REVIEW ARTICLE

# The Assessment of Patient Clinical Outcome: Advantages, Models, Features of an Ideal Model

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## Abstract

**Background:** The assessment of patient clinical outcome focuses on measuring various aspects of the health status of a patient who is under healthcare intervention. Patient clinical outcome assessment is a very significant process in the clinical field as it allows health care professionals to better understand the effectiveness of their health care programs and thus for enhancing the health care quality in general. It is thus vital that a high quality, informative review of current issues regarding the assessment of patient clinical outcome should be conducted. **Aims & Objectives:** 1) Summarizes the advantages of the assessment of patient clinical outcome; 2) reviews some of the existing patient clinical outcome assessment models namely: Simulation, Markov, Bayesian belief networks, Bayesian statistics and Conventional statistics, and Kaplan-Meier analysis models; and 3) demonstrates the desired features that should be fulfilled by a well-established ideal patient clinical outcome assessment model. **Material & Methods:** An integrative review of the literature has been performed using the Google Scholar to explore the field of patient clinical outcome assessment. **Conclusion:** This paper will directly support researchers, clinicians and health care professionals in their understanding of developments in the domain of the assessment of patient clinical outcome, thus enabling them to propose ideal assessment models.

## Keywords

Patient; Clinical Outcome; Assessment; Models.

## Introduction

In recent years, patient clinical outcome assessment in health care have been affirmed towards accurate judgment of the results of health care for patients who have undergoes under medical intervention. Patient clinical outcome will give health care professionals better understanding about the effectiveness of their health care programs and thus for improving the health care quality (1,2).

Choosing a scale or model is the most important factor in choosing any therapeutic procedure; the model should fit its intended purposes. Although there are a considerable number of patient clinical outcome assessments models, it is important for researchers and health care professionals to understand that those models must satisfy a set of requirements to be used in medical field or any research related to clinical field. These requirements covers all portions of the instrument from the development of the model, measuring its properties, and interpretability to clinical usefulness from the patient's point of view and the clinician's point of view (1,2). There are eight criteria for selecting a Patient-based Outcome model.

1. *Model development:* A health professional should determine whether a systematic process was used in developing model or not. The first anxiety regarding any patient-based outcome model is the procedure used for developing it. Development of patient-based model is complex; and involves multiple stages such as: model substances generation, initial substance reduction, field testing and final substance reduction and finally, establishment of the properties of the scale measurement (1,2).
2. *Suitability*: Suitability is how closely the model relates to a specific clinical situation (3). A health care professional should identify a particular purpose for the use of the scale, which should relate to the condition/disease or injury. A health care professional should determine whether the content of the model is appropriate for the clinical question, condition, or patient being treated, and whether the model is suitable for a variety of patients observed clinically (4).
3. *Reliability*: to which degree does the model measures a case or score, as dissenting to random error. A health care professional should determine whether the results produced from the model consistent and stable. Stability can be measured through administration of the model on two separate periods, during an interval in which condition of the patients is unchanged (4,5,6).
4. *Validity*: refers to the ability of the model to measure the scale or dimension that the model intended to measure (1).
5. *Responsiveness*: A model is responsive if it is able to detect any changes over time as they occurred (4,5). Thus, the model measures improvement or deterioration patient's condition once it happened. Responsiveness must not be discarded when selecting a model, because it might happen that a model is valid and reliable but not responsive. There are many ways to measure responsiveness such: change score, effect size (ES), standardized response mean (SRM), sensitivity and specificity to change, and receiver operating characteristics (6).
6. *Interpretability*: The obtained score derived from a model is understandable easily by health care professionals. One critique of patient-based outcome models is that their scores are not as interpretable as other clinical measures, such as heart rate and blood pressure (1,4). To increase interpretability of models, it is important to report the minimal detectable change (MDC) and the minimal important difference (MID).
7. *Acceptability*: means that is the model is acceptable by the patient, in other word can the model be completed in a short period of time and do its questions short, clear and easy to understand (4). Generally speaking, short questionnaires that are easy to read are accepted easily by the patients and provide a high response rate.
8. *Feasibility*: refers to how easy the model to administer as part of the routine care delivery process (4,5). How much training time needed clinicians to administer the model and how much effort and cost needed to administer the model (4,5).

In general, patient-based outcome assessment models are classified according to their field of use as either generic outcome models or specific outcome models; both have specific applications and limitations. Generic outcome models are more suitable for patient populations and injuries; they focused typically on Health-related quality of life (HRQoL) (7). On the other hand, specific outcome measures criteria or scale.

This paper can lead to better understanding of developments in the assessment of patient clinical outcome domain. This paper will support researchers, clinicians and health care professionals in their: understanding of the advantages of the assessment of patient clinical outcome; understanding of some of the existing patient clinical outcome assessment models; and knowing the desired features that should be fulfilled by a well-established ideal patient clinical outcome assessment model. Based on this study, researchers will be able to design ideal assessment models for patient clinical outcome that reserve the conceptual merits of existing models, avoid their limitations, and achieve the desired features of the existing models.

## Aims & Objectives

1. To Summarizes the advantages of the assessment of patient clinical outcome.
2. To Reviews some of the existing patient clinical outcome assessment models namely: Simulation, Markov, Bayesian belief network, Bayesian statistics and Conventional statistics, and Kaplan-Meier analysis models.

3. Describes the desired and ideal features that should be fulfilled by a well-established patient clinical outcome assessment model.

## Material & Methods

An integrative review of the published literature (studies from 2000 to the present) using the Google Scholar database, due to its broader data coverage, has been performed. The key words Patient, Clinical Outcome, Assessment of Clinical Outcome, Healthcare, Assessment Model, Advantages of Assessment and similar pre-identified terms were used in separate searches and in conjunction with each other to identify all related publications. Related publications including papers, books and reports were evaluated and selected if they met a criteria of presenting information that authors believed could be accredited to the purpose of this study. The collected literature has been summarized and divided into the following sub-sections:

### 1.1 Why the Assessment of Patient Clinical Outcome is Needed?

- Monitoring and comparing the health status of different populations at different times (3, 8), tracking and comparing the performance of health professionals and organizations over time (9), identifying the best choice among therapeutic alternatives, and comparing different medicinal products (10,11).
- Evaluation of health programs, health care interventions, different health conditions (9,12), and treatment efficacy in clinical trials (9,10) for health policy analysis, economic evaluation, and resource allocation (11).
- For better communication of benefits and safety information of therapeutic alternatives (10,11,13) and to facilitate the sharing of information among regulatory agencies (14).
- Prevent cognitive biases in making decisions. A conventional decision making is subjective, non-quantitative, sometimes inconsistent and lead to uncertainty in the effectiveness of the decisions (15). It is affected by cognitive biases (16). People will most often take decisions on intuitive or heuristic basis (17). This way of thinking prevent considering all options, outcomes, and probabilities at once (18,19). Different bias types are thoroughly discussed in the literature (19).
- Improve healthcare decision making (17, 20), increase transparency, consistency, and objectivity of regulatory decisions and recommendations (21), and protect patient's safety (22).
- Pharmaceutical development faces complex decisions that require tradeoffs between the desirable and undesirable effects of new therapies (23), and the existence of more standardized and quantifiable methods will encourage innovative drug development programs (24).

### 1.2 Assessment Models for Patient Outcome

There are a number of models, as shown in Table1, which have been employed for some role in the assessment of patient outcome. These models are Simulation, Markov model, Bayesian belief networks, Bayesian statistics, Conventional statistics, and Kaplan-Meier analysis (25). These models or techniques are nonspecific and used widely in the medical and other non-medical fields.

#### 1.2.1 *Simulation Model*

Simulation is attempts to mimic the behavior of a system in reality, for example, a flight simulator mimics the behavior of an aircraft and used for training purposes (26). it is a methodical framework for generating the best available evidence about how the world works by joining knowledge and data from many diverse sources (26). it is used when direct implementation is impossible, to better understand and predict the future behavior of a system, and to aid in decision-making (27). Simulation starts with a set of assumptions about the simulated system and resembles its functioning using some mathematical formulae (27). It offers the flexibility to characterize complex situations, incorporate time-dependent events and evaluate the consequences of a given strategy or set of strategies (27). it can be used to evaluate the cost-effectiveness of different clinical interventions and policy strategies (27). Simulation in healthcare decision problems has increased

considerably with the advances of computing power (27, 28). There are many simulation methods; for example, discrete event simulation (29), system dynamics simulation (27), probabilistic simulation, and Monte Carlo simulation (26, 27).

1.2.2 *Markov Model*

Markov model is useful when a decision problem involves exposure to risks or events over time, in which the specific timing of an event is uncertain (26). Markov model affords a way of modeling which evaluates clinical problems with ongoing risk. The model consists from Markov states, which represents the states of health of interest for patient who is transited from one state to another with transition probability (27). The states should be mutually exclusive, in which the patient cannot be in more than one state at a time (30, 31). Every state is assigned a utility scaled from zero to one, and the value of this utility depends on the length of time spent in the state. The time horizon of the analysis is divided into equal intervals of time, every interval is called as Markov cycle. The length of the cycle usually represents a clinically meaningful time interval. For a model that covers the entire life of a patient, the cycle length can be one year. During every cycle, the patient may move from one state to another. Every state is represented graphically by a circle. Circles are connected by arrows, and the direction of the arrow represents the transition from a state to another. Not all transition is allowed. For example, if there is three health states Well, Disabled, and Dead, the transition from Disabled to Dead is allowed while the transition from Dead to Disabled is not allowed. Arrows emanates from a state to itself show that the patient may remain in that state in many cycles. The resulting diagram is called a state-transition diagram (32).

Markov process must have at least one state in which the patient cannot leave. These states are necessary for the process to terminate. Those states are called absorbing states and death is an example for an absorbing state (33). Markov models are mainly suitable to model chronic diseases (33).

Markov models simply and directly can handle both costs and outcomes simultaneously (33). The summation of all state durations in which every state multiplied by its utility will yield the quality adjusted of life years (33). Cost-effectiveness could be performed for every cycle, state or for the entire lifetime of the model (33).

1.2.3 *Bayesian Belief Network Model*

A Bayesian belief network structure or causal networks is a finite directed acyclic graph composed from nodes and arcs, nodes represent the variables or events of concern, and arcs from parent nodes to child nodes represent a probabilistic relationship between the child and its parents. For example, one node could represent the benefits of a drug, and another node the risks (33). These probabilities represent the uncertainty of the relationships among the variables (26). This method is used for probabilistic inference in case of uncertainty (34) and translate information into dependence relations among variables under specified conditions (26).

Bayesian belief network can calculate for the joint probability of combination of variable states (35). Building a Bayesian belief network structure includes the selection of relevant variables by experts, the identification of the relationships among the variables, the identification of qualitative logical and probabilistic constraints, the evaluation of probabilities, and sensitivity analysis and evaluation (34). Bayesian belief network can use different types of evidence including both objective data and expert judgments, change believes in the light of the new information and making predictions even with incomplete data (36).

Bayesian belief network was used for the estimation of cause-specific mortality rates (37), the prediction of the outcome of disease and treatment (38), the diagnoses of disease, the selection among treatment alternatives, the construction of disease models, and in bioinformatics (39).

1.2.4 *Bayesian Statistics and Conventional Statistics Model*

The probability of causal inference could be calculated mainly by two types of statistics, which are Bayesian and conventional statistics (36). A Bayesian statistics analysis uses a previous knowledge to form a prior probability distribution for the value of interest and adds new evidence, which is constituted by a model to produce a posterior probability distribution (40). Different experts will have different prior beliefs, and this issue will affect the posterior results, in this case, sensitivity analysis is performed to evaluate the effects on the posterior distributions of these different beliefs (41).

Bayesian statistics is used widely in the medical statistics, including clinical trials, epidemiology, meta-analyses and evidence synthesis, molecular genetics and decision-making for new technologies (41). Bayesian statistics is complex and requires advanced mathematical expertise and extensive computing power of hardware and software (42).

In contrast to the previous approach, the use of prior information in conventional (frequentist) statistics tends to be unsystematic and informal (43), and introducing prior subjective assumptions into an inference is unpalatable to some statisticians (41). Conventional statistics uses only information obtained from the study, and does not include any prior information into the inferential process (44, 45).

1.2.5 *Kaplan-Meier Survival Analysis Model*

Kaplan-Meier analysis is a measure for the fraction of subjects surviving or the probabilities of occurrence of an event for a certain amount of time after therapy (46). This method can also be used for any single measurable quantity of either undesired or desired effects to check the change over time of this effect (26, 47). Examples of events are myocardial infarction, recovery of renal function, first renal transplant, or time to discharge from hospital (26). The Kaplan-Meier approach can evaluate the cumulative survival over time (48). If d subjects are surviving at the end of the time interval and n are the total number of subjects, then the estimated probability of surviving this interval is d/n (49). Total probability of survival until particular time interval is calculated by multiplying all earlier probabilities of survival for all preceding time intervals (47). Kaplan-Meier approach does not support multi-criteria analysis for many effects, nor include uncertainty (47, 50) and it is not suitable to use for more than one competing risk (26,48).

### 1.3 Desired Features of an Ideal Patient Clinical Outcome Assessment Model

Based on the literature research of patient clinical outcome assessment models, we identify the following desired features that should be fulfilled by a well-established ideal patient clinical outcome assessment model.

- An ideal model should first be clinically relevant (49). Main patient concerns seek components which are improved in the clinical setting. Also, the model should focus on fields under medical practitioner's expertise.
- Model assumptions should be disclosed and explicit (51).
- Consistency: in which states that are logically worse or better must be reflected in the model (52), and intuitively reasonable to clinicians and researchers (53). It should make sense to anyone using it and be seen as a realistic way for evaluation.
- Coherency: to ensure that model's based decisions do not contradict each other or the objectives that are to be met (26).
- Interpretability: which is the degree at which the meaning of quantitative scores can be easily understood (26). The outputs of the model should be understandable in quantitative form and interpretable in the user's terms, to facilitate comparison between options (10, 11).
- Answer important and practical clinical questions (26, 54), and the output of the model should lead clearly to action.
- Be relatively simple (26).
- The structure of a model should not be driven by the availability of data with which to populate the model (55).
- The model should develop insight and promote learning about the evaluation process. It should also be easy to teach and use (56).

- The model should provide a clear audit trail so that all aspects of the assessment process can be traced (26).
- Be based upon the concept of health (26), and should consider quantitative aspects of health as the capacity for improving and maintaining health (57). It should also covers the domains of the ICF (58), exceeds ICF conceptual limitations, and covers domains which are clinically important and not covered by ICF.
- Combines and includes all clinical heterogeneous components of benefits, harms and risks with different units and parameters using single scale (59).
- The model's results should not change relative evaluations when alternatives are added or removed (52).
- The model's values should be aggregated to evaluate the health system, state, and national health levels (51).
- Sets a conception for weighting and trading off benefits and harms (60). This is still a difficulty, and this step is necessary to avoid the limitations of old methods.
- Handles multiple benefits and harms concurrently (61). This is necessary to avoid the limitations of old methods.
- The model should have the ability to be supported by computer software in which user can make changes quickly and get immediate feedback (62); so, the model could be used in everyday clinical practice through an operational system.
- Used for any purpose, setting or population. This is necessary to avoid the limitations of old methods.
- Flexible and adaptable (26), and allows assessment update as new information and therapy modalities become available (54). This is still a challenge in the clinical field.
- Considers and recognizes time factor for both benefits and harms (63).
- Considers natural history of the disease, natural history of the disease includes incidence or prevalence, extent of control or cure for disease, self-limiting conditions, chronic and progressive disease states, underlying disease with intermittent acute exacerbations, mortality, life expectancy, severity and seriousness of diseases and adverse events, and the times of events occurrences and their sequence. Also is should include emotional and psychiatric disorders. The desired method should provide a clinical numerical rather than subjective value. This is necessary to avoid the limitations of some old methods.
- Handles patient health preferences through real rather than hypothetical clinical situations. This is necessary to avoid the limitations of old methods.
- Patient health preferences should be time dependant (23), in which the model could track if patient preferences change with time. This is necessary to avoid the limitations of old methods.
- Deals with both active and passive patients regarding their remedy. Both types of patients are handled in the clinical setting (26).
- Considers the causality specially with the presence of comorbidities and concomitant therapies associated with patient disease (25).
- Considers the strength or quality of the clinical evidence (64)

## Conclusion

Patient outcome is the status upon a patient's adherence to treatment, and is increasingly seen as significant for clinical adherence and uptake of healthcare interventions (65). Assessment of patient clinical outcome is one of the important aspects of patient safety, and requires the assessment of the benefits, harms and risks of therapeutic options and comparing between them. Qualitative, and quantitative models are developed for this process. However, very few models are developed for the clinical field and there is still a need for more accurate models for such evaluation.

## Recommendation

Thus, it is very important for researchers, clinicians and health care professionals to understand the developments in the domain of the assessment of patient clinical outcome, thus enabling them to propose ideal assessment models.

## Relevance of the study

This paper presents an informative review of current issues regarding the assessment of patient clinical outcome. First, it sums up the advantages of the assessment of patient clinical outcome. Then, it provides a review of a number of the existing patient clinical outcome assessment models, namely:

Simulation, Markov, Bayesian belief networks, Bayesian statistics and Conventional statistics, and Kaplan-Meier analysis models. Each model has been studied with emphasis on its usage in the medical assessment. Finally, based on the literature research of patient clinical outcome assessment models, it demonstrates the desired features that should be fulfilled by a well-established ideal patient clinical outcome assessment model.

## Authors Contribution

All the authors had made substantial contributions to conception, design, data collection, analysis and interpretation of data; drafting the article, revising it critically for important intellectual content; and final approval of the version to be published.

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## Tables

### TABLE 1 OVERVIEW OF ASSESSMENT MODELS FOR PATIENT OUTCOME

| Models for patient outcome assessment | References |
|---|---|
| **Simulation Model** | EMA (26)<br>Stahl (27)<br>Rutter *et al* (28)<br>Weinstein (29) |
| **Markov Model** | Brandeau (30)<br>Barton *et al*(31)<br>Patten & Lee (32)<br>Briggs & Sculpher (33) |
| **Bayesian Belief Network Model** | Maglogiannis *et al* (34)<br>Garbolino & Taroni (35)<br>Lucas *et al* (36)<br>Gregori *et al* (37)<br>McMahon *et al* (38)<br>Van Gerven *et al* (39) |
| **Bayesian Statistics and Conventional Statistics Model** | Lecoutre (40)<br>Austin *et al* (41)<br>Ashby (42)<br>Berry (43)<br>Briggs (45)<br>Eddy (44) |
| **Kaplan-Meier Survival Analysis Model** | Petrie & Sabin (46)<br>Goel *et al* (47)<br>Jager *et al* (48)<br>Ludbrook & Royse (49)<br>Rich *et al* (50) |