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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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)

**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 (12)**
  - Kidney Disease Quality of Life Measure (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)

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)
A Modest Proposal…

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.

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

**Opportunities**
- Reduce burden & improve value assessment by measuring the outcomes that matter most to patients.
- Gain efficiency for pharma sponsors regulators and patients via core data sets.
- Accelerate progress in value-based health care reimbursement.

**Challenges**
- Time & Cost to establish and implement patient-centered core data sets.

**Strategic Solutions**
- Collaborative Consortia among stakeholders to align on outcome domains and to share cost of development and implementation.
- Systematic outcomes measurement and accountability for health care results.
- Essential measures of performance include patient-reported health status, standardized nationally (and even globally) with financial incentives.

**ICHOM Example**