

Patient Reported Outcomes

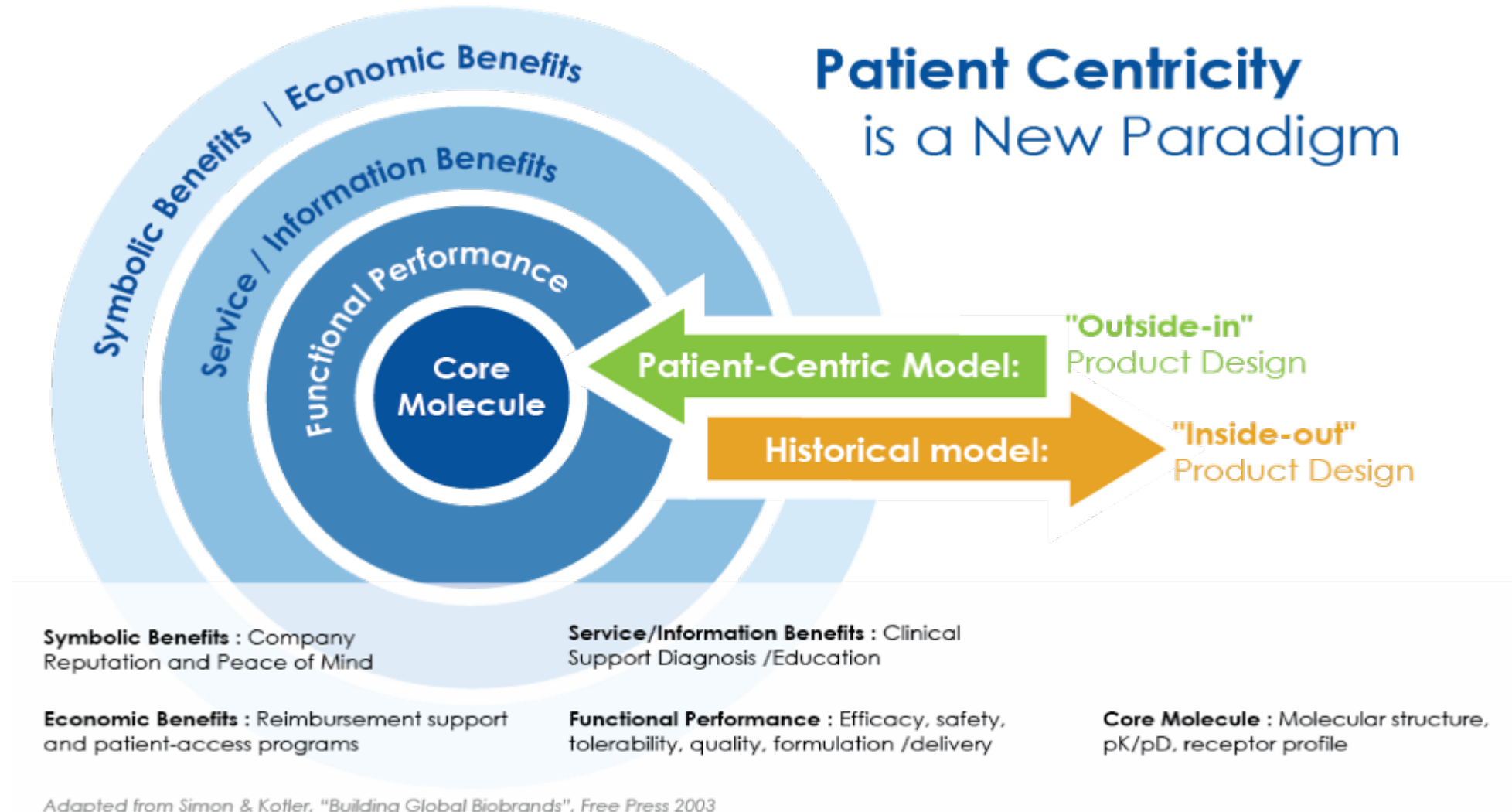
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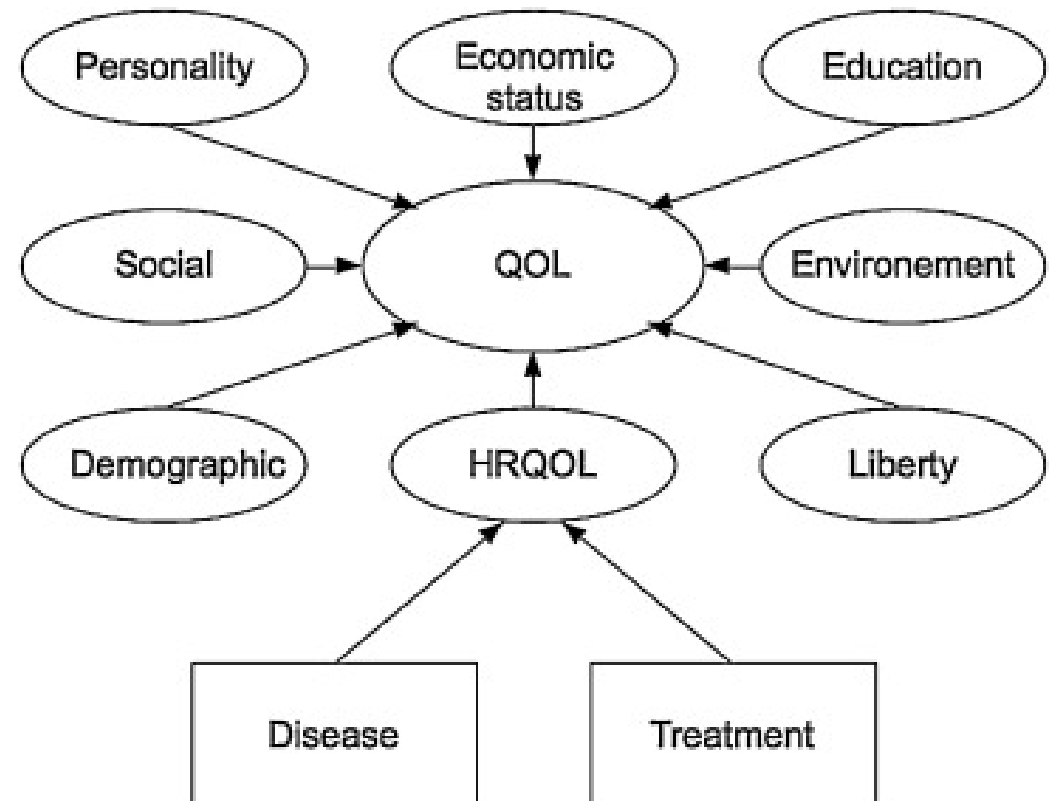
Patient Centricity

Patient Centricity is a New Paradigm



QoL (Quality of life) and HRQoL (Health Related Quality of Life)

QoL: “the perception of the individual of their position in life in the context of the culture and value systems in which they live in relation to their goals, expectations, standards and concerns.” (WHO)



Why do we want to measure HRQoL

Decision Making

- **Clinicians** might be interested in knowing the impact that their care is having on patients
- **Policymakers** may be interested in learning which intervention offers the largest benefit to patients
- Technology evaluation in some countries through **QALY (Quality adjust life year) or QOL**

Evaluation

- Evaluate the **effectiveness** of an intervention
- Used in **relation to their costs** in an economic evaluation
- Technology evaluation in some countries through

QALY (Quality Adjust Life Year)

- QALY represents the impact a therapy has on the length of life while also taking into account any changes in the health-related quality of life (HRQoL).

$$\text{QALY} = \text{Time} \times \text{HRQoL}$$

1 Give a value to HRQoL where:
 Dead = 0
 Perfect health = 1

2 On a specific time



Mrs Mandy


Diagnosis: Head ache

- No light
- No work or any activity
- No eat

HRQoL in 1 year

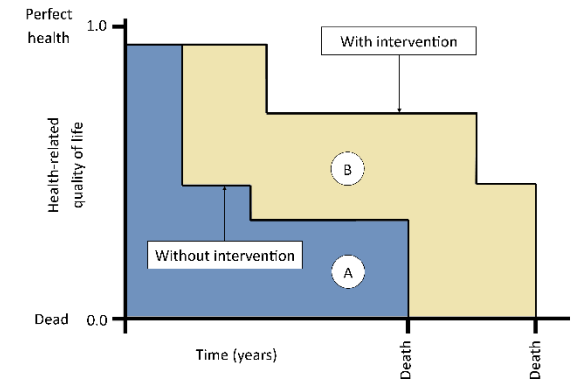


A  = 0.5

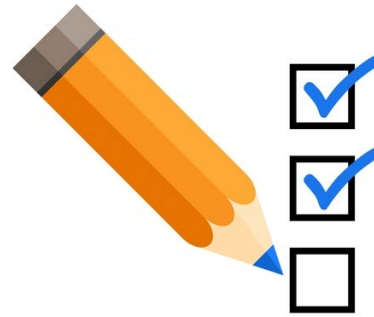
B  = 1

A  **QALY = 1 x 0.5 = .5**

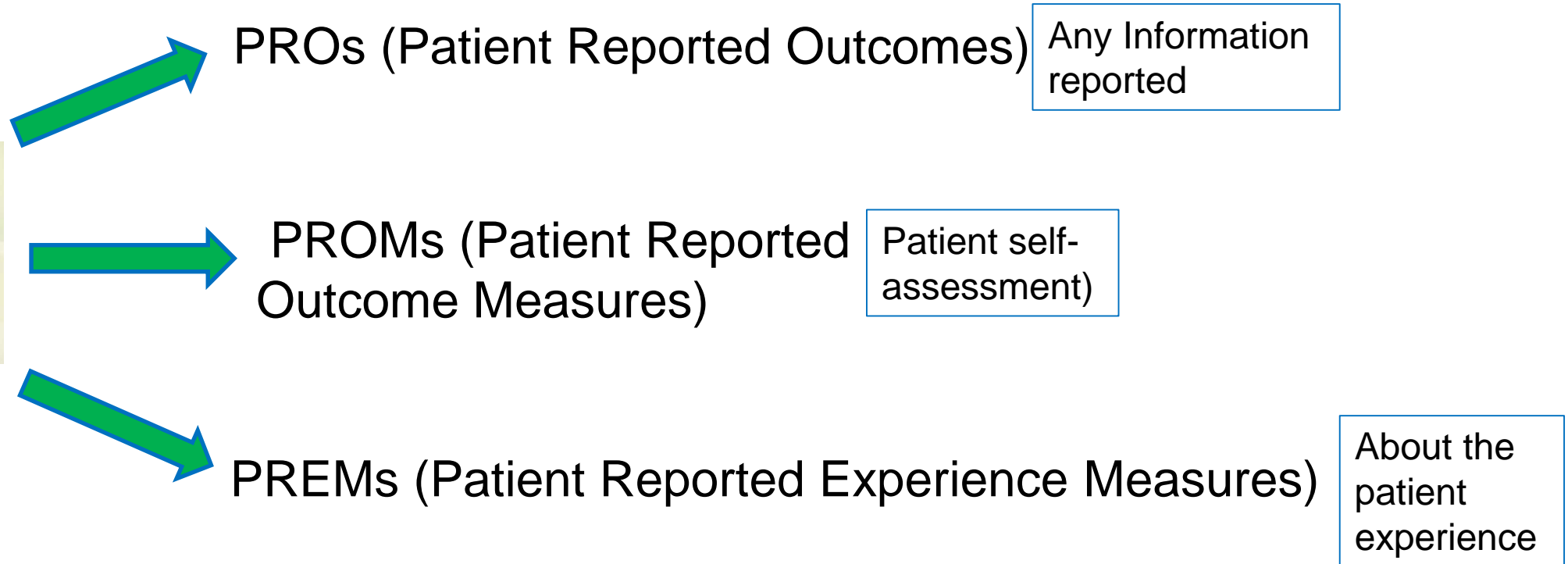
B  **QALY = 1 x 1 = 1**



How to measure HRQoL



- HRQoL is frequently measured with 'tools' in the form of **questionnaires**



Use of PROMs in Clinical Trial and Clinical Practice

Clinical Trial

- The study is subject to a specific patient cohort with similar characteristics
- Specific time duration
- Focus on symptoms of the specific disease

Clinical Practice

- It is applied to ALL the patient population without any restriction
- Long term duration
- Focus not just on symptoms but all what can worry and/or affect the patient

Some examples of PROMs to measure HRQoL

Generic

- EQ5D (Euro Qol 5 Dimensions)
- SF-36 (Short Form Health Survey)
- EORTC QLQ C30 (Cancer patients)
- ESAS (Edmonton Symptom Assess System)
- MQOL (McGill Quality of Life Questionnaire)

Symptom specific

- PERFORM (Fatigue)
- U-FIS (Fatigue)
- CFS (Cancer fatigue scale)
- IPAQ (Physical activity)
- CASQ (Cancer appetite)

Disease specific

- Brest-Q
- ALIS (Asthma)
- QoLIAD (Atopic dermatitis)
- MSQOL (Migraine)
- PRIMUS (Multiple sclerosis)
- MyPOS (Multiple Myeloma)
- EORTC (Specific Cancer Questionnaires)

Myeloma UK experience with PROMs



The existing tools

- A systematic literature review identified **13 QOL tools**
- Existing QOL questionnaires are **not myeloma-specific**
- No existing questionnaire **captured the most important aspects** to myeloma patients
- Existing questionnaires are also generally designed for **research purposes**
- **No tool designed specifically for clinical use, and only one disease-specific questionnaire:** the European Organisation for Research and Treatment of Cancer core cancer questionnaire (EORTC-QLQ-C30) with its myeloma-specific module (MY20)
- Most existing QOL questionnaires ask **only about symptom *status* and so may not capture all that is important to QOL for patients**



The need

- Review suggested that the **presence or absence of physical symptoms *per se* was not the most important determinant of QOL**
- Not a questionnaire captures **what matters to myeloma patients**
- There was a need for patients on other domains such as **activities, participation, and emotional wellbeing**
- Most existing QOL questionnaires ask only about **symptom *status***

Development

- Experts from the fields of haematology, palliative care, psychology and psychometrics
- Research (systematic review and patient involvement)
- The prototype questionnaire was pretested using cognitive interviews in a purposive sample of myeloma patients
- Validation of MyPOS in a multi-centre, cross sectional survey of myeloma patients recruited from 14 hospital trusts across England / 380 patients



Osborne TR, et al 2015. Improving the assessment of quality of life in the clinical care of myeloma patients: the development and validation of the Myeloma Patient Outcome Scale (MyPOS). BMC Cancer

Use and next challenges

Q10.	Over the past week, have you been able to carry out your usual activities without help from others?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>
Q11.	Over the past week, have you been able to pursue your hobbies and leisure activities?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>
Q12.	Over the past week, have you been able to spend quality time with family and friends?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>

We would like you to answer this question whether or not you are sexually active

Or if you would prefer not to answer then please tick here:

	<i>No, not at all</i>	<i>Occasionally</i>	<i>Sometimes</i>	<i>Most of the time</i>	<i>Yes, always</i>
Q13. Over the past week, have you been worrying about your sex life?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>
Q14. Over the past week, have you been worrying about infections?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>
Q15. Over the past week, have you been worrying about your physical appearance?	0 <input type="checkbox"/>	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>	4 <input type="checkbox"/>
Q16. Over the past week, have you been	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>



Measuring Patient Preferences

An exploratory study to determine how patient preferences data could be used in health technology assessment (HTA)

Project report

June 2019



Workshop

- Objective: Identify some of the outcomes that are relevant for patients with a specific disease and are not easy to identify during a routine visit with the doctor.
- Type of cancer _____
- Write down the relevant patient reported outcomes that are important for patients during their daily life living with the specific type of cancer
- Compare your outcomes with the ones asked by the routine EQ-5D questionnaire and discuss them in groups

Workshop

EQ-5D

Mobility

I have no problems in walking about
I have some problems in walking about
I am confined to bed

Self-Care

I have no problems with self-care
I have some problems washing or dressing myself
I am unable to wash or dress myself

Usual Activities (e.g. work, study, housework, family or leisure activities)

I have no problems with performing my usual activities
I have some problems with performing my usual activities
I am unable to perform my usual activities

Pain / Discomfort I have no pain or discomfort
I have moderate pain or discomfort
I have extreme pain or discomfort

Anxiety / Depression I am not anxious or depressed
I am moderately anxious or depressed
I am extremely anxious or depressed

Where to start?

- European Organisation for Research and Treatment of Cancer (EORTC)
Madelaine Pe

Thank you!