemporary society underscore the need to increase the links between researchers and policy makers to develop, evaluate and implement appropriate interventions. [7] As well as assessing clinical outcomes, we also need to capture the unique perspective of the individual and their social determinants of health, to effectively inform health care planning. [8] This is of particular significance in chronic and aged care conditions where psychological and social issues play an important role in etiology and prognosis. [9, 10] Balancing treatment burden in the elderly is of concern and often gains in longevity are not matched by symptom relief and quality of life. [11] The health status of a population has traditionally been measured in terms of mortality and morbidity rates. Yet, with the epidemiologic transition from infectious to chronic diseases, quantifying health in terms of death and disease rates is seen to be increasingly inadequate. [12] Moreover, the ageing of the population means that a greater proportion of the population will receive treatment for chronic disease for a longer period of time. In chronic diseases, the goal of treatment commonly changes from cure to control of symptoms through targeted interventions.

Patient reported outcomes

The increasing complexity of treatment allocation, acceptability and utility makes the views of consumers more critical in intervention development, evaluation and health service planning. [13] One way to achieve this perspective is through assessing patient-reported outcomes (PROs). This
term refers to information and measures reported directly by the individual affected by a health condition, treatment or life experience. [14] Further, PROs is an umbrella term to capture the patient’s subjective perceptions of the broad spectrum of disease and treatment outcomes. Health-related quality of life (HRQoL) is one of several types of PROs. Others include subjective symptoms, functional status, psychological well being, treatment adherence, and satisfaction with treatment. For example, capturing information to bathe without assistance and participate in activities of daily living is important in determining the impact of an intervention. Further, if an individual is unable to either fill their medication prescription or open the medication container – pharmacotherapy is unlikely to be effective. Patient reported outcomes can be either generic or specific to a clinical condition or disease state. Often the term “PROs” has been used to refer to the concept being measured, the instrument used to measure that concept and the actual endpoint. There is a need to distinguish the concept and outcome one is attempting to measure and the endpoint for statistical analysis. [15] It is vital to have sufficient evidence that PRO concept is adequately measured by a PRO instrument. [16] In recent decades there has been an exponential growth in the measures and it is important to consider not only the psychometric properties but also the utility in making treatment decisions and policy development.

Despite benefits of a proposed treatment there is also the risk of intervention having deleterious effects on the individual’s quality of life and capacity to undertake activities of daily living. In such a case, the cure can be worse than the disease. Likewise, extended life can mean living for a prolonged period with a disability. [17] As complexity, burden and cost of treatment escalates, it is vital that patients and their families, clinicians, policy makers and funding bodies have a realistic expectation of outcomes, not merely in relation to the physical, but from a psychological and social dimension as well. [18] Gathering the unique perspective of patients and their families is paramount. These data will be crucial in informing policy makers need to plan and implement strategic initiatives.
Therefore it is increasingly an important consideration that the unique perspective of the patient be represented in not only individual clinical encounters, including patient assessment, but also in health policy, clinical trials and health service evaluation. [19]

The Innovative Care for Chronic Conditions (ICCC) framework (Figure 1) has been developed to help reorient health care systems to manage the demands of the rising burden of chronic conditions around the world. [35] At the centre of the framework is the healthcare triad (micro level of care); the partnership between patients and families, health care teams, and community supporters. To achieve optimal outcomes this triad needs to be supported by the broader community and the integrated health care organisations (meso level of care). This in turn needs to influence the broader positive policy framework (macro level of care) and to be influenced by them. It is contingent on every member of triad (patients and families, health care teams, and community supporters) being informed, and to maintain communication and collaboration. The ICCC framework emphasizes the importance of patients and families, forming one-third of the key ‘partnership triad’ at the most basic level. Furthermore, because management of chronic conditions requires lifestyle and daily behaviour changes, emphasis needs to be placed on the patient’s central role and responsibility in health care. When we refer to the patient, we consider family members and carers as part of this unit. Inclusion of this important dimension is contingent upon developing and testing of a model to measure the patient’s unique perspective.

In order to provide a more in depth discussion of the notion of PROs and how these can inform the metric that assists policy makers in developing and implementing health policy, we have chosen heart failure (HF) as an exemplar of a chronic condition. [20] Heart failure is a disabling and progressive condition and is the end stage of most heart disease. The unpredictability and severity of physical symptoms such as dyspnea, fatigue and pedal edema has led to great deal of anxiety and
fear in patients living with heart failure. [21]. Numerous studies have also shown that HF is associated with depression, and that this association is linked with a worse prognosis. [22] In studies with comparative normative data the degree of physical, mental and social functioning impairment was greater in heart failure patients than other chronic diseases sufferers. [23, 24] In fact, many patients with advanced heart failure ascribe greater importance to quality than to duration of life which may be limited by heart failure. [25] Furthermore, HF is the leading cause of hospitalisation in industrialised countries [26] with high re-admission rates [27] and prolonged length of stay which all lead to an increasing burden on resources both personally for patients, and financially for health care services. [28] In developed countries it accounts for 1% to 2% of all healthcare expenditure. [29].

Heart failure is primarily a condition of ageing. As treatment of hypertension, acute myocardial infarction and valvular disorders has met with increasing success, the incidence and prevalence of heart failure has increased dramatically. The prevalence of HF has been shown to increase from less than 1% in the 20-39 years to over 20% in 80 years and older. [30] In addition the incidence of heart failure doubles between 65-74 years and 75-84 age bands. [31] Increasingly, ethical and treatment conundrums arise out of the need to accurately assess the wishes of patients and their families and further tailor services to meet the needs of the vulnerable elderly. [32, 33]

A diagnosis of HF presents many of the challenges associated in caring for the elderly with a chronic condition from the perspective of the individual with the condition, their family and carers, as well as health professionals and the systems to support them. [34] Namely, it is a recurrent, costly and resource intensive chronic condition with an illness trajectory punctuated by episodes of decompensation and poor prognosis. [35] In spite of extensive evidence, there is evidence of a treatment gap that necessitates researchers, clinicians, administrators and policy makers to collaborate on strategies to achieve an evidence-based approach to health care. [36] Equally, we are
aware that some treatments may impact adversely on patients’ perception of quality of life in spite of improving more traditional endpoints such as mortality. It is important to remember that the definition of evidence-based health care relates not only to the best practice treatments, but the administration of these in accordance with the patient’s values and preferences. [37] Although substantive literature exists in discrete categories, such as quality of life and health service evaluation, there is considerably less experience in the integration and synthesis of this information to provide an outcome measurement model that takes into consideration clinical, organizational and patient factors. [38]

As discussed above, PROs in the context of health care have become an increasingly important focus of regulatory bodies and health care administrators. [18] The potential for interventions and treatments to be assessed from the perspective of the patient through validated psychometric measures is a critical issue for clinical practice, outcome evaluation and research. At a conference to assess the contribution of the Agency for Health Care Policy and Research (AHCPR) in enhancing outcomes, it was concluded that researchers and policy makers need to build upon descriptive studies and methodological advancements with the goal of measurably improving outcomes, quality, and efficiency of care. [39] Developing this science is dependent upon collaboration between consumers, academics and clinicians from a range of disciplines as well as policy makers and administrators.

Materials and Methods
An integrative review was undertaken to summarize how PROs have been defined, measured, and used in chronic heart failure research and identify their possible implications for policy initiative. We searched the electronic databases CINAHL, Medline, Embase and the Internet were searched using key words including ‘heart failure’, ‘instruments’, ‘psychometric instruments ’ and ‘patient reported outcomes.’ Furthermore the reference lists of published materials were hand searched for
additional data sources. The aim of the review was to explore PRO measures in CHF that may provide new insight in policy decisions. A range of measures contributing to the impact of the outcomes of CHF, such as medication adherence and self-management were explored. Inclusion criteria were those papers that explored PROs measures that would provide new dimension in outcomes of CHF. Exclusion criteria were papers not published in English. Abstracts were appraised that most fitted the aims of the review and met the inclusion criteria.

Examples of commonly used PROs are provided to illustrate the importance of including these issues in policy decisions. Table I provides examples of these constructs that assess the impact of CHF on an individual, ranging from limiting activities of daily living through to existential distress. Although this list is not exhaustive it provides insight into the range of measures available. Despite many potential uses of PRO measures in CHF, the primary area of application has been in randomised clinical trial investigation, particularly HRQOL. This is in line with the recognition that the changes in physiological measures may not always translate into a tangible benefits perceived by the patients. In closer inspection of these measures, outcomes important to patients are affected not only by symptoms and disease severity but also by a complex interaction of physical, social and psychological factors. By incorporating patients’ perspective they account for differences, subjective as well as objective among individual patients and to cater for patient’s preference. When the individual is unable to complete such measures, the use of proxies can be considered.

Importantly, PROs extend beyond traditional clinical efficacy and adverse effects and represent the patient’s perspective on the impact of disease and its treatment on daily functioning and wellbeing. [40] In many situations patient report is the sole source of data on frequency and severity of symptoms and also the side effects and the impact of treatment on functioning and well being. [41] Hence they are managed and monitored almost entirely on patient reports. Indeed in conditions where there are no physical or physiological markers of disease activity, PROs become the
outcome of choice for evaluating disease activity and in providing comprehensive understanding of severity of symptoms and their impact on daily functioning and well being. Palliative and supportive care is a striking example of such a strategy. [42]

However, it is not uncommon for there to be a mismatch between the patient’s perception and the clinician’s assessment. [38] For example, in some instances the patient’s perception of CHF and disease severity has also been overestimated when compared to physician’s clinical findings. [43] This incongruence may be due to the validity of tools used to assess patient perception or, an underestimation by clinicians of patient’s with HF. Therefore valid and reliable PROs can be an important communication tool. These measures provide a useful way to gather and communicate evidence about treatment risks and benefits. This information can be used to highlight particular treatment benefits or to provide a way to differentiate the patient benefits among competing treatments with similar clinical efficacy. [44] This will assist clinicians in providing patients with better information about potential effects of treatment, and thus lead to better treatment decisions. Data derived from PROs can also enable patients to increase their understanding about their illness and treatment risks and benefits. This is also a potentially useful strategy in increasing individuals’ participation in their own treatment and in health care decision making. Patient adherence is a major impediment to the effectiveness of therapies. Increased patient satisfaction with a treatment has been shown to be related to adherence. [9] Accordingly, evaluating satisfaction with treatment may assist health care providers in understanding the issues influencing treatment adherence and may help identify aspects of the management plan that require improvement to enhance long term treatment outcome. [45] The Innovative Care for Chronic Conditions (ICCC) framework (Figure 1) describes the importance of community and policy aspects of improving health care for chronic conditions. [46] This model highlights the importance of considering discrete yet linked attributes at the micro (patient and family), meso (health care organisation and community), and macro (policy) levels, underscoring the need for a multifaceted approach to health care outcome
assessment. To date, a comprehensive model for health service evaluation including all these critical elements has not been tested.

**Discussion**

Patient assessments are important elements of the evaluation of treatment impact, alongside other clinical indicators. Bioethics has emphasised the importance of the patient’s point of view in health care decisions through its call to respect patient autonomy. Outcome research has specified the importance of the patient’s perspective on the goal of medical care in its bid to accentuate patient-centred outcome such as quality of life. [38] It is recognised that linking patient-reported health with physiological markers of disease provide not just unique information in patient care, but also help to determine the severity of disease and monitor the trajectory of illness. [32] These factors are also important in informing cogent policy decisions.

It is hard to dispute that the science of PROs is advanced, as illustrated in the vast numbers of psychometric instruments available to assess these items. Perhaps what remains is the greatest challenge is moving assessment of these constructs beyond the research setting to routine clinical practice, administrative data sets and in contexts that will inform clinical and policy makers. The relevance of the applicability of clinical trial evidence to real world populations is commonly questioned. [47] Often participants in clinical trials are commonly younger, have less comorbid conditions and commonly do not have the challenges of poor health literacy and cognitive impairment that impact on outcomes of HF. [48] This conundrum is illustrated in the adverse events related to pharmacotherapy when agents move from the clinical trial to the usual care setting.

Registry data provides a useful insight into real world situations that can provide policy makers with reliable and valid data to inform policy decisions. A number of registries have provided useful data to inform HF management in the real world setting. [49-52] Many of these registries provide
useful data – particularly relating to how factors such as socioeconomic determinants, level of
insurance, and ethnicity impact on health related outcomes. [53] Data for these registries is often
collected from administrative data sets that do not routinely use patient-reported outcomes.
Including valid and reliable PROs in these data sets may be useful in health service planning

As shown in the Innovative Care for Chronic Conditions Framework in Figure 1, a Positive Policy
Framework is contingent upon understanding the needs of patients and their families. This can be
achieved through a range of means, such as community consultations, representations of
democratically-elected candidates and lobbying from particular consumer organisations. A
potentially more equitable, just, reliable and valid mechanism would be to include PROs in routine
clinical assessments, clinical trials and registries to allow an informed decision on how conditions,
treatment and health care interventions impact on the lives of individuals and their families. For
example, in Australia, the most rapidly increasing population are centenarians - many of whom will
endure and die of HF. Yet, we know little of their needs and service planning requirements. [54]
Further, the development of reliable and valid metrics that allow for the integration of micro, meso
and macro elements of health service delivery are needed. Health care policy, often constrained by
partisan politics and influence of powerful lobby groups, can struggle to keep pace with the
strategies needed to administer and monitor the increasing expense and complexity of healthcare.
[55] In HF, the development of innovative treatments, such as implantable cardiac defibrillators,
left-ventricular assist devices have outpaced the debate and discussion of the applicability and
relevance to particular groups. [56] [42] Despite benefits some patients may derive from these
medical interventions, the default plan of providing these devices or procedures regardless of
patient’s wishes and priorities need to be reconsidered by policy makers. Furthermore, their use
entails substantial financial, physiological, and psychological costs to patients, health care system
and community in general. Policymakers and clinicians alike need to allocate limited resources to
patients with HF to serve their interests and perspectives. Understanding the impact of these
interventions on individuals is likely to be critical in the future and require extensive debate and discussion.

**Conclusions**

Health care policy needs to be concerned with the financing of health care systems, access to and the outcomes of the quality of care. Contingent in this assessment is how health care services and treatments impact on the individual and their families. This article has used HF as an exemplar of a chronic condition that is costly, deadly and burdensome to individuals and communities. We need to consider the impact of CHF on the individual to inform health care policy. As the burden of chronic conditions grow and the population ages, we need to develop and refine the metrics of including the perspectives of patients on both an individual and population level to effectively evaluate the efficacy of health care intervention, treatment and planning. This in turn will lead policy makers to make decisions about service supply and health care spending that reflects the balance of extending life with improved quality. Health span or life span will become the issues that will be critical to address for both clinicians and policy makers alike.
References


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