Towards end-user centered outcome measurement: An example from Parkinson's disease

Peter Hagell, RN PhD

Professor of Neurological Caring Science

The PRO-CARE Group
School of Health and Society
Kristianstad University, Kristianstad, Sweden

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CURRENT OPINION
Patient and Public Involvement in Patient-Reported Outcome Measures
Evolution Not Revolution
Sophie Staniszewska, Kirstie L. Haywood, Jo Brett and Liz Tutton
1 Royal College of Nursing Research Institute (RCN RI), School of Health and Social Studies, University of Warwick, Coventry, UK
2 Trauma Unit, Oxford University Hospitals NHS Trust, Oxford, UK
Seeking What Matters
Patients as Research Partners
Musa Mayer
AdvancedBC.org, New York, NY, USA
In their excellent and timely article 'Patient and public involvement in patient-reported outcome measures: evolution not revolution,[1]' Sophie Staniszewska and colleagues make important
intention to imply that they are the only experts on a given research question, or that they know better than professionals
In Context
Patients have their say on research priorities
Traditional ways of prioritising research are giving way to a more democratic process, with researchers working in partnership with patients. David Holmes reports.
"Research which reflects the needs and views of the public is more likely to produce results that can be used to improve practice in health and social care."
VIEWS & REVIEWS
The Alzheimer's Society, drug firms, and public trust
PERSONAL VIEW Iain Chalmers
For about five years before he died, my father had Alzheimer's disease. When it was first diagnosed we asked him how he wished in is condition to be described when other people asked us about his health. He chose "bewildered", and
Challenging NICE: Neil Hunt, Alzheimer's Society chief executive (centre), outside the High Court
alliance with drug manufacturers in bringing the first legal challenge to judgments reached by the National Institute for Health and Clinical Excellence (NICE). On 10 August Justice Linda Dobbs ruled against the alliance of the Alzheimer's Society and drug manufacturers on five of the six charges they had brought against NICE (BMJ 2007;335:319).
NICE is an independent body established to tackle the difficult—some would say near impossible—but essential task of trying to judge how the resources of the NHS can be used effectively and equitably in a service built on the principles of shared risk and social solidarity. This process is bo
decision making can simply guess at vital data. This is simply unacceptable . . . To retain its authority as a public body it must command the confidence of the public. The result of this case must call into question whether NICE has lost that confidence."
But how might the Alzheimer's Society's alliance with drug manufacturers erode its own authority as a charity subsidised by public—which thus also needs to retain public's confidence? The society could take the following steps to restore my respect for and confidence in it.
Firstly, it should declare clearly on its website the sources and amounts of support it receives for its work.
Secondly, having challenged NICE's judgments about the most effective use of NHS resources for the care of people with dementia, the society should make clear what alternative distribution of limited resources it regards as more appropriate—and why.
Thirdly, it should campaign for treatments for dementia to be evaluated using outcomes that are meaningful to patients and carers, and challenge the licensing and use in the NHS of any new drugs that fail to conform to these expectations.
Fourthly, it should insist that all data from clinical trials are published and that anonymised data for individual
made available
A broader base of expertise, derived from a variety of realms and perspectives, can lead to better research designs, tools and outcomes

The Alzheimer's Society, drug firms, and public trust

PERSONAL VIEW Iain Chalmers

For about five years before he died, my father had Alzheimer's disease. When it was first diagnosed we asked him how he wished his condition to be described when other people asked us about his health. He chose "bewildered"; and, indeed, bewilderment characterised his slow decline over the next five years. By far the most important part of his care was the loving support he received from a carer, who helped our family to cope.

We briefly considered the possibility of drug treatment at one point. The particular drug that we looked at caused diarrhoea in some patients, and that was not going to be welcome in someone who was already incontinent of urine and faeces. But the main problem was that we couldn't interpret the outcome measures from research studies (often scales of some sort). Although sufficient to secure drug marketing licences they had little practical meaning in my father's bewildered life.

In a paper published in 2001 Julia Cream and Harry Cayton of the Alzheimer's Society drew attention to this problem—the mismatch between the outcome measures used by researchers assessing the effects of drugs or other interventions for dementia and the manifestations of dementia that were rated most important to people with the condition and those caring for them.

This mismatch was probably one of the important factors in prompting the Alzheimer's Society to launch its "quality research in dementia" programme, in which patients and carers were integrated into the charity's decision making processes. The Alzheimer's Society was the first large medical research charity in the United Kingdom to do this, and because I have a longstanding interest in promoting such patient and carer involvement, I regarded the society's initiative as a beacon to other funders of research.

I suppose that it is particularly because I used to regard the society as a trail blazer that I have been disappointed by its recent



Challenging NICE: Neil Hunt, Alzheimer's Society chief executive (centre), outside the High Court

alliance with drug manufacturers in bringing the first legal challenge to judgments reached by the National Institute for Health and Clinical Excellence (NICE). On 10 August Justice Linda Dobbs ruled against the alliance of the Alzheimer's Society and drug manufacturers on five of the six charges they had brought against NICE (*BMJ* 2007;335:319).

NICE is an independent body established to tackle the difficult—some would say near impossible—but essential task of trying to judge how the resources of the NHS can be used effectively and equitably in a service built on the principles of shared risk and social solidarity. This process is bound to result in "winners" and "losers," but those like the Alzheimer's Society that now regard themselves as losers must make it clearer whether they support the principles upon which NICE was established.

I find it disturbing that in response to the legal judgment, Neil Hunt, the society's chief executive, stated, "NICE failed to listen to the views of thousands of carers who told them drug treatments make a huge difference to their lives. It is deeply disturbing that a public body, required to use rigorous standards of evidence based decision making, can simply guess at vital data. This is simply unacceptable . . . To retain its authority as a public body it must command the confidence of the public. The result of this case must call into question whether NICE has lost that confidence."

But how might the Alzheimer's Society's close alliance with drug manufacturers erode its own authority as a charity subsidised by the public—which thus also needs to retain the public's confidence? The society could take the following steps to restore my respect for and confidence in it.

Firstly, it should declare clearly on its website the sources and amounts of support it receives for its work.

Secondly, having challenged NICE's judgments about the most effective use of NHS resources for the care of people with dementia, the society should make clear what alternative distribution of limited resources it regards as more appropriate—and why.

Thirdly, it should campaign for treatments for dementia to be evaluated using outcomes that are meaningful to patients and carers, and challenge the licensing and use in the NHS of any new drugs that fail to conform to these expectations.

Fourthly, it should insist that all data from clinical trials are published and that anonymised data for individual patients are made available, so that researchers can try to identify which patients are most likely to be helped by treatments and which patients are unlikely to benefit or may be harmed.

These steps would be more effective and enduring ways for the Alzheimer's Society to serve the interests of people with dementia and their carers than forming alliances with organisations with vested commercial interests to take a public body to court.

Iain Chalmers is coordinator, James Lind Initiative, Oxford OX2 7LG ichalmers@jameslindlibrary.org

Competing interests: The author is a member of the NICE research and development advisory committee and provided written evidence challenging the Alzheimer's Society's charge that NICE's assessment and consideration of the AD2000 study (of donepezil in Alzheimer's disease) was irrational.

"…the main problem was that we couldn't interpret the outcome measures from research studies (often scales of some sort). Although sufficient to secure drug marketing licenses, they had little practical meaning."

Iain Chalmers
National Institute of Clinical Exellence (NICE)
R&D Advisory Committee
BMJ 2007; 335: 400

Rating Scale Requirements in Interventional Studies

- To be meaningful and meet the purpose of their use in clinical trials, rating scales need to represent
  - rigorous *measures* of the variables they intend to quantify
  - the variables that therapies are intended to target
  - *the variables that are important to people with PD*

Patient-Reported Outcome Measures

- Rating scales completed by patients themselves
- Often intended to provide patients with a voice in the decision making regarding effectiveness of therapies by assessing the impact of illness and therapy from *their* perspective

→ Need to be relevant to those who respond to them

- If not, they…

  …are unlikely to be valid representations of patients' perspectives

  …may discriminate against those to whom they are less relevant

  …may threat scientific validity and basic bioethical principles

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VALUE IN HEALTH

Whose Quality of Life? Ethical Implications in Patient-Reported Health Outcome Measurement

Peter Hagell, PhD,[1,2] Jan Reimer, RN,[2] Per Nyberg, PhD[1]

- 202 people with Parkinson's disease (PD)
- 3 health status/HRQL rating scales:
  - NHP (38 items; generic)
  - SF-36 (36 items; generic)
  - PDQ-39 (39 items; PD-specific)
- Rating of overall content relevance (1-10; 1=worst possible, 10=best possible)

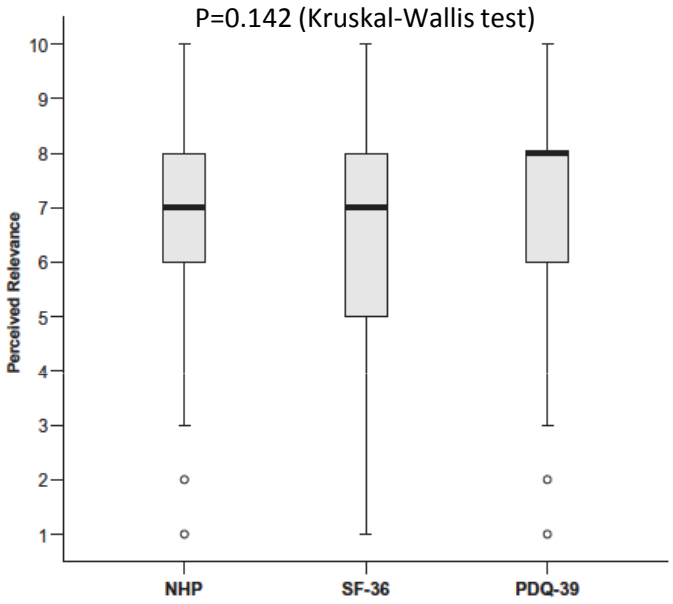


Table 4 Predictors of poor perceived relevance of contents in patient-reported health status questionnaires*

Dependent variable†	Predictors‡	Odds Ratio (95% CI)	*P*-value
NHP‖	—**	—	—
SF-36¶	Poor overall QoL	2.459 (1.074, 5.630)	0.033
	Poorer mental health	1.023 (1.004, 1.044)	0.020
PDQ-39#	Higher age	1.040 (1.004, 1.077)	0.031

IVs: Gender, Age, PD duration, Clinical and Perceived PD severity, Dyskinesias, Overall QoL, Respective summary scores

What outcomes are important to assess in Parkinson's disease from an end-user perspective?

Qualitative Literature Review

- PD impact from the patients' perspective
  - Independence in daily life
  - Walking/mobility
  - Lack of energy/fatigue
  - Unpredictability (due to fluctuating drug responses and dyskinesias)
  - Communication

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journal homepage: www.elsevier.com/locate/parkreldis



Short communication

Motor and non-motor predictors of illness-related distress in Parkinson's disease

Catharina Sjödahl Hammarlund [a], Peter Hagell [a,b,c,*], Maria H. Nilsson [a]

[a] Department of Health Sciences, Lund University, Lund, Sweden
[b] Department of Neurology, Skane University Hospital, Lund, Sweden
[c] School of Health and Society, Kristianstad University, Elmetorpsvägen 15, SE-291 88 Kristianstad, Sweden

Table 2
Multiple linear regression models with distress (NHPD score) as dependent variable among people with Parkinson's disease.[a]

Significant independent variables[b]	B (95% CI)	β	*P*-value	Adjusted R^2	
				Stepwise change	Cumulative
Model 1: Non-motor aspects[c]					
Age	−0.039 (−0.192 0.114)	−0.025	0.613	0.048	0.048
Fatigue (FACIT-F)	0.468 (0.284, 0.652)	0.310	<0.001	0.473	0.521
Pain (NHP Pain)	0.212 (0.154, 0.270)	0.364	<0.001	0.153	0.674
Sleep quality (PSQI)	1.029 (0.633, 1.426)	0.269	<0.001	0.073	0.747
Depression (HADS)	1.312 (0.718, 1.905)	0.252	<0.001	0.055	0.802
Anxiety (HADS)	0.473 (0.007, 0.940)	0.119	0.047	0.006	0.808
Model 2: Motor aspects[d]					
Age	−0.028 (−0.306 0.250)	−0.020	0.840	0.037	0.037
Mobility (NHP Mobility)	0.357 (0.233, 0.482)	0.577	<0.001	0.270	0.307

[a] All models controlled for age.
[b] Listed by order of entry into the models (forward method).
[c] IVs: PD duration (years), Cognition, Fatigue, Daytime sleepiness, Depression, Anxiety, Pain, Sleep quality, Motivation, Psychiatric complications.
[d] IVs: Parkinsonism, Mobility, Dyskinesias, Motor fluctuations, Falls and/or balance problems.

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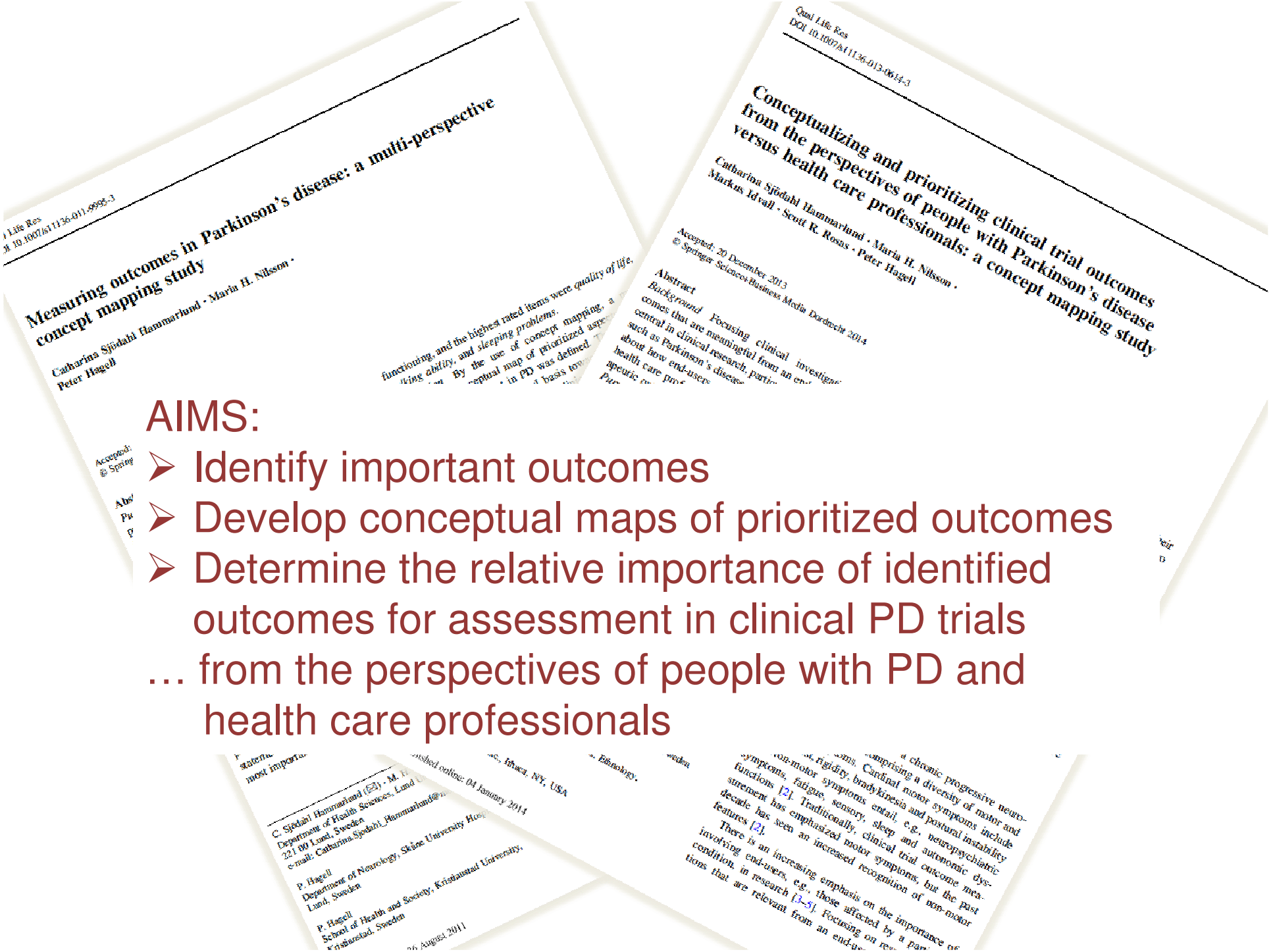
Measurement of patient-centered outcomes in Parkinson's disease: What do patients really want from their treatment?☆

Anne N. Nisenzon [a,*], Michael E. Robinson [a], Dawn Bowers [a], Evangelia Banou [a], Irene Malaty [b], Michael S. Okun [b]

[a] University of Florida, Department of Clinical and Health Psychology, Health Science Center, P.O. Box 100165, Gainesville, FL 32610-0165, USA
[b] University of Florida, Movement Disorders Center, McKnight Brain Institute, Department of Neurology, Gainesville, FL, USA

	Domain	N	Mean	Std. Deviation
	Importance			
10	Pain	135	46.44	42.32
4	Fatigue	135	57.18	36.05
9	Emotional Distress	135	51.88	39.50
3	ADL[a]	134	57.91	37.98
8	Tremor	133	52.02	40.67
5	Stiffness	135	57.10	37.81
2	Slowness	134	60.06	34.60
1	Walking	135	62.24	37.07
7	Thinking	133	54.71	41.50
6	Sleep	134	56.01	40.23

Limited by pre-defined areas/independent variables…



AIMS:

- Identify important outcomes
- Develop conceptual maps of prioritized outcomes
- Determine the relative importance of identified outcomes for assessment in clinical PD trials

… from the perspectives of people with PD and health care professionals

Group Concept Mapping

Qualitative

Quantitative

Patients – Clinicians – Researchers



"A concrete example of what is most important to assess when treating PD, regardless of whether there is such a treatment available or not, is…"





freezing	gångförmåga
medrörelser i armarna	fall
smärta	svängningar (motoriskt / mentalt off)
överrörlighet	svårt att äta (svälja och tugga ev finmotorik)
kramper	stickningar (i fingrarna)
svårt att andas	rörlighet
benskörhet	balans
talproblem (sluddrigare tal, svagare röst)	mimikfattigdom
förmåga att uppfatta vad folk säger	muntorrhet
värk	sväljförmåga
viktförändring	tvångsmässiga upprepade handlingar
aspirationsbesvär	lågt blodtryck
stelhet	kroppshållning
ökad salivering (dregla)	muskelsvaghet
vända sig i sängen	uppresning
nedstämdhet	inkontinens
lukt	skakningar
frustration	blodtrycksfall
inre darmningar	finmotorik
psykiskt välbefinnande	erektion
skrivsvårigheter	smak
motorisk långsamhet	svettningar
mardrömmar	sömnproblem
yrsel	svindlingskänsla
förstoppning	trångningar
somna mitt på dagen	impotens
reaktionsförmåga	känslomässig balans
oro	social förmåga
håglöshet	depression
skamkänsla	matt/ orkeslös/fatigue
omdöme	planera
insikt	distanslöshet
ångest	stresskänslighet
aggressivitet	initiativförmåga
trötthet	företagsamhet
handlingsförmåga	socialt umgänge
simultanförmåga	minne
organisera	hitta strategier



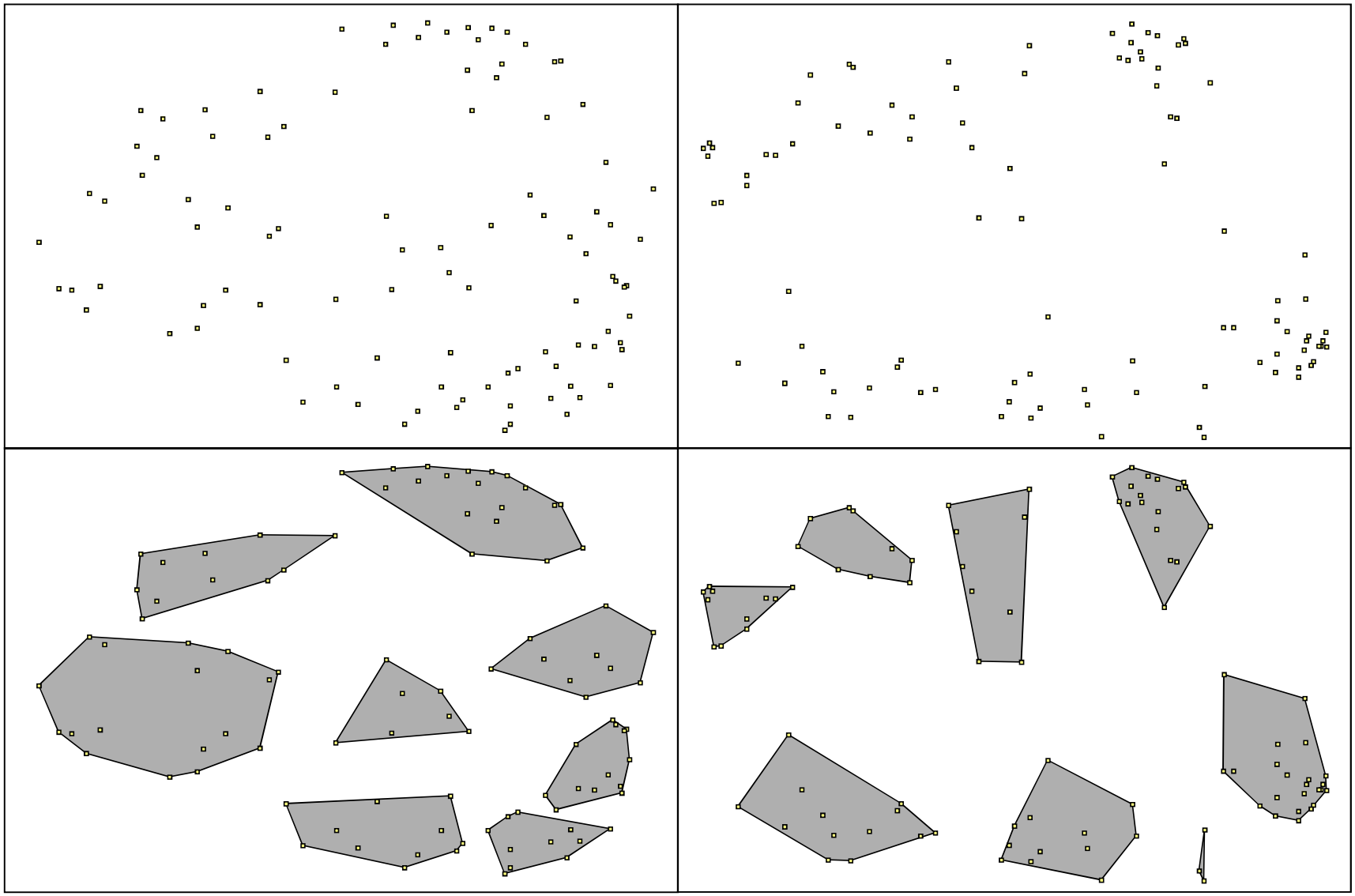


→ 99 statements (outcomes)

People with PD vs. Health Care Professionals

People with PD

Health Care Professionals



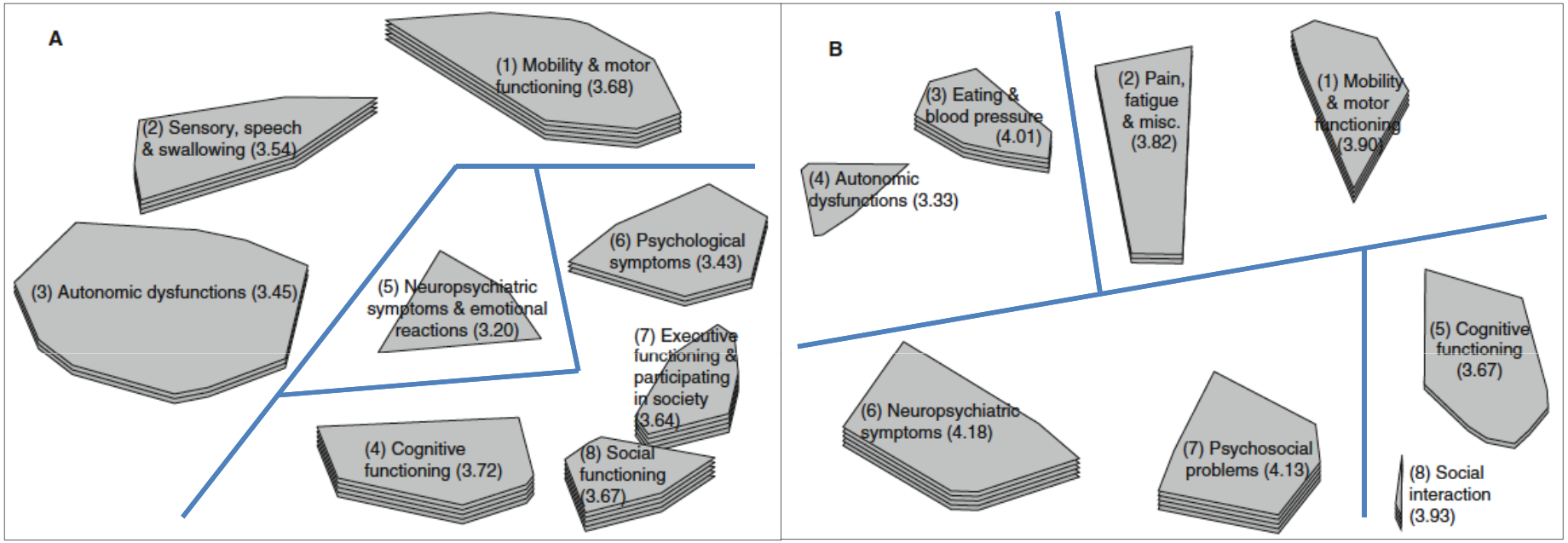
Heterogeneous and unidimensional

Homogeneous and multidimensional

People with PD vs. Health Care Professionals

People with PD

Health Care Professionals



Highest Importance-Rated Statements

People with PD	Clinicians
Quality of life	Quality of life
Walking	Depression
Mobility	Fluctuations
Psychological well-being	Walking
Sleep problems	Sleep problems
Control over disease process	Falling

- Despite overall similarities, patient perspectives differ from that of clinicans'
  - Lived experience vs. scholarly/biomedical perspective

- End-user prioritized outcomes
  - Quality of life, walking/mobility, sleep, fatigue, perceived disease control; fluctuations, depression, falls

  →Evidence from clinical trials that are more meaningful and interpretable for end-users

- Implications
  - Needs for new and refined outcome measures
  - Priorities for therapeutic research