

A MODEST PROPOSAL: CAN WE EASE THE BURDEN OF MYRIAD MEASURES WITH A MINIMUM DATA SET THAT ACTUALLY MATTERS TO PATIENTS?

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The presenter is an employee of Pfizer Inc..

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A day in the life....

- **The Case**
 - Kidney Disease example
- **The Challenge**
 - What matters most to patients?
 - Is there a common intersection on what to assess?
 - Patients – Clinicians – Regulators – Payers
 - Aligned incentives?
- **Solutions**
 - Parsimony, Consistency, Transparency



KD & Renal Lit Search: non-Pharmacological Primary & Secondary Endpoints

Among the 92 non-pharmacological studies, **primary endpoints** were assessed by:

- **Patient-Reported COAs (75)**
 - Kidney Disease Quality of Life Instrument (KDQOL) (n=49)
 - EQ-5D (n=8)
 - MOS SF-36 (n=6)
 - SF-6D (n=6)
 - Short Form 12 (SF-12) (n=1)
 - Hospital Anxiety and Depression Scale (n=2)
 - Kidney Disease Component Summary (n=1)
 - Pittsburgh Sleep Quality Index (PSQI) (n=1)
 - Kidney Transplant Questionnaire (TKQ) (n=1)
- **Functional assessments (6)**
 - Two minute walk test (2MWT) (n=2)
 - Timed-up-and-go (TUG) (n=2)
 - Six minute walk distance (6MWD) (n=1)
 - Peak O2 capacity (n=1)
- **Clinical assessments (3)**
 - Serum albumin level (n=1)
 - Serum glucose level (n=1)
 - Cholesterol level (n=1)

Among the 92 studies non-pharmacological studies, **secondary endpoints** were assessed by:

- **Clinical assessments (17)**
 - Hemoglobin level (n=7)
 - Serum albumin level (n=5)
 - Cholesterol level (n=2)
 - Urea clearance (n=2)
 - Protein catabolic rate (n=1)
- **Patient-reported COAs (12)**
 - Kidney Disease Quality of Life Instrument (KDQOL) (n=9)
 - MOS SF-36 (n=1)
 - Temperament and Character Inventory (n=1)
 - Coping Inventory for Stressful Situations (n=1)
- **Functional Assessments (1)**
 - Six minute walk distance (6MWD) (n=1)



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KD & Renal Primary & Secondary Endpoints in CT.gov

Among the 24 trials with a COA measure identified, **primary endpoints** were assessed by:

- **Patient-Reported COAs (15)**
 - MOS SF-36 (n=6)
 - Kidney Disease Quality of Life Measure (KDQOL) (n=5)
 - CDC Activity Limitations Module (n=1)
 - Hopkins Symptom Checklist (n=1)
 - Miller Social Intimacy Scale (n=1)
 - Duke's Activity Scale Index (n=1)
- **Clinical assessments (11)**
 - Change in serum creatinine (n=3)
 - Reduction in eGFR (n=2)
 - Change in kidney volume (n=2)
 - Rate of kidney enlargement (n=1)
 - Proportion of patient with adequate dialysis measurement (n=1)
 - Six minute walk test (6MWD) (n=1)
 - Change in left ventricular mass (n=1)

Among the 24 trials with a COA measure identified, **secondary endpoints** were assessed by:

- **Patient-reported COAs (17)**
 - Kidney Disease Quality of Life Measure (KDQOL) (n=6)
 - EQ-5D (n=3)
 - MOS SF-36 (n=2)
 - SF-12 (n=1)
 - Global rating of change scale (n=1)
 - FACIT Fatigue Scale (n=1)
 - Sleep Quality Index (PSQI) (n=1)
 - MOS Sleep Questionnaire (n=1)
 - Pediatric Quality of Life Inventory (PedsQL) (n=1)
- **Clinical assessments (2)**
 - Hospitalizations (n=2)



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How'bout we ask our patients?

What matters most to you in treatment outcomes?

What do you value?

What to measure?

How to measure?

Patient-centered outcomes measurement is a key element in value-based health care.

Absence of patient-reported health status in core data sets fundamentally undermines value assessment.



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The What and The Why....

What is a core or minimum data set?
Why do this?

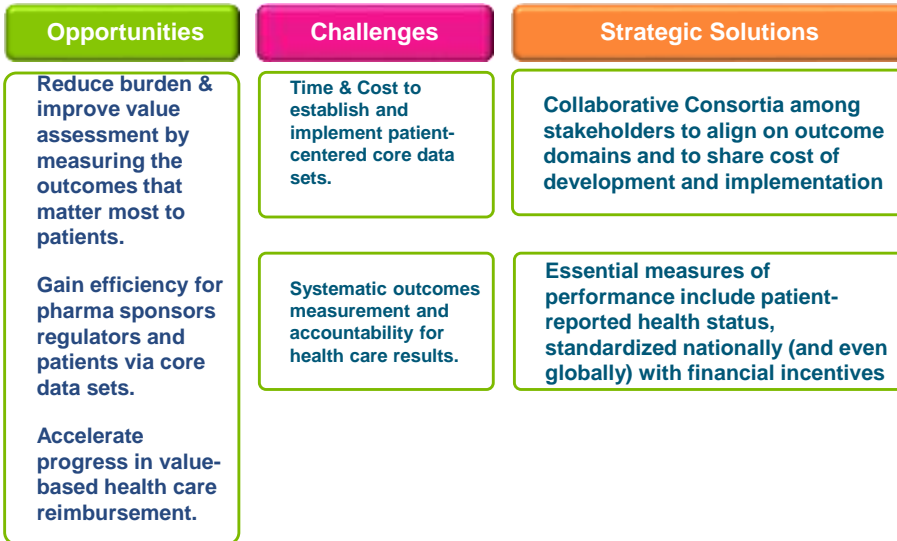
- **Core Data Set:** an agreed minimum set of outcomes measured and reported in all clinical trials of a specific disease condition
- **Why? ...what are the basic benefits of doing so?**
 - Common measures across studies readily enables comparisons across clinical trials (ie, product differentiation and value appraisal)
 - Reduces uncertainty and improves efficiency for sponsors
 - Including patient input into endpoint selection (and study design) ensures that outcomes are patient relevant and studies produce results meaningful to patients as well as health care providers, regulators, payers and HTA authorities
 - Other???



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“To everything there is a season, and a time for every purpose....”



ICHOM Example

International Consortium for Health Outcomes Measurement's Standardized Outcome Sets.*			
Standard Sets Complete			Under Consideration for 2016 and Beyond
2013	2014	2015 (Final Approval Pending)	
1. Localized prostate cancer	5. Parkinson's disease	13. Breast cancer	22. End-stage renal failure
2. Lower back pain	6. Cleft lip and palate	14. Dementia	23. Oral health
3. Coronary artery disease	7. Stroke	15. Frail elderly	24. Brain tumors
4. Cataracts	8. Hip and knee osteoarthritis	16. Heart failure	25. Drug and alcohol addiction
	9. Macular degeneration	17. Pregnancy and childbirth	26. Bipolar disorder
	10. Lung cancer	18. Colorectal cancer	27. Burns
	11. Depression and anxiety	19. Overactive bladder	28. Melanoma
	12. Advanced prostate cancer	20. Craniofacial microsomia	29. Head and neck cancer
		21. Inflammatory bowel disease	30. Pediatric oncology (conditions to be determined)
			31. Rheumatoid arthritis
			32. Liver transplantation
			33. Congenital hand malformations
			34. Chronic rhinosinusitis
			35. Congenital hemolytic anemia
			36. Rotator cuff disease
			37. Malaria

* The standard outcomes sets completed or pending in the first 3 years cover conditions accounting for 45% of the global burden of disease.

Table Extracted From: Porter, Larsson & Lee. Standardizing Outcomes Measurement. NEJM, 2016; 374; 6.

