Incorporating Validated Measures for Patient-reported Outcomes in Clinical Practice

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Abstract—Patient-reported outcomes (PROs) reflect the subjective experience of the patient with respect to their well-being and quality of life. Historically there have been barriers to routine assessment of PROs, including methodological challenges and clinical realities. This paper presents three approaches that have capitalized on clinical realities and technological advances to integrate PROs into an improved case management approach to health care. Empirical data indicate that PROs are prognostic for patient survival and can have profound impact on the course and success of treatment.

Keywords—Quality of life, patient-reported outcomes, web-based system, case management.

I. INTRODUCTION

The standard tools of clinical practice for assessing, screening and diagnosing the health status of a patient are observer-driven: they rely on medical imaging techniques and processes, laboratory tests, other monitoring or gauging devices, and on the analyses and interpretations of medical professionals. They form the foundation for clinical decision-making in the endeavor to deliver quality medical care to the patient. These biometric approaches, however, do not address or capture the patient’s subjective impressions of their condition in their daily life, or the subjective patient experiences.

It has long been recognized in medicine as important to understand the perceived quality of an individual’s daily life, or ‘quality of life’ (QOL) [1]. That is, the patient’s perceived assessment of their general well-being — obtained from the patient rather than the observer — is an important measure of a patient’s health status.

Quality of Life (QOL) is an umbrella term for things other than survival [2]. QOL is measured by patient-reported outcomes (PROs), which are subjective judgments of any aspect of a patient’s health status that come directly from the patient. PROs can include functional status, symptoms (intensity, frequency), satisfaction (with medication), multiple domains of well-being, and global satisfaction with life.

At the end of the 20th Century, a plethora of assessment tools were available for exploring QOL and measuring PROs. The use of PROs, however, was significantly constrained by disappointing clinical trial results that were confounded by missing data, ambiguity on clinical significance, and reliability issues. Today, virtually all validity issues have been either resolved or clear guidelines have been established [3], [4].

Clinicians have always been interested in assessing their patient’s QOL, but there have been other lingering methodological hurdles to fully incorporating PROs in practice. QOL instrument bases worldwide contain myriad measures of well-being. The length, breadth of scope and the sheer volume of instrument choices alone, however, can make appropriate scale selection and translation from research to clinical practice highly impractical, given time and resource constraints.

Further, routine clinical assessment of patient QOL requires measures that are specific and easy to understand for both the clinician and the patient. Brevity and simplicity are also essential for reducing burden on the patient and practitioner. The efficacy and utility of the measures must be clear to the practitioner: “what do I do with PRO data?”, and “how are these data linked to ‘hard outcomes?’” The nature of the questions, response options and the results must all be meaningful, relevant and reliable for achieving the clinical practice outcomes of quality patient communication and evidence-based decision making, regardless of the patient’s level of health literacy.

The challenge is to measure QOL through PRO with the observational rigor of any other vital sign or lab test, yet with the ease of administration and rapid processing the clinical environment demands. Much work has been done in the last decade toward the development of a theoretical model of QOL that has now been generally accepted [5]. There are also numerous online psychometric resources to facilitate QOL research. For example, a forms bank has been built which sources validated survey instruments that are applicable in a variety of contexts and generalizable to a wide array of populations (www.qolpro.org).

The overarching vision for the work presented in this paper is to apply QOL-related PROs as an ‘integrated vital sign’ that helps the health care team quickly understand the baseline resiliency of the patient and potential vulnerabilities to adverse events. This ‘individualized medicine’ approach using QOL as a biomarker, equips the provider with a better roadmap for designing not only a treatment program, but also a preventive
health program that reduces needs for emergent care, improves quality of life, and ultimately provides longer survival.

II. METHODS

The PRO approaches that we have found efficacious, for both patient and observer, are as follows:

A. Screening via Single-item QOL Measures

Use of a simple, single-item global measure of overall patient QOL was explored for relevance to clinical practice (Fig. 1, question 1) and was found to be diagnostic and prognostic for survival in a wide spectrum of cancer patients [6].

Please circle the number (0-10) best reflecting your response to the following that describes your feelings during the past week, including today.

How would you describe:

1. your overall Quality of Life?

   0 1 2 3 4 5 6 7 8 9 10
   As bad as it can be
   As good as it can be

2. the severity of your pain, on the average?

   0 1 2 3 4 5 6 7 8 9 10
   No pain
   Pain as bad as it can be

3. your level of fatigue, on the average?

   0 1 2 3 4 5 6 7 8 9 10
   No fatigue
   Fatigue as bad as it can be

More specifically, an overall QOL PRO score of 5 or less on a 0-10 point numerical analogue response scale indicates a clinically significant deficit in overall quality of life [7]. This measure has been validated repeatedly in cancer patients and other populations and the cutoff has been independently verified by other investigations [8]. Normative results indicate that healthy volunteers will average about 8.2, hospice patients will average 7.8, advanced cancer patients will average between 6.0 and 7.5, newly diagnosed patients will average 4.4. A score below 5.0 or a change of 2 points over time is indicative of a need for immediate exploration and intervention for the QOL deficit [9]. Multiple studies have indicated that a deficit in overall quality of life is associated with a doubling of the risk of death at one, two, and five years across a broad spectrum of cancer patients [7]. This single-item QOL measurement approach was expanded to include the domains of pain and fatigue (Fig. 1, questions 2 and 3) based on literature that indicates they are prognostic indicators for how well a patient will deal with the ardors of cancer and its treatments, even to the point of survival. Extensive data in the literature indicates that both fatigue and pain are related to disease and treatment outcomes as well as being prognostic for survival [7-9].

B. Systematic Monitoring of Key QOL Domains and Pathways for QOL Deficit Management

QOL literature reviews and meta-analyses have identified the most important aspects of well-being to monitor when an overall QOL deficit emerges [10]. For circumstances requiring more in-depth assessment, a series of twelve items were developed using the same numerical analogue scale (LASA), covering more domains than the short form for overall QOL, pain and fatigue.

A web-based system was then designed to collect scores from 0-10 on these twelve domains (Fig. 2) [11]. If a score on any domain is indicative of a clinically significant deficit or a significant drop from the previous visit, the score is color coded to draw attention to the problem. Fig. 2, for example, displays the report for a patient who has completed eight weeks of radiotherapy, providing PROs on the 12 domains at baseline, weeks 1, 3, 5 and end of treatment. The patient reported many deficits across numerous domains (e.g. all pain scores indicated a clinically significant deficit).

Attached to each domain is an algorithm delineating clinical pathways based on the US National Comprehensive Cancer Network guidelines for dealing with distress in cancer patients [12]. The system report also has web links that generate a flow chart of pathways for deficit management for every clinically
meaningful deficit or change from previous visit (Fig. 3). The pathway in Fig. 3 for the overall QOL domain indicates that upon identification of a deficit, further investigation to delineate the source and nature of the deficit is indicated.

C. Identifying Key QOL Patient Concerns and Matching Resources to Improve PROs

The realities of modern clinical practice are such that in many visits it is not practical to be able to deal with more than one issue beyond the routine clinical outcomes update (e.g., disease status, blood work, imaging discussion). This clinical reality triggered a practical evolution in measurement approach: focusing on the patient’s reality of their biggest challenge in managing their health circumstances.

An interactive system (PROQOL) was developed that expands on the clinical pathway approach by delineating ten categories of possible patient concern that are depicted with visual cues, and then asks only one question: “Please touch the picture that corresponds to your single biggest concern right now ….” (Fig. 4).

Next, the patient is presented with a checklist of potential aspects affecting them within the identified domain of concern and asked to check all that apply or to specify something else not listed. They are subsequently asked a few items relating to key domains of QOL for longitudinal follow up.

In response to each potential concern a detailed set of potential actions is constructed from a documented repository that involves not only clinicians (doctors, nurses, etc.) but every stakeholder in the patient’s health care (e.g., social network, financial health network, spiritual network, other personal and community resources). This allows for every possible stakeholder to be involved in addressing the patient’s biggest concern in a comprehensive and integrated manner.

The PROQOL system, initially developed and tested in patients with diabetes, is easily translatable to all disease conditions [13].

Fig. 3: Clinical pathway for a deficit in overall QOL

Fig. 4: PROQOL system biggest concern screen

III. RESULTS

A. Screening via Single-item QOL Measures (Case Studies)

Since July 2010, over 30,000 individual clinical visits to the Mayo Clinic Cancer Center have incorporated the three single-item measures of overall QOL, pain, and fatigue. It has been well-received by both patients and clinicians [14]. Between 20% and 50% of patients, depending upon oncology clinic, have reported QOL deficits and had clinical interventions or treatment modified as a result, exemplified in three cases below:

A patient with a previously treated hematological malignancy indicated a QOL score of 2 (out of 10) upon a follow-up visit 8 years post treatment. When queried, the patient indicated that they were not sleeping well due to “stupid thoughts.” These thoughts turned out to be suicidal ideation. The patient was referred to a psychologist and their QOL increased to 7 the next month. This indicated that a simple general question can tap into unresolved issues that do not arise in the typical visit.

A second patient with colorectal cancer had completed chemotherapy three months prior to a visit during which he indicated a QOL score of 0 (as bad as it can be). When queried, the patient indicated he was experiencing erectile dysfunction. Counseling and a prescription were provided and
his QOL rebounded to 8. Here again, this issue would not have arisen in routine visits.

A third patient was recovering from surgery when he indicated a pain score of 7 (out of 10). The clinician administered a physical range of motion test which produced a score that would not be likely achievable if the patient was in pain. The clinician indicated that they were afraid to give more pain medication as that might cause more problems for the patient but they did not want the pain to go unchecked either. The clinician decided to increase the pain medication slightly and to closely monitor the patient for any signs of side effects. The patient reported a drop of pain to 2 and experienced no side effects. This example illustrates how PROs and laboratory tests can be integrated together into improve care.

B. Systematic Monitoring of Key QOL Domains and Pathways for QOL Deficit Management

A total of 148 patients receiving radiotherapy over five to eight weeks were involved in a pilot project of the real-time 12-Item (LASA) QOL assessment monitoring system. Individual QOL item scores were transformed to a scale of 0 to 100 points, with 100 being the best possible score and paired comparisons procedures (t-test, Wilcoxon procedures) used to assess statistical significance (p-value<0.05) over time.

This protocol not only obtained psychometric scores via the QOL tools and algorithmically-linked the LASA scores to treatment pathways. It also sought to enhance PROs by providing the opportunity (time) and conducive environment (space) for patient-provider discussions about individual scores and available clinical pathways for managing QOL deficits.

Over 80% of patients reported they felt they could easily talk about personal issues with their provider, felt comfortable asking questions, felt their provider listened to them and made an effort to give patients control over treatment decisions, and seemed to think providers understood how they felt. After participating in this protocol, patients reported on average a 5-point QOL score improvement in pain severity; 4-point QOL score improvement for fatigue level; and 1-2 points better QOL in the PRO domains of mental well-being, social activity, spiritual well-being, and pain frequency. Roughly 22% and 32% of patients reported a clinically significant improvement over the 5-8 week course of radiotherapy in mental well-being and emotional well-being respectively.

Using this monitoring system increased clinic visit time by 4 minutes on average but was not seen to add more work to the clinical practice. The primary concern raised by clinicians regarding the QOL assessment system was that the clinical guidelines needed to be more directive and specific. Over 96% of patients endorsed the use of the system and over 90% of the clinicians indicated that they were satisfied with the enhanced communication provided by the system for the clinic visits.

C. Identifying Key QOL Patient Concerns and Matching Resources to Improve PROs

Following participant recruitment for the initial field testing at four clinical sites serving diabetes patients, the PROQOL system was implemented by local teams as was practical within their endocrinology clinic flow. A total of 50 patients with diabetes per site were included in the initial pilot. Patients completed PROQOL measurement either in-clinic or in their homes.

In one case example, a patient indicated the single biggest concern for them was ‘money.’ Fig. 5 displays the ten possible items that the patient can check once they have identified that the money domain is their biggest concern for that visit. The patient reported problems paying medical bills, resulting in forced choices to cut prescribed pills in half and to skip dental, vision or mental health care needs due to cost.

![PROQOL system money domain checklist](image)

The report generated by the PROQOL system is exemplified in Fig. 6. This report is shared with the physician and patient, and optionally to other key stakeholders in the clinic and community as needed. Continuity of care is assured by identifying a person responsible for taking specific actions on behalf of the patient at the bottom of the report. The example in Fig. 6 displays a report for a fictional patient, indicating that their biggest concern expressed for that visit was “money”. Within the domain of money, three specific aspects were indicated: problems paying medical bills, cutting pills in half or skipping medicine, and skipping other health care visits because of cost. For both the general money domain and the specific aspects identified, the report lists a number of potential actions that can be taken from across the spectrum of stakeholders from both inside the clinic and within the community.
Fig. 6: PROQOL system money domain sample report

Fig. 7 demonstrates that to deal with the patient-reported concerns about money, actions could be taken by a wide variety of stakeholders than can significantly reduce a patient’s burden and improve patient well-being. The doctor can review medications for less expensive alternatives. The nurse can review available supportive care options and other aspects of case management. Community resources can be identified regarding financial services and government programs. Finally there are things that the patients can do for themselves including accessing web-based resources.

In summary, the protocols established within the PROQOL System Parameters produced a clinical pathway system for this patient that: 1) clarified the issue with further probative items (Fig. 5); 2) identified the actions that the clinician and patient could take (Fig. 6); 3) tracked this information and provided feedback for future visits and further needs assessment (Fig. 6); and 4) engaged appropriate referral/supportive services at the clinical and community levels (Fig. 7). The case management flow of the PROQOL system is the same for each major concern identified although the actions associated with each concern may be different.

Fig. 7: PROQOL system stakeholder flow

Patients in the pilot project selected the full range of concern domains. money was chosen as the biggest concern by 29% of the patients, followed by physical health, emotional health, monitoring health and health behaviors. Five out of 10 patients reporting money concerns indicated that they had problems paying their medical bills and 4 patients had put off or postponed getting health care they needed. No concerns were raised with the PROQOL administration. Comments from participants and clinicians indicated that the PROQOL fulfilled previously unassessed and unmet needs, remedying a longstanding omission in traditional diabetes care.

IV. DISCUSSION

The overall goal of this research was to integrate PRO consistently and efficiently into clinical practice with minimum burden to the patient, clinicians and affiliated systems to improve clinical outcomes. The clinical trials and pilot projects described herein indicated overwhelming support for the incorporation of the simple PROs and the clinical pathways. Simple single-item PROs have demonstrated merit and using modern technology can make longer assessments achievable within the realities of clinical practice. These findings are consistent with outcomes of a prospective study demonstrating a dramatic increase in 6-month QOL survey compliance rates from ~50% to ~90% (p<0.001) by taking advantage of web-based technology [15]. Velikova and colleagues have found similar results with European patients [16]. The web-based approach almost eliminated institutional error as a cause of missing data by using real-time email reminders.
There is now substantial evidence that PROs are indeed credible targets for assessment and intervention. There is a sound scientific and empirical underpinning for the clinical use of PROs. Clinical significance cutoffs have been established allowing for the calibration of PROs. Empirical data show that how the patient says they feel is related to how they thrive and survive. Preliminary clinical pathways based on PRO scores have been validated and are being refined. Patients can complete brief targeted PRO assessments at different points of care. PRO data can be coded and displayed to healthcare providers in real time with suggested clinical pathways, which allows for triggering of need for supportive care or treatment modification as well as longitudinal surveillance.

Incorporating PROs into clinical practice ensures that the most outstanding issue will be identified, essentially through a process of patient ‘self-triage,’ opening the door for clear and unambiguous self-reporting of the greatest challenge or threat the patient is experiencing to their quality of life, and possibly, to their survival. This ‘self-triage’ is critical, not only for the patient-reported outcomes, but also for effective practice because no set list can capture all possible concerns that a patient may have as their biggest issue for a given visit. PRO systems allow for the voice of the patient to be routinely integrated into health care, which ultimately is the most important voice.

REFERENCES


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