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General and Scalable Versus Disease-Specific and Targeted: To What Extent Do Generic Outcomes Represent Well-Being?

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Patient-reported outcomes (PROs) constitute an integral part of health outcome measurement and are an essential component in the implementation of value-based care. However, no widely agreed consensus exists on patient-reported outcomes that could be measured across diseases, thus allowing for better comparability of outcome states between diseases and across the life course of individuals. Generic outcomes could also be applicable throughout the life cycle of medical interventions and reduce a potential overburdening of multi-morbid patients with too many questionnaires.

Aim: To develop an international, multi-stakeholder consensus on generic, patient-reported outcome measurement and assess potential latent underlying factors.

Objectives

The European Health Outcomes Observatory (H2O) initiative aimed to develop an updated patient-centered generic outcome set (COS) to inform treatments using a combination of patient-reported outcome (PRO) instruments and clinical data, as a means to drive value-based health-care in Europe. Patient-reported outcomes (PROs) constitute an integral part of health outcome measurement and are an essential component in the implementation of value-based care. However, no widely agreed consensus exists on patient-reported outcomes that could be measured across diseases, thus allowing or better comparability of outcome states between diseases and across the life course of individuals. Generic outcomes could also be applicable throughout the life cycle of medical interventions and reduce a potential overburdening of multi-morbid patients with too many questionnaires.

Methods

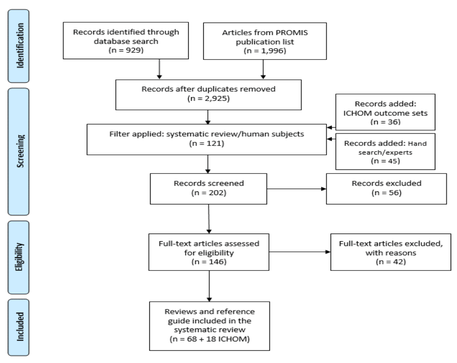
We **1)** first systematically reviewed existing generic outcome sets. Secondly, we performed a Delphi exercise to weigh the domains which we extracted from the existing sets. We then **2)** investigated the generic overlaps between outcome domains that were defined in parallel disease-specific work streams in three chronic diseases (cancer, diabetes, and inflammatory bowel disease). Following **3)** we held a virtual consensus meeting with representatives from all stakeholder groups, including patients, regulators, clinical experts and researchers from both private and public organisations. Lastly, **4)** a mapping exercise of the results was conducted using Max-Neef's model of human needs to assess the thematic and ontological cores present in the generic outcome set.

Conclusion

General themes of patient well-being are present in the literature. More macro concepts are often used to identify potential problem areas. Considering time burdens for patients, starting with a generic set during evaluation which can be artificially guided in real-time, i.e. computer-adapted testing, could be an ideal approach to their implementation. The findings were consistent with the results of the Delphi study which also identified broad concepts for inclusion. This makes intuitive sense as generic outcomes of health would relate to more holistic domains of well-being. Further research should be done to assess the differences in generic outcome set needs for acute or non-chronic illnesses. In addition, the comparison of Max-Neef's needs model to the output from the Delphi study highlight the areas well represented and the areas potentially disregarded at present. An emphasis on the qualities or attributes associated with the domains are consistently inquired, but the environmental and materials factors are not. However, these likely have as much impact on the associated outcomes of well-being as their traits, which at most would only indirectly capture such variables as available resources. Lastly, higher order needs such as freedom and identity do not appear to be fully recognized as important needs in the assessment of human well-being.

Results

left column top-down: SLR results, PROM mapping of 3 diseases, Generic overlap by disease:; **Right column top-down:** Delphi generic domains, Human Needs mapping



PROs/PROMs	PRO disease coverage*	EQ-5D	EQ-5D-3L	EQ-5D-5L	EQ-VAS	HUI3	NHP	PROMIS 10	PROMIS 29	PROMIS Global or Global 10	SF-12	SF-20	SF-36	SF-6D	SF-8	WHODAS 2.0	WHOQoL-100	WHOQoL-BREF
Mental Wellbeing	77%	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x
Physical wellbeing	66%	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x	x
Social Wellbeing	53%	x	x	x		x	x	x	x	x	x	x	x	x	x	x	x	x
Pain	48%	x	x	x	x	x	x	x	x	x	x	x	x	x	x			x
Overall wellbeing	45%	x	x	x	x		x	x		x	x	x	x	x	x	x		x
Quality of Life	30%	x	x		x			x	x	x	x		x				x	x
Energy Vitality	30%						x	x			x		x	x	x			x
Sleep	22%						x		x	x								x
Fatigue	17%					x	x	x	x	x	x		x	x	x			x
Cognition	17%					x		x		x						x		
(Health Utility)	8%	x	x	x	x	x								x				
Comprehensiveness score		7	7	6	6	7	8	9	7	9	8	5	8	8	7	5	4	9
PROM disease coverage**		30%	13%	5%	16%	5%	9%	2%	3%	19%	22%	1%	53%	8%	5%	6%	2%	7%

Generic Outcome Set	Diabetes	Breast cancer	Lung Cancer	IBD	Coverage
Overall wellbeing/QoL		x			Not included in IBD
Health status	x				
Health-related QoL	x		x		
General quality of life		x			
Mental wellbeing	x				100%
Emotional functioning		x	x	x	
Depression	x		x		
Stress and anxiety			x	x	
Cognitive functioning		x	x		
Body image				x	
Physical function		x	x	x	100%
Activities of daily living		x			
Mobility			x		
(Diabetes-specific restrictions)	x				
Social wellbeing					Not included in Diabetes, Lung cancer and IBD
Daily functioning		x			
Social functioning		x			
Role functioning		x			
Relationship(Marital problems)		x			
Fatigue			x	x	Not included in Diabetes and Breast cancer
Pain			x	x	Not included in Diabetes and Breast cancer
Sleep quality	x			x	100%
Insomnia		x	x		
Sexual function	x	x		x	Not included in Lung cancer
Disease management	x	x	x	x	100%
Treatment satisfaction	x			x	Not included in Breast and Lung Cancer
Work productivity		x		x	Not included in Diabetes and Lung Cancer
Other symptoms	x	x	x	x	100%

	PRO domain	Construct in lay language
1	**Overall health status**	A person's general physical, mental, and social well-being. It reflects the presence or absence of dysfunctions, symptoms or impairment and is also one's health-related quality of life[1].
2	**Mental wellbeing**	A person's psychological health status including among others emotions, mood, stress, fear, anxiety, depression, coping, sense of coherence, body images – both positive and negative.
3	**Physical wellbeing**	A person's overall physical health mostly related to physical functioning, i.e. ability to perform both basic and instrumental activities of daily living without or with minimum support.
4	**Social wellbeing**	A person's level of participation in social life, such as taking part in family events, other social activities or fulfilling work responsibilities – in general but also specifically related to limitations due to chronic disease[2].
5	**Fatigue**	A sense of persistent tiredness or exhaustion that is often distressing to the individual. It is closely linked to the energy level: in the absence of fatigue a person might feel totally active or alive.
6	**Pain**	A person's subjective experience of physical pain including specifically it's degree or severity.
7	**Sleep quality**	A person's own perception of their sleep quality, including depth, and restoration.
8	**Sexuality**	A person's overall sexuality including different aspects, such as satisfaction with their sex life, physical and emotional sexual functioning, as well as sexual identity and orientation.
9	**Self-efficacy**	A person's perceived personal control over their health in general as well as their perceived capability for managing (general or health-related) issues.
10	**Treatment satisfaction**	A person's subjective assessment of how satisfied they are with their treatment regimen in general (including medication). This is closely related to the perceived burden of a treatment (e.g. side effects) and the reaching of treatment goal.

	Needs	Qualities	Things	Actions	Settings
1	**subsistence**	Overall health status Mental wellbeing Physical wellbeing Fatigue Pain Sleep quality			
2	**protection**	Overall health status Physical wellbeing Self-efficacy Treatment satisfaction	Social wellbeing	Social wellbeing Self-efficacy	Social wellbeing
3	**affection**	Overall health status Sexuality	Social wellbeing Sexuality	Social wellbeing Sexuality	Social wellbeing Sexuality
4	**understanding**	Overall health status Treatment satisfaction	Treatment satisfaction	Treatment satisfaction	
5	**participation**	Overall health status	Social wellbeing Self-efficacy	Social wellbeing Self-efficacy	Social wellbeing
6	**leisure**	Overall health status			
7	**creation**	Overall health status	Self-efficacy	Self-efficacy	Treatment satisfaction
8	**identity**	Overall health status Mental wellbeing Sexuality		Sexuality	
9	**Freedom**	Overall health status Self-efficacy		Self-efficacy	

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