Seminar Briefing
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How do we Measure the "Value" in Value-Based care?

Ian McKenna, General Manager, Galen Research

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1. Introduction

Determining and defining value in health care is a persistent challenge in every country. To some extent, what constitutes value depends on perspective, e.g. a pharmaceutical company may view value somewhat differently than a doctor or a hospital administrator.

Appropriate measurement, essential to understanding value, thus requires that the right things be targeted using the right measures. A critical issue is ensuring that, as much as possible, measures allow valid comparisons across diseases. This seminar includes discussion of that, with examples of what works and what does not. It also provides examples of an approach, which focuses on the patient's needs, including how disease may affect identity and the ability to function day to day.

2. Change, Complexity and Challenge

Health care and the role of the pharmaceutical industry both are changing. Health care costs are rising, particularly for exciting new therapies that involve genetic engineering. CAR-T treatment, for example, represents startling advances but is undeniably expensive, requiring either new funding or reallocation of resources. New funding, whether in the form of taxes or insurance premiums, are unable to keep pace with the rising cost of providing health care goods and services.

At the same time, the patient population is becoming more complex; the greater number of older patients present multiple morbidities, lifestyle diseases (diabetes, obesity) are becoming more prevalent, and non-clinical social services increasingly influence patient care. Measurement, as a result, also becomes increasingly complex.

The traditional health care market is being challenged by new entrants. The largest technology companies, such as Apple, Google and Amazon, are threatening to disrupt the distribution model for pharmaceuticals, at least in the US, by distributing directly to patients. This may affect pricing as the power of scale may enhance negotiating power; such pricing may or may not accurately capture the value of therapies.

In the context of this change and uncertainty, truly value-based care is even more important. NEJM Catalyst, which brings together top thought leaders in health care, describes value-based care as follows.

- Value-based healthcare is a healthcare delivery model in which providers, including hospitals and physicians, are paid based on patient health outcomes. Under value-based care agreements, providers are rewarded for helping patients improve their health, reduce the effects and incidence of chronic disease, and live healthier lives in an evidence-based way.

- Value-based care differs from a fee-for-service or capitated approach, in which providers are paid based on the amount of healthcare services they deliver. The "value" in value-based healthcare is derived from measuring health outcomes against the cost of delivering the outcomes. (NEJM Catalyst, 2017)

In this model of health care, outcome measures would evolve to capture the extent to which patients' fundamental needs are met. Just what those measures should be is not yet clear, but the idea is to define value as gains that are important for the patient.

Porter describes that as follows, with the fundamental goal being improving value for the patient (Porter, 2017).

$$\text{Value} = \frac{\text{Health outcomes that matter to patients}}{\text{Costs of delivering those outcomes}}$$

Value, then, is not necessarily return on R&D investment, or how many patients are treated, but how patients are affected. The challenge is to measure and deliver health care in ways that add value.

The traditional model of health care, as figure 1 suggests, has been less focused less on the patient than on treatment. Whether the patient benefits in ways important to that patient often has been secondary in importance, perhaps at least in part because too little attention has been given to what affects the patient other than their having a disease or condition.

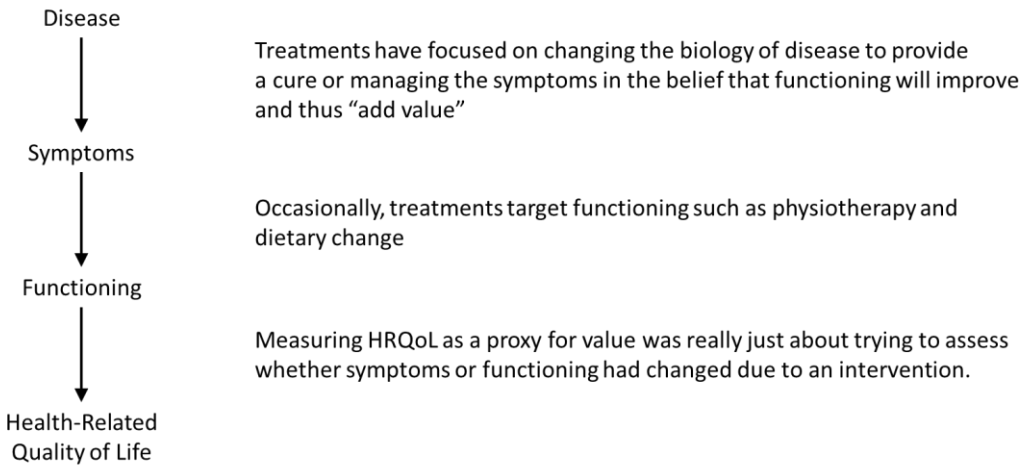


FIGURE 1. TRADITIONAL MODEL OF HEALTH

Factors that affect patients and their quality of life are numerous. Certainly, this includes what we think of as health-related quality of life (HRQoL) that results from the disease, its treatment, and its effect on functioning. But they also include factors such as family interactions, the patient's own capacity to cope with illness (personality), age, the availability of social services, the environment and culture in which the patient lives, and income. These all influence the outcome of any treatment to varying degrees. Without understanding what matters to the patient, it is impossible to know which of these influences could be adapted to positively influence health outcomes. As figure 2 suggests, thinking of the patient's milieu as suggesting possible points of intervention provides a much broader understanding of both patient care and approaches for improving outcomes.

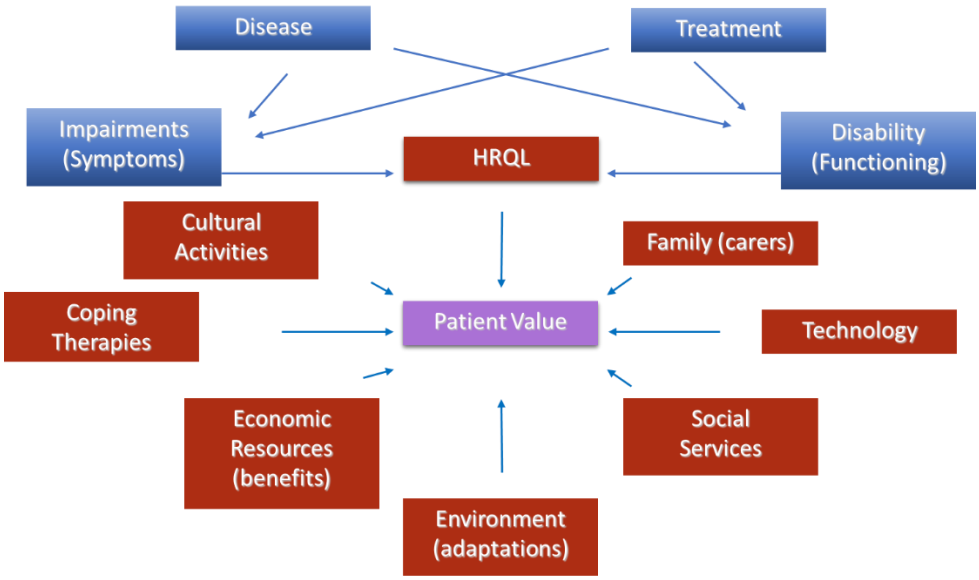


FIGURE 2. PATIENT'S MILIEU PROVIDES POTENTIAL POINTS OF INTERVENTION

Environmental adaptation provides a simple example of the importance of interventions that support a patient's health. This might be no more than providing stairlifts that allow people to remain in their own homes; research shows that people living at home live longer and can regain health sooner. As important, but perhaps even more difficult to measure, are the level of benefits provided by, say, transportation. This can present unique challenges to valuing treatment when the effects of improving health potentially lower the cost of providing other services to the patient. If a new drug therapy reduces or eliminates the need for a dietician, or physiotherapist, or home care, for example, societal costs decrease, but these are benefits that seldom are considered. As Porter's definition of value suggests, these kinds of expenses should be considered.

3. The Needs-Based Approach

The needs-based approach to patient value grew out of research we did on clinical depression. In interviews, patients did not talk about symptoms or function loss. Their concerns focused on issues of self-worth, emotional fulfilment, and relationships. Most of those we interviewed were not in a relationship or employed, and many were isolated at home — they missed interacting with others, having a structured day and a sense of purpose. What had defined them before the disease, was changed by the disease in ways that were damaging to their personal identity. The value of a treatment that could help mitigate that damage would be far greater for a patient than our traditional measures are designed to capture.

The measures we apply all are based on semi-structured interviews with patients only. Our interviews demonstrated that the impact of a disease for the patient is more about its effect on their ability to meet human needs than on functional limitations. The needs-based approach is not new and has been used by others—the Chilean economist Max-Neef has applied it to development economics, for example. Deci and Ryan have applied some of their ideas about motivation and behaviour change to health care (see Ng et al., 2012). Many applications of the idea of human need, however, are prescriptive, based on perceptions of how the world should operate or what a "reasonable" person would do. What actually happens can be quite different. Patients do not always take their medicine as prescribed; and those who are ill can be difficult, often because the disease makes them tired and grouchy. Any useful model must take account of both desirable and undesirable behaviour—the wide range of behaviours people exhibit in practice.

Our approach to understanding needs is "bottom-up", based entirely on interviews of patients with a specific condition over a number of years and in a variety of cultures. Although needs may be expressed quite differently from one country to another, the needs themselves are the same. The challenge is to determine, and then measure, how such needs are affected by a disease.

Crohn's disease can provide an example. No matter where a patient lives, the concerns will be the same: hygiene, freedom from infection, continence, safety/security, self-esteem, attractiveness, relationships, intimacy, clear-mindedness, pleasure, and autonomy. Planning for an outing, for example, will involve knowing where toilets are on the route and the time required to reach one, in turn determining which activities the patient can do when and where. The possibility of a flare-up that produces fatigue, pain, and feeling poorly can mean that planning a holiday far into the future is uncertain, at best.

Personal attractiveness and intimacy may be an issue for Crohn's patients. Those who wear a colostomy bag, for example, can face difficulties in developing relationships and achieving intimacy with another person. Some Crohn's patients may withdraw from social activities and tend to lose friendships over time, which can lead to greater isolation.

One of the results of Crohn's can be complete removal of the gut, which then requires parenteral nutrition through intravenous feeding for perhaps as long as 12 hours a day. Autonomy is seriously compromised. One of the patients whom we interviewed about the effects of that constraint wrote us later to explain how he had adjusted: "I know my life sounds really bad and I'm hooked up for 12 hours, but I have adapted to it. I bought an old ambulance and I have kitted it out with all the feeding equipment and me and my wife will go off for weekends. I go surfing or mountain biking or walking and then I hook myself up at night in my ambulance and get my feed". He had adapted his way of life and was using available technology to achieve a fulfilling life.

Of all the concerns that patients raise, across diseases, one of the most important is identity, what makes each of us who we are to ourselves and to others. We all have multiple identities: identities as children to our parents, as friends, as spouses, as work colleagues. Certain chronic diseases can have a huge impact on our ability to be who we want to be in the various contexts of our lives. Imagine what it must be like, for example, to have a condition that prevents playing with your children or caring for an elderly parent. Emotionally, disease constraints can be a difficult in a way that standard quality of life measures rarely capture well enough.

Possible future identifies are strong motivators. Think about a teenager imagining attending a particular university, or any of us imaging success in our careers or personal relationships. Visualize the achievement of such objectives is powerful, encouraging an individual to do what is required to reach that goal, even if rather unpleasant now.

Being affiliated to others also makes us feel good — being part of a team, a gang, or a family. A disease can impact our ability to achieve or maintain close affiliations. Acceptance as part of a group may require that we wear the "uniform", adopt the language of that group, and follow its customs in, say, diet and activities. Restrictions on the ability to fit in can isolate a patient. Some diseases can threaten a patient's perceived ability to be part of couple, which is a major part of identity.

A disease's threat to identity can be based on functional problems. One of the items that is part of our measurement is "I can't go to the places I want to go". Although this may not seem particularly important, it can be. For example, not being able to go to services at the mosque or church can affect one's self-regard as a member of a religion and affect membership in that group. Similarly, going to football games with a close group of friends may help maintain a sense of belonging. Or a mother being unable to pick up her child from school, as other mothers do, means more to her than a functional measure can capture, even though the barrier she faces may be functional in kind.

As an example of the impact of functional loss, let us return to the Crohn's disease patient who relies on parenteral nutrition. Eating fulfils various needs, other than nutrition: we romance over food, we go on dates that centre around food, we gather together as family for meals, we do business over meals, we mark important occasions with food, and we have religious traditions that rely on food as central icons. Not being able eat, then, is life-changing in more ways than just a change in source of nutrition.

Employment is another important component of one's identity, in addition to being a source of income that can help reach other personal goals. Work generally requires we keep to a certain schedule of days, do things in a particular way, or perhaps participate in such things as away-days that involve team building. Someone with, say, severe rheumatic disease or gastrointestinal disease will find this challenging, if not impossible; the disease will change both how they perceive themselves and how others perceive them. The limitations may be functional, but the consequences are more far-reaching than functional measures alone can capture.

The impact of disease is more than the functional limitations imposed; it is the effect of those functional limitations on the person's ability to meet their needs. What has value, then, are clinical or non-clinical interventions that enhance quality of life by helping patients meet these needs, or at least

come closer to meeting them. For patients who must live with pain, cognitive behavioural therapy that helps them adapt to that reality has value, even though the pain persists. Another example is social care, where a care worker helps the patient bathe, or feeds them, or even just socializes. The patient's functional status does not change, but the patient's emotional status is enhanced. Conventional patient status measures fail to capture this.

NEEDS-BASED MODEL OF QOL

For outcome measurement to be useful, it must be based on four fundamentals. The first is a valid theoretical model of outcome — what is being measured and why. Second, the measurement scale must be unidimensional, i.e. measure one thing only. A ruler, for example, measures only distance. Third, the possible response to the measurement question should be based only on how much it affects quality of life and the respondent's own level of quality of life. Finally, measurement should use interval values, wherever possible, not ordinal or cardinal values, because the results are more accurate when it is possible to discern the distance between each item on a measure.

To ensure that our measures are capturing what they are intended to capture, we apply a Rasch model (Rasch, 1960/1980). Developed primarily for the field of education by a Danish statistician, the approach has recently been applied to the health field (see, e.g., Browne and Cano, 2019). This allows us to:

1. Confirm that scales are unidimensional
2. Identify misfitting items
3. Explore whether the response format works
4. Determine if items are affected by factors other than what the scale is intended to measure (differential item functioning)
5. Provide interval level scores, which in turn allows allowing means and change scores to be calculated and
6. Put items and patients on the same measurement scale and in their correct locations

One-dimensionality is important particularly when designing measures for a disease that commonly occurs with another — obesity and diabetes, for example. Including measures that are more related to obesity will distort results for diabetes because not everyone with diabetes is obese. The Rasch approach identifies how well a measure fits what it is intended to measure and whether it represents accurately the population being studied.

The Rasch model also will identify any items that work differently for parts of the population—even men and women. Not surprisingly, men will respond to a question about being able to do housework differently than women. The differences also may surface in questions about limitations on movement. "I can't go to the places I want to go" may mean for women being able to do family shopping, or visit friends, or go for a coffee; for a man, it may mean going to the football ground.

When a measure fits the Rasch model it has dimensional homogeneity. Only variables that measure the same attribute can be compared, equated, added, or subtracted. That may seem to be a controversial statement because many HRQoL measure measures add, subtract and multiply different variables and different constructs to create a profile or a multi-dimensional model. We argue that this produces misleading results, as would happen if one were to try to add a kilometre to an hour. HRQoL cannot be expressed, validly, as a single totalled score.

An item may have different relevance for different people, a phenomenon that Rasch models helps capture. Placement on the scale should be the same for everyone, not a 4 for a man and a 3 for a woman. Interval scoring, the distance between each item along the scale, from very mild to very severe, must reflect the experience of the patient population.

Figure 3 takes as an example the dermatology life quality index (DLQI), a widely used measure. Items are clustered around a small area, which means the breadth of the disease is not being accurately measured. The reason is that two diseases are included in this measure: psoriasis and topical dermatitis, two different things. The experience of only half the patient population, those above the -1.0 mark in this example, is captured. Despite this, the DLQI is used to determine access to care in England and Wales.

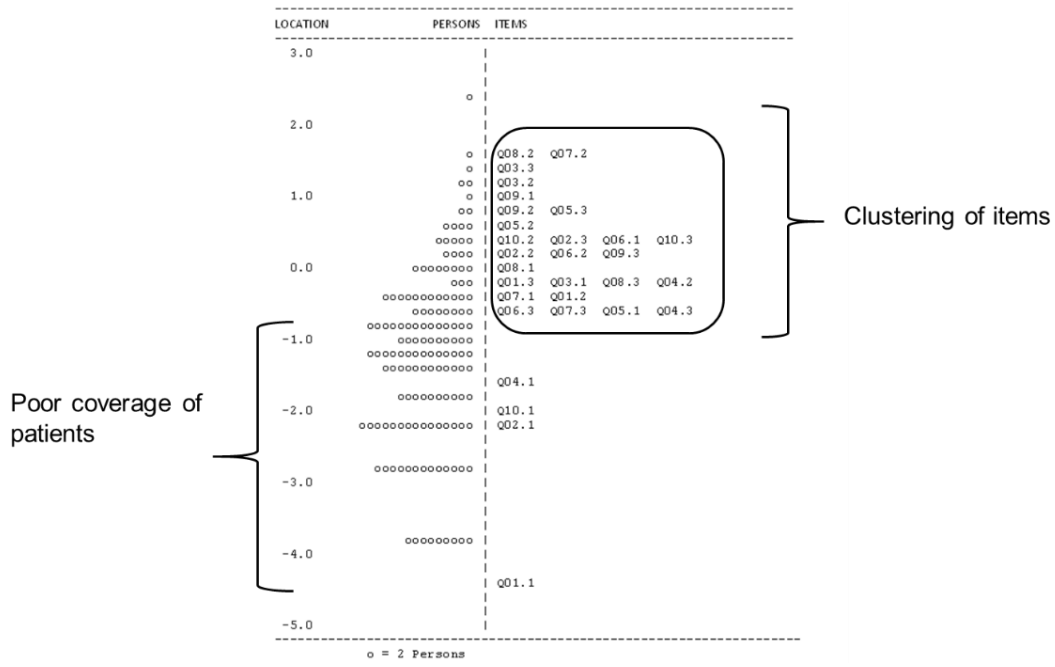


FIGURE 3. DLQI ITEM MAP

Compare the DLQI map in Figure 3 with the psoriasis quality of life (PSORIQol) map in Figure 4. When the patient and the item are reflected as a spread, as in Figure 4, measurement is far more accurate. One of the reasons the PSORIQol map shows better spread is that it is based on interviews with patients rather than reflecting the opinions of health care professionals.

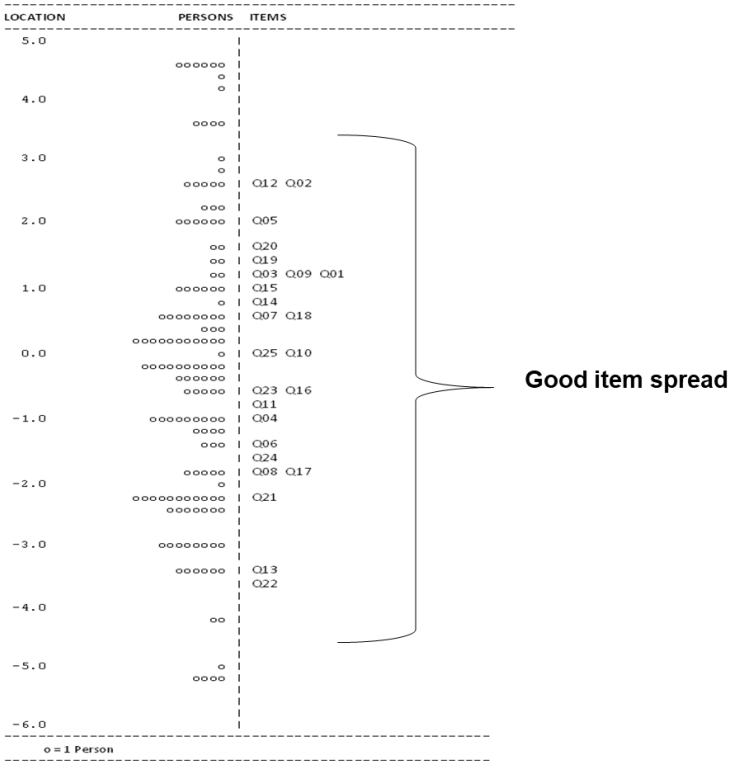


FIGURE 4. PSORIQOL ITEM MAP

Responsiveness is an important aspect of reliable measurement. "Smallest detectible difference" is an indicator of responsiveness. A simple numerical rating scale of, say, 1 to 10, has a mean score of 5; an extreme change of 1 or 10 would be necessary to reliably indicate that an intervention has had a significant effect. Figure 6 shows what percentage of change is required to ensure that the change is real, i.e. not due to measurement error.

Measure	Change required
Numerical rating scale	40%
SF-36 sections	39 – 97%
EQ-5D	36%
SF-6D	22%
HUI	38%
BASDAI	18%
ASQoL (QoL)	19%

FIGURE 6. CHANGE IN SCORE FOR WHICH ANYTHING SMALLER CANNOT BE DISTINGUISHED FROM MEASUREMENT ERROR

Van der Heijde et al. (2015) provide an example of measurement approach responsiveness in their study of adalimumab treatment for ankylosing spondylitis, as figure 7 shows. Ankylosing spondylitis is a progressive rheumatic disease that results eventually in the fusing of spine and hips. Using the SF-36, the actual change is lower than the smallest detectable difference—the margin of error is larger than the actual change. In comparison, the ASQoL measure, based on a Rasch model that reflects the patient population, detects a difference as small as 18%. This shows with certainty that the change in the patient population has occurred because of the intervention.

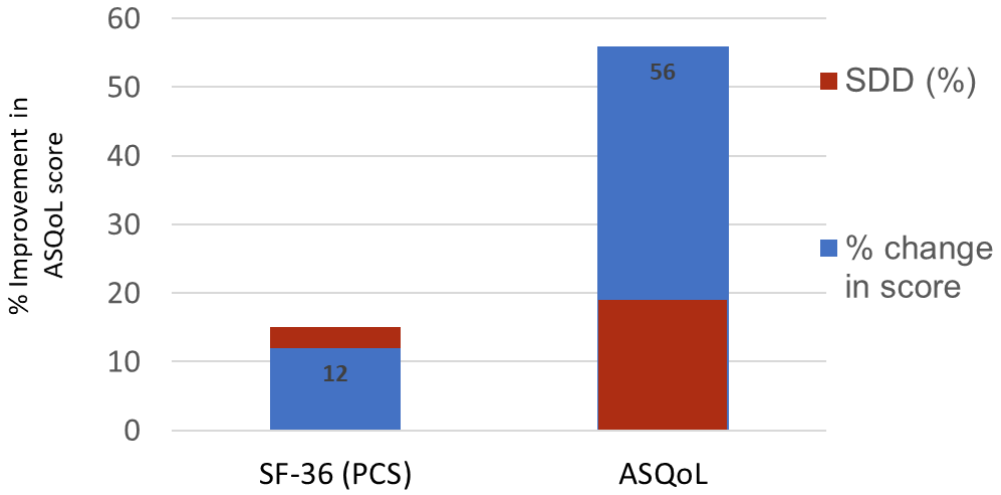


FIGURE 7. COMPARING THE RESPONSIVENESS OF ASQOL AND SF-36 PCS FOR FIVE YEARS OF ADALIMUMAB TREATMENT IN PATIENTS WITH ANKYLOSING SPONDYLITIS

Source: van der Heijde et al. (2015)

Figure 8 compares the CAMPHOR utility for pulmonary hypertension, developed using the Rasch model, with the EQ-5D and the SF-6D. The items to the right are the New York Heart Association's functional classification, ranging from 1, with no symptoms but a potential problem, to 4, where the disease imposes severe limitations on what the patient can do. The CAMPHOR model relies on interviews with patients intended to identify what is most important to them; it has a quality of life measure, a symptoms measure and an activities measure. The CAMPHOR index measures change with greater granularity, making small changes detectible. This is important because it can show the positive effects of drug treatment even for mild cases, which the other measures cannot do.

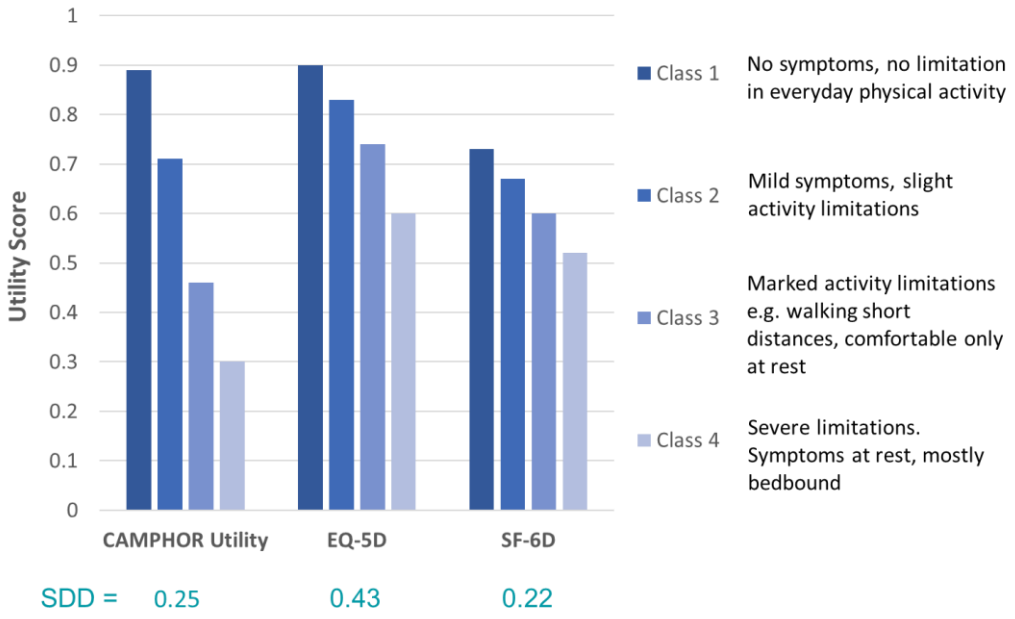


FIGURE 8. RESPONSIVENESS OF CAMPHOR UTILITY INDEX FOR PULMONARY HYPERTENSION

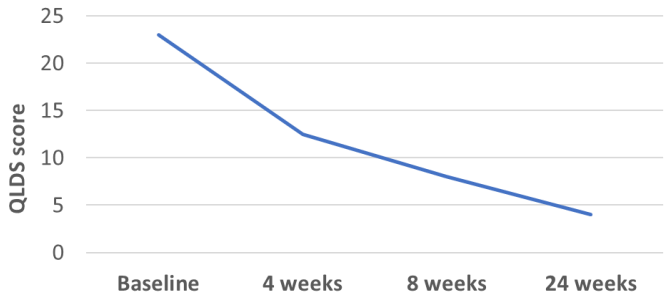
Figure 9 shows the value of using the Rasch model for small sample sizes. This is a non-clinical intervention study about hand exercise programs in rheumatoid arthritis. The measures detect change accurately between .03 and .01, which is particularly good.

	Isotonics (n=23)	**Isometrics (n=24)**
Baseline RAQoL score	19	18
4-week RAQoL score	15	14
Probability	.003	.001

FIGURE 9. HAND EXERCISES FOR RHEUMATOID ARTHRITIS (RAQOL IN SMALL SAMPLE)

The QoL-AGHDA provides another example of a responsive non-clinical measure for small samples. The measure is used by the NHS to determine whether a patient should receive treatment for adult growth hormone deficiency and whether the patient continues to have the treatments. A study by Danilowicz, et al. (2008) was based on a small sample of 11 people, but still was able to show change. In 6 to 12 months of reduced hydrocortisone treatment, subjects lost an average of 7.1 kg of body fat. QoL-AGHDA scores improved (p = 0.018).

Figure 10 shows the use of the QLDS to measure QoL changes from the use of fluoxetine for depression. Again, it demonstrates the feasibility of measuring small changes with high reliability.



Improvement of 19 points = 83%

Smallest detectable difference for QLDS = 5.3 = 15.6%

FIGURE 10. MEASURING IMPACT OF FLUOXETINE ON QOL (QLDS) N=540

Source: Tuynman-Qua, de Jonghe and McKenna (1997)

4. Conclusion

Capturing what is of value to the patient, rather than what may be of interest to the clinician or other health care professional, is fundamental to value-based health care. Our needs-based approach is developed using a theoretical model that is disease-specific, patient-centric, and also goes beyond symptoms that affect function alone. We are not measuring function but are measuring what a particular function can influence. By allowing us to factor in the effects on quality of life of other assistance, such as social care, it should allow spending to be allocated more efficiently and in ways that better reflect what aspects of life patients value.

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About Galen Research

Galen Research is an organisation that views value from the perspective of the patient, at the centre of health care decisions. Cultural norms and linguistic expressions for the various aspect of health care vary across countries, making adaptation — rather than just translation — a critical part of accurate measurement.

Galen Research has developed 35 disease-specific patient-reported outcome measures (PROMs) that have been adapted for use in up to 75 countries. Their measures are available for commercial and non-commercial use. They published over 350 papers in peer-reviewed journals.

About us
Founded in 1962 by the Association of the British Pharmaceutical Society, the Office of Health Economics (OHE) is not only the world's oldest health economics research group, but also one of the most prestigious and influential.

OHE provides market-leading insights and in-depth analyses into health economics & health policy. Our pioneering work informs health care and pharmaceutical decision-making across the globe, enabling clients to think differently and to find alternative solutions to the industry's most complex problems.

Our mission is to guide and inform the healthcare industry through today's era of unprecedented change and evolution. We are dedicated to helping policy makers and the pharmaceutical industry make better decisions that ultimately benefit patients, the industry and society as a whole.

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Areas of expertise

- Evaluation of health care policy
- The economics of health care systems
- Health technology assessment (HTA) methodology and approaches
- HTA's impact on decision making, health care spending and the delivery of care
- Pricing and reimbursement for biologics and pharmaceuticals, including value-based pricing, risk sharing and biosimilars market competition
- The costs of treating, or failing to treat, specific diseases and conditions
- Drivers of, and incentives for, the uptake of pharmaceuticals and prescription medicines
- Competition and incentives for improving the quality and efficiency of health care
- Incentives, disincentives, regulation and the costs of R&D for pharmaceuticals and innovation in medicine
- Capturing preferences using patient-reported outcomes measures (PROMs) and time trade-off (TTO) methodology
- Roles of the private and charity sectors in health care and research
- Health and health care statistics

OHE
7th Floor, Southside
105 Victoria Street
London
SW1E 6QT

Telephone
+44 (0)20 7747 8850

Email
info@ohe.org

ohe.org

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