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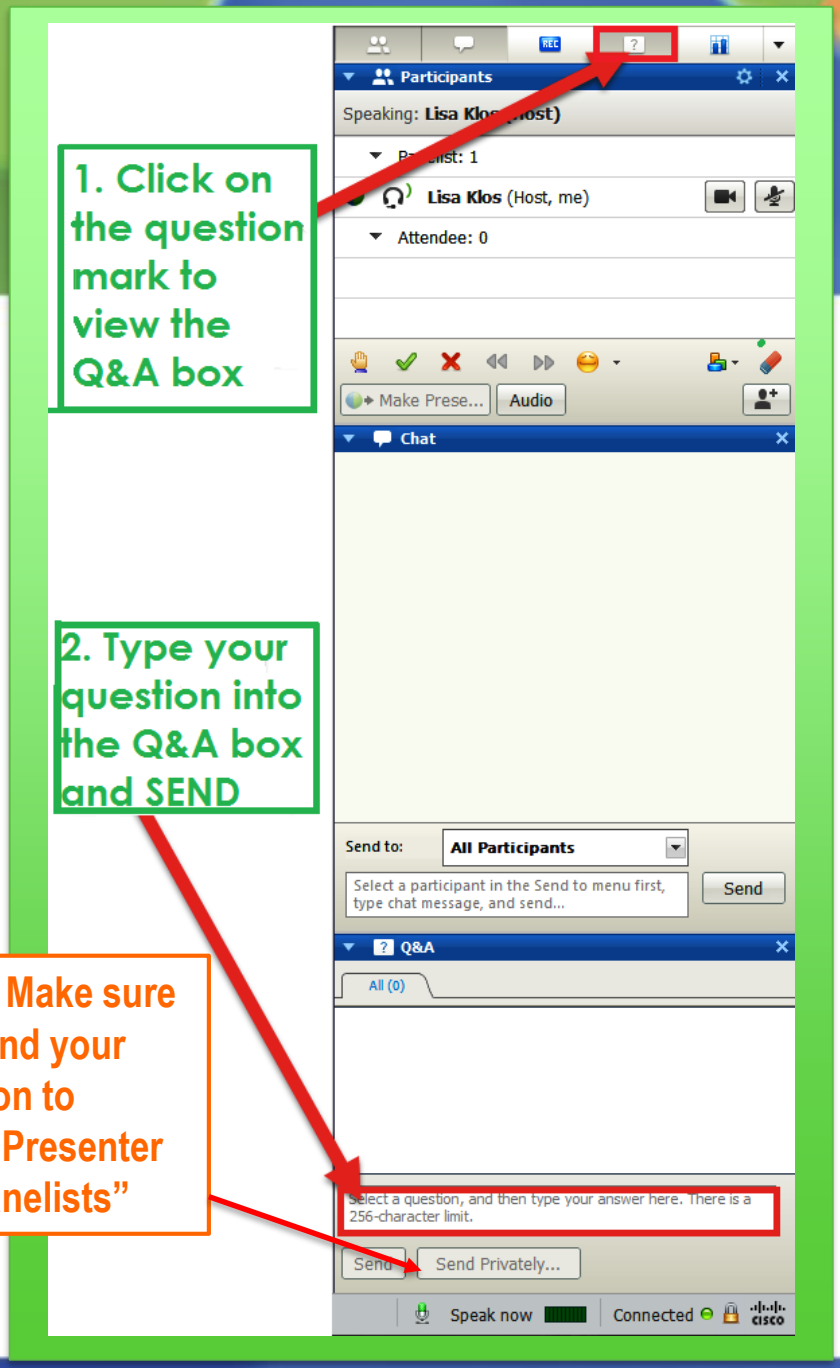
- To ask a question, please type your query into the Q&A box
  - To ensure anonymity and that all panelists receive your question, please choose the drop down box option, **"Hosts, Presenters and Panelists."** Otherwise, all audience members will be able to see your submitted question
- We will make every effort to respond to all questions



PATIENT INSIGHT, PROS AND PATIENT ENGAGEMENT – WHAT ARE THE OPPORTUNITIES FOR PUBLICATIONS DEVELOPMENT?

Richard White MA PhD
Lizzie Perdeaux MA MPhil PhD

Oxford PharmaGenesis

DONNA SIMCOE
… A BIT ABOUT ME

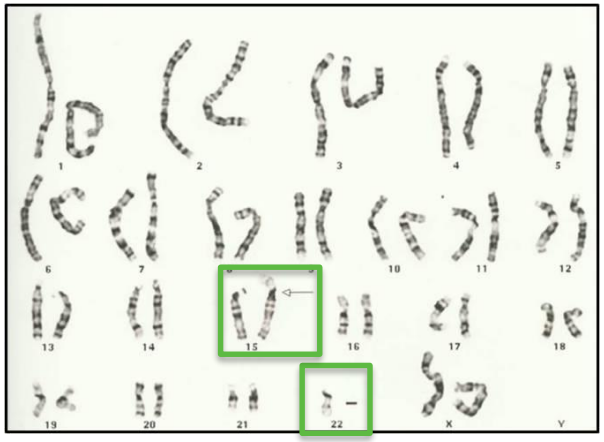
- Background
  - Managed publications for over 20 years
  - Holds three master's degrees (in Biomedical Writing, Biotechnology and an MBA)
  - ISMPP Certified Medical Publication Professional™ (CMPP)
  - Active member of AMWA, ISMPP and TIPPA
  - Medical Publications consultant and principal at Simcoe Consultants, Inc., a biomedical company focusing on medical publication development and medical writing.
- Patient
  - Mitral valve prolapse

RICHARD WHITE
… A BIT ABOUT ME

- Background
  - MA, PhD Pharmacology (Cambridge)
  - Training in Marketing (INSEAD) and Health Economics (Oxford)
  - Honorary Research Fellow (Oxford Brookes University)
- Oxford PharmaGenesis
  - Publication planning for major brand launches
  - Founder of the Value Demonstration Practice
    - Publications, communications and training in HEOR, RWE and PROs
  - Invited presentations on HEOR, RWE and PRO publications at TIPPA and ISMPP
- Patient
  - Severe hearing loss and tinnitus (ringing in the ears)

LIZZIE PERDEAUX … A BIT ABOUT ME

- Background
  - MA, MPhil, PhD Genetics (Cambridge)
  - Post-doctoral Research Fellow (Institute of Cancer Research, London)
  - Charity officer at the Myrovlytis Trust, a patient-support charity
- Oxford PharmaGenesis
  - Medical Writer since January 2015
  - Member of the Patient Engagement Practice
- Patient/carer
  - Robertsonian Translocation t(15;22)(q10;q10)
  - Dad was diagnosed with early-onset Alzheimer's at the age of 58 years



DISCLAIMER

- Information presented reflects the personal knowledge and opinion of the presenters and does not necessarily represent the position of their current or past employers or the position of ISMPP

WHAT YOU SHOULD TAKE AWAY FROM THIS PRESENTATION …

Confidence

- Not being afraid of the jargon around PROs
- Identifying the publications opportunities provided by PROs
- Understanding what health literacy is, and what patients understand
- Knowing how you can be patient-centric in your role

AUDIENCE QUESTION

Describe your current level of confidence in PROs and patient engagement

A. Very confident – high level of experience in this area
B. Quite confident – I'm no expert but I know enough
C. Not very confident – I have some understanding but would like to know more
D. Not confident at all – I really don't know very much about this area
E. Unsure – this stuff isn't relevant to my role

WHY PATIENT-REPORTED OUTCOMES (PROS) ARE IMPORTANT

BEING PATIENT-CENTRIC: WHY DO PHYSICIANS TREAT PATIENTS?

- Treatment is offered to patients to:
  - increase longevity
  - prevent future morbidity
  - make them feel better
- 'Feeling better' includes avoiding:
  - discomfort (e.g. pain, nausea, breathlessness)
  - disability (i.e. loss of function)
  - distress (i.e. emotional problems)

- 'Feeling better' is a subjective assessment that cannot necessarily be measured by a physician using traditional endpoints, such as:
  - clinical status (e.g. peak flow for patients with lung disease)
  - surrogate markers (e.g. bone density for patients prone to fractures)

WHAT IS A PATIENT-REPORTED OUTCOME?

- Food and Drug Administration (FDA) definition of a PRO:

  "A measurement based on a report that comes directly from the patient (i.e. study subject) about the status of a patient's health condition without amendment or interpretation of the patient's response by a clinician or anyone else"[1]

- PRO measures are usually questionnaires and can be categorized as:

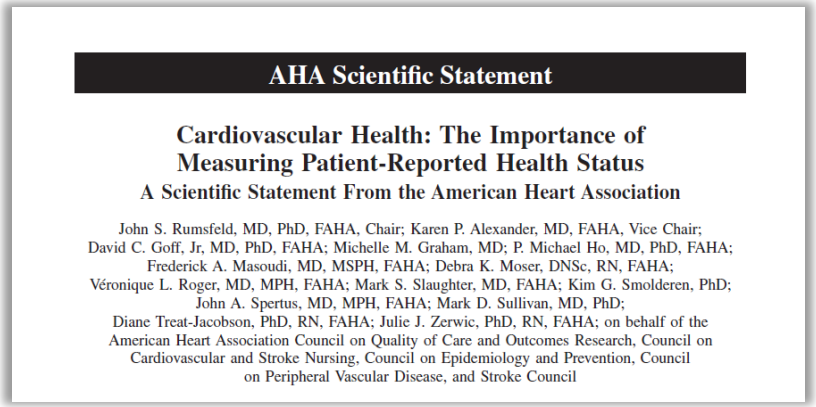
Generic HRQoL
- SF-36, SF-12
- EQ-5D
- HUI-2, HUI-3

Symptom-specific
- Fatigue Severity Scale

Disease-specific
- Asthma Quality of Life Questionnaire

- Also clinician-reported outcomes and observer-reported outcomes (out of scope for today)

ALL MAJOR STAKEHOLDERS ARE INCREASINGLY INTERESTED IN PRO DATA



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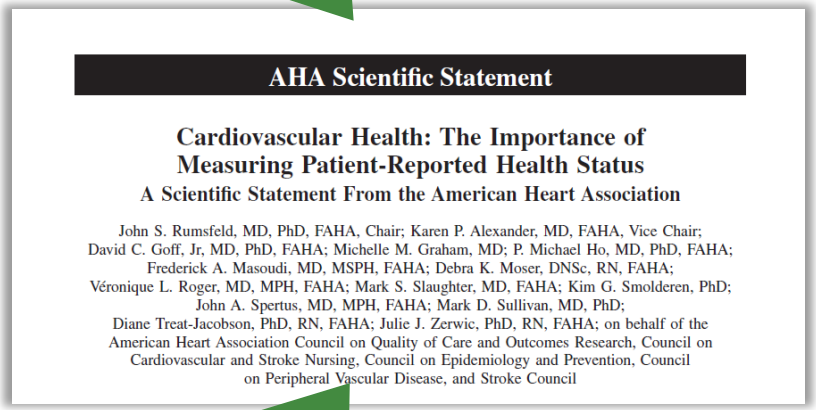

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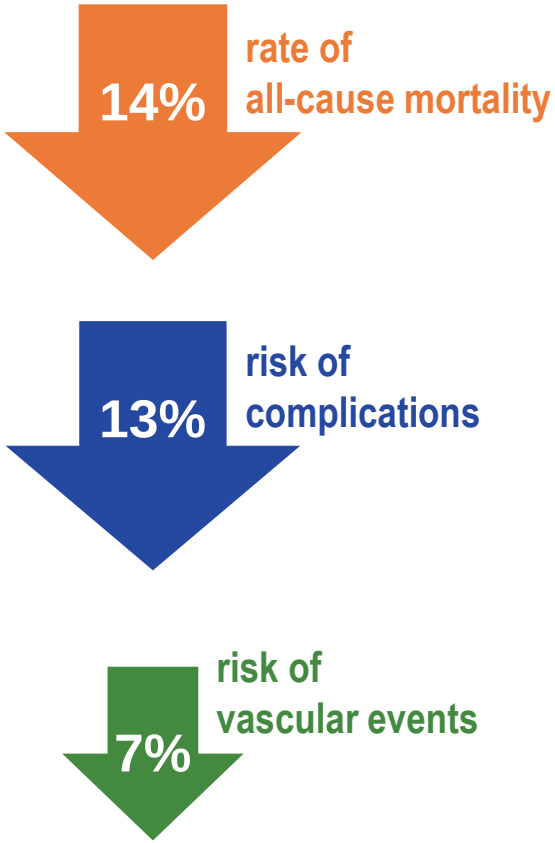
"Cancer drugs often carry substantial toxicities that may affect how people feel and function … it seems counter-intuitive that PRO end points are not central in the evaluation of cancer drugs"[3]

IMPORTANCE OF PRO MEASURES (1): THEY PREDICT 'HARD' PATIENT OUTCOMES

- FIELD (Fenofibrate Intervention and Event Lowering in Diabetes)
  - 5-year cohort study of 7348 patients with type 2 diabetes, aged 50–75 years
  - Multivariate analysis of baseline predictors of risk in the trial, correcting for multiple factors
  - EQ-5D is a general PRO measure **from 0 (death) to 1 (perfect health**)

"Index scores derived from the EQ-5D are an independent predictor of the risk of mortality, future vascular events, and other complications in people with type 2 diabetes"

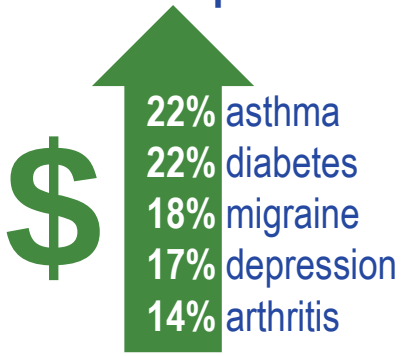
An EQ-5D score 0.1 points higher was associated with:



IMPORTANCE OF PRO MEASURES (2): THEY PREDICT PATIENT MEDICAL COSTS

- Medical Expenditures Panel Survey (MEPS; n = 20 624) data 2006–2007
- Medical expenditures (prescription medicines, hospital inpatient, ER, out-patient and office-based provider visits) in the 6 months following administration of the SF-12
- SF-12 is a general PRO measure **from 0–100, higher scores = better health**
- Model effect of physical (PCS) and mental (MCS) component summary scores correcting for age, sex, marital status, comorbidities count and insurance status

A 5-point lower PCS score was associated with an increase in medical expenditures



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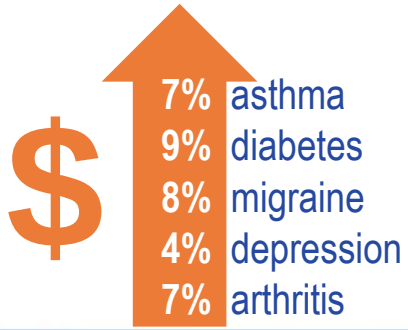
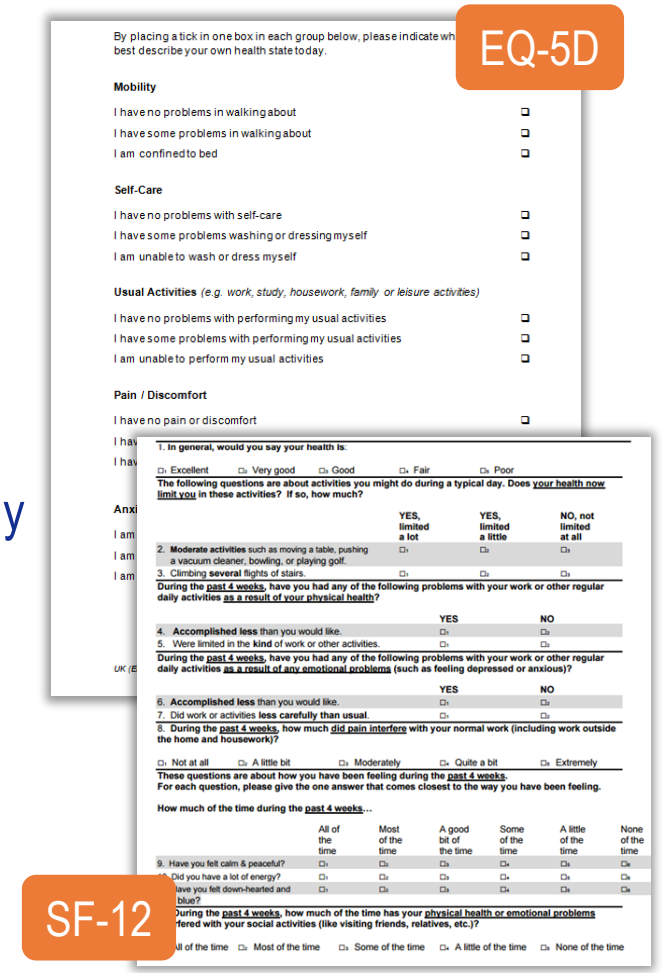


WHAT ARE PROS AND WHAT DO THEY DO?

GENERIC PRO MEASURES: 'OFF-THE-SHELF' TOOLS TO MEASURE HRQOL

- Examples of generic PRO instruments
  - EQ-5D, SF-36, SF-12, HUI-2, HUI-3
- Allow comparisons across:
  - different patient groups
  - different disease types
- Can be used for comparison with data in published studies
- Do not require development and refining before a study can commence
- More familiar to stakeholders
  - EQ-5D (essentially) gives a utility value between 0 and 1



AN EXAMPLE UTILITY CALCULATION USING THE EQ-5D (UK TARIFF)

Attribute	Level	Me, today
Mobility	1. No problems 2. Some problems 3. Confined to bed	**1**
Self-care	1. No problems 2. Some problems 3. Unable to	**1**
Usual activities	1. No problems 2. Some problems 3. Unable to	**1**
Pain/discomfort	1. None 2. Moderate 3. Extreme	**1**
Anxiety/depression	1. None 2. Moderate 3. Extreme	**2**
	Calculated utility	**0.85**

AN EXAMPLE UTILITY CALCULATION USING THE EQ-5D (UK TARIFF)

Attribute	Level	Me, today	Me, last week
Mobility	1. No problems 2. Some problems 3. Confined to bed	**1**	**2**
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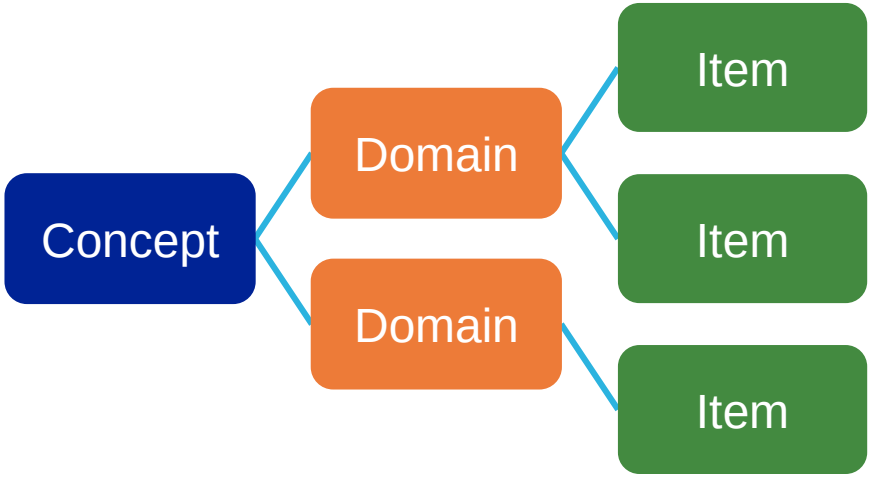
Pain/discomfort	1. None 2. Moderate 3. Extreme	**1**	**2**
Anxiety/depression	1. None 2. Moderate 3. Extreme	**2**	**2**
	Calculated utility	**0.85**	**0.62**

DISEASE-SPECIFIC PRO MEASURES: SOME KEY TERMINOLOGY

- **Conceptual framework**
  - Provides a picture of the relationships between items in a PRO instrument and the concepts measured by that instrument



- **Concept**: what is being measured
  - e.g. arthritis symptoms
- **Domain**: a sub-concept of the overall concept being measured
  - e.g. fine motor skills of the hand
- **Item**: an individual question that is evaluated by the patient
  - e.g. do you have difficulty moving your fingers/making a fist/ picking up objects?

DEVELOPMENT OF A DISEASE-SPECIFIC PRO MEASURE

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review

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	What do we need to do?	How do we do it?	
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2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews	

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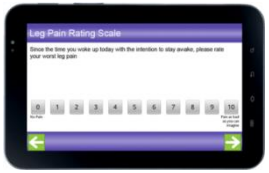
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs	

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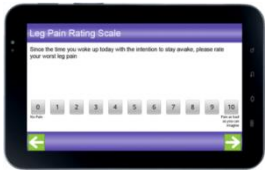
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs	
5	Modify PRO measure for wider usage	Cultural adaptations, translations, evaluations in related diseases	

PATIENT-REPORTED OUTCOMES: THE TERMINOLOGY MADE SIMPLE

COMMUNICATING EFFECTIVELY MEANS EXPLAINING THE TECHNICAL JARGON

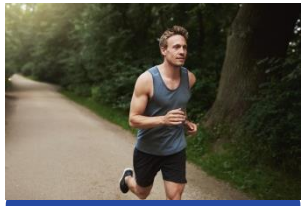
COMMUNICATING EFFECTIVELY MEANS EXPLAINING THE TECHNICAL JARGON

PRO SCALES – AN ANALOGY…

PRO SCALES – AN ANALOGY… AND A SIMPLE CONCEPTUAL MODEL

- Conceptual model – *a set of domains, as defined by patients and physicians, that determines the overall concept [body weight]*

Calorie intake



Physical activity

Body composition

Metabolic status

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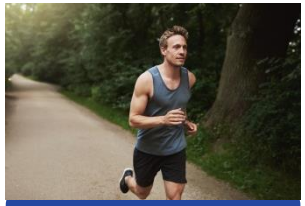


Physical activity

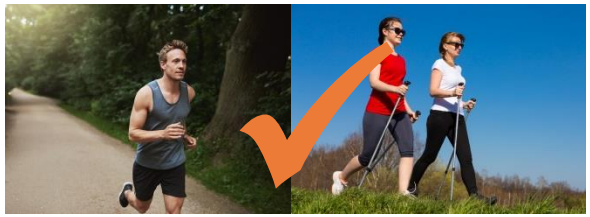
Body composition

Metabolic status

- Content validity – *does the scale contain everything about the concept [body weight] that is relevant to patients, physicians etc.?*
  - Interviews yield different themes; when no more new themes are uncovered ('saturation'), content is likely to be valid

WHAT DO WE EXPECT FROM A GOOD SCALE? RELIABILITY

- Reliability – *does it measure the concept [body weight] in a reproducible way?*
  - **Internal consistency**
    - Items within a domain should correlate with each other and with the total score
    - *Cronbach's alpha > 0.70 between elements in the same domain indicates internal reliability*



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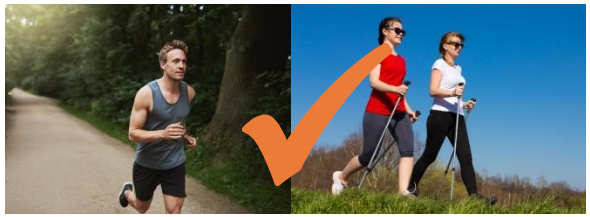
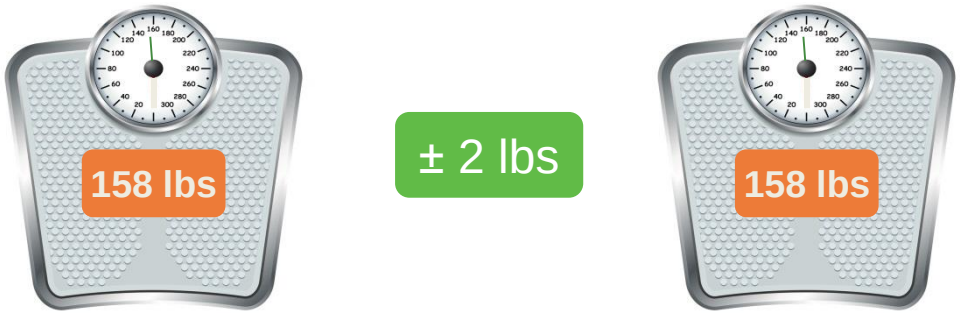


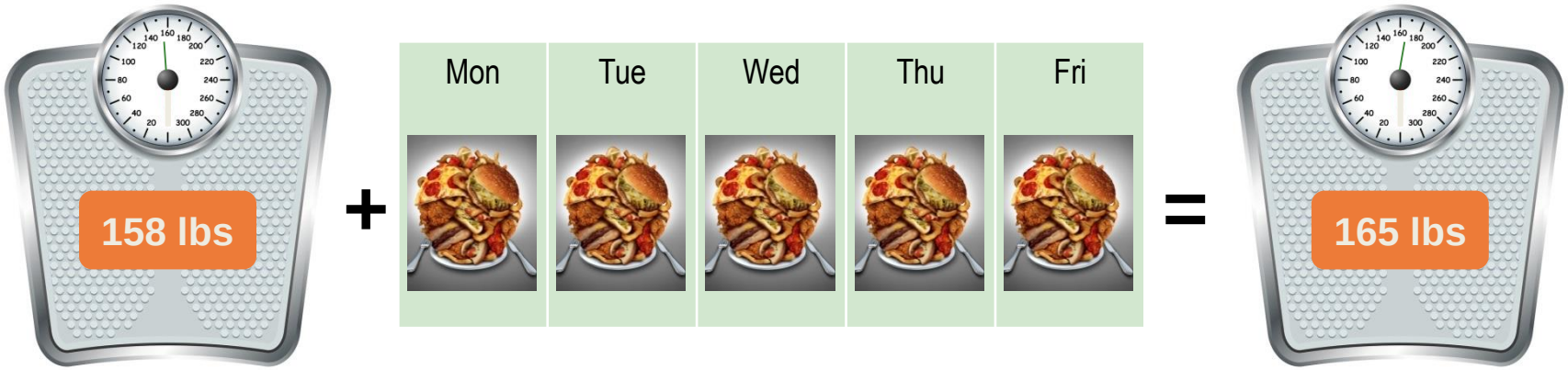
  - **Test–retest reliability**
    - Where nothing has changed in the subject, the scale should give the same result over time, when tested and retested after a reasonable interval (e.g. 2 weeks)



    - *Test–retest coefficient > 0.70 indicates good test–retest reliability*

WHAT DO WE EXPECT FROM A GOOD SCALE? RESPONSIVENESS

- Responsiveness – *does it detect meaningful changes [in body weight]?*
  - When a meaningful change happens, the scale should be able to detect it



  - *Effect size (mean difference ÷ SD baseline score)*
    - *over 0.8 is considered large*
    - *0.5–0.8 is considered clinically meaningful*
    - *0.2–0.5 is considered small*

HOW DO WE KNOW A SCALE IS MEASURING WHAT IT SHOULD? PSYCHOMETRIC VALIDITY

- Construct validity – *does the scale actually measure what it is supposed to [body weight], and not something else?*
  - **Concurrent validity** – measurements from the scale should agree with other instruments that measure the same concept [body weight]

HOW DO WE KNOW A SCALE IS MEASURING WHAT IT SHOULD? PSYCHOMETRIC VALIDITY

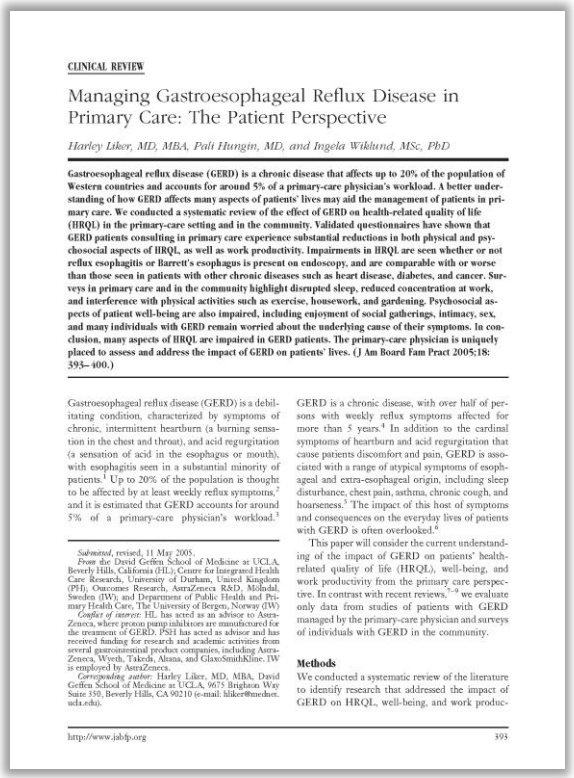
- Construct validity – *does the scale actually measure what it is supposed to [body weight], and not something else?*
  - **Concurrent validity** – measurements from the scale should agree with other instruments that measure the same concept [body weight]
  - **Known-groups validity** – the scale should show differences [in body weight] between patient groups known to be different
  - *Pearson correlation coefficients*
    - *over 0.6 indicates a strong correlation*
    - *0.3–0.6 indicates a moderate correlation*
    - *below 0.3 indicates a low correlation*

PATIENT-REPORTED OUTCOMES: PUBLICATION OPPORTUNITIES

PRO MEASURES OFFER A WIDE RANGE OF PUBLICATIONS OPPORTUNITIES (1/5)

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review
2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews
3	Confirm conceptual framework and assess properties of PRO measure	Validation study in relevant patient samples
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs
5	Modify PRO measure for wider usage	Cultural adaptations, translations, evaluations in related diseases

JABFM
JOURNAL OF THE AMERICAN BOARD OF FAMILY MEDICINE



CLINICAL REVIEW

Managing Gastroesophageal Reflux Disease in Primary Care: The Patient Perspective

Harley Liker, MD, MBA, Pali Hungin, MD, and Ingela Wiklund, MSc, PhD

Gastroesophageal reflux disease (GERD) is a chronic disease that affects up to 20% of the population of Western countries and accounts for around 5% of a primary-care physician's workload. A better understanding of how GERD affects many aspects of patients' lives may aid the management of patients in primary care. We conducted a systematic review of the effect of GERD on health-related quality of life (HRQL) in the primary-care setting and in the community. Validated questionnaires have shown that GERD patients consulting in primary care experience substantial reductions in both physical and psychosocial aspects of HRQL, as well as work productivity. Impairments in HRQL are seen whether or not reflux esophagitis or Barrett's esophagus is present on endoscopy, and are comparable with or worse than those seen in patients with other chronic diseases such as heart disease, diabetes, and cancer. Surveys in primary care and in the community highlight disrupted sleep, reduced concentration at work, and interference with physical activities such as exercise, housework, and gardening. Psychosocial aspects of patient well-being are also impaired, including enjoyment of social gatherings, intimacy, sex, and many individuals with GERD remain worried about the underlying cause of their symptoms. In conclusion, many aspects of HRQL are impaired in GERD patients. The primary-care physician is uniquely placed to assess and address the impact of GERD on patients' lives. (J Am Board Fam Pract 2005;18: 393–400.)

Gastroesophageal reflux disease (GERD) is a debilitating condition, characterized by symptoms of chronic, intermittent heartburn (a burning sensation in the chest and throat), and acid regurgitation (a sensation of acid in the esophagus or mouth), with esophagitis seen in a substantial minority of patients.[1] Up to 20% of the population is thought to be affected by at least weekly reflux symptoms,[2] and it is estimated that GERD accounts for around 5% of a primary-care physician's workload.[3]

GERD is a chronic disease, with over half of persons with weekly reflux symptoms affected for more than 5 years.[4] In addition to the cardinal symptoms of heartburn and acid regurgitation that cause patients discomfort and pain, GERD is associated with a range of atypical symptoms of esophageal and extra-esophageal origin, including sleep disturbance, chest pain, asthma, chronic cough, and hoarseness.[5] The impact of this host of symptoms and consequences on the everyday lives of patients with GERD is often overlooked.[6]

This paper will consider the current understanding of the impact of GERD on patients' health-related quality of life (HRQL), well-being, and work productivity from the primary care perspective. In contrast with recent reviews,[7–9] we evaluate only data from studies of patients with GERD managed by the primary-care physician and surveys of individuals with GERD in the community.

Methods

We conducted a systematic review of the literature to identify research that addressed the impact of GERD on HRQL, well-being, and work produc-

Submitted, revised, 11 May 2005.
From the David Geffen School of Medicine at UCLA, Beverly Hills, California (HL); Centre for Integrated Health Care Research, University of Durham, United Kingdom (PH); Outcomes Research, AstraZeneca R&D, Mölndal, Sweden (IW); and Department of Public Health and Primary Health Care, The University of Bergen, Norway (IW)
Conflict of interest: HL has acted as an advisor to AstraZeneca, where proton pump inhibitors are manufactured for the treatment of GERD. PSH has acted as advisor and has received funding for research and academic activities from several gastrointestinal product companies, including AstraZeneca, Wyeth, Takeda, Ataxa, and GlaxoSmithKline. IW is employed by AstraZeneca.
Corresponding author: Harley Liker, MD, MBA, David Geffen School of Medicine at UCLA, 9673 Brighton Way Suite 350, Beverly Hills, CA 90210 (e-mail: hliker@mednet.ucla.edu).

PRO MEASURES OFFER A WIDE RANGE OF PUBLICATIONS OPPORTUNITIES (2/5)

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review
2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews
3	Confirm conceptual framework and assess properties of PRO measure	Validation study in relevant patient samples
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs
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Journal of the American Academy of Child & Adolescent Psychiatry

Eur Child Adolesc Psychiatry (2012) 21:87–99
DOI 10.1007/s00787-011-0235-8

ORIGINAL CONTRIBUTION

The decisions regarding ADHD management (DRAMa) study: uncertainties and complexities in assessment, diagnosis and treatment, from the clinician's point of view

Hanna Kovshoff · Sarah Williams · May Vrijens · Marina Danckaerts · Margaret Thompson · Lucy Yardley · Paul Hodgkins · Edmund J. S. Sonuga-Barke

Received: 17 June 2011 / Accepted: 6 December 2011 / Published online: 18 December 2011
© Springer-Verlag 2011

Abstract Clinical decision making is influenced by a range of factors and constitutes an inherently complex task. Here we present results from the decisions regarding ADHD management (DRAMa) study in which we undertook a thematic analysis of clinicians' experiences and clinicians spoke about the use of symptom thresholds or specific impairment criteria. Furthermore, systematic or operationalised criteria to assess treatment outcomes were rarely used. Decision making in ADHD is regarded as a complicated, time consuming process which requires

Matheson et al. BMC Health Services Research 2013, 13:184
http://www.biomedcentral.com/1472-6963/13/184

BMC Health Services Research

RESEARCH ARTICLE Open Access

Adult ADHD patient experiences of impairment, service provision and clinical management in England: a qualitative study

Lauren Matheson[1*], Philip Asherson[2], Ian Chi Kei Wong[3], Paul Hodgkins[4], Juliana Setyawan[4], Rahul Sasane[5] and Sarah Clifford[6]

Abstract

Background: There is limited evidence of the unmet needs and experiences of adults with Attention Deficit Hyperactivity Disorder (ADHD) in the published scientific literature. This study aimed to explore the experiences of adults in England with ADHD regarding access to diagnostic and treatment services, ADHD-related impairment and to compare experiences between patients diagnosed during adulthood and childhood.

Methods: In this qualitative study, 30 adults with ADHD were recruited through an ADHD charity (n = 17) and two hospital outpatient clinics for adults with ADHD in England (n = 13). Half of the participants were diagnosed with ADHD during childhood or adolescence and the remainder during adulthood. Semi-structured interviews were conducted and data was analysed using a thematic approach based on Grounded Theory principles.

Results: Analysis revealed five core themes: 'An uphill struggle': the challenge of accessing services, 'Accumulated Psychosocial Burden and the Impact of ADHD', 'Weighing up Costs vs. Benefits of ADHD Pharmacological Treatment', 'Value of Non-pharmacological Treatment' and 'Barriers to Treatment Adherence'. Accessing services and the challenges associated with securing a definitive diagnosis of ADHD in adulthood was an *'uphill struggle'*, often due to sceptical and negative attitudes towards ADHD by healthcare professionals. ADHD-related impairment had an overwhelmingly chaotic impact on every aspect of patients' lives and many felt ill equipped to cope. A

BMC Health Services Research

PRO MEASURES OFFER A WIDE RANGE OF PUBLICATIONS OPPORTUNITIES (3/5)

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review
2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews
3	Confirm conceptual framework and assess properties of PRO measure	Validation study in relevant patient samples
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5	Modify PRO measure for wider usage	Cultural adaptations, translations, evaluations in related diseases

AP&T

Alimentary Pharmacology & Therapeutics

The Gastro-oesophageal Reflux Disease Impact Scale: a patient management tool for primary care

R. JONES*, K. COYNE† & I. WIKLUND‡

*Department of General Practice and Primary Care, King's College London, London, UK; †Evidera BioSource Corporation Center for Health Outcomes Research, Bethesda, MD, USA; ‡AstraZeneca R&D Mölndal, Mölndal, Sweden

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Submitted 19 February 2007
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Resubmitted 15 March 2007
Second decision 16 March 2007
Resubmitted 27 March 2007
Accepted 10 April 2007

SUMMARY

Background
Symptoms of gastro-oesophageal reflux disease have a substantial impact on patients' everyday lives.

Aim
To develop and test a short questionnaire to aid patient–doctor communication.

Methods
The Gastro-oesophageal Reflux Disease Impact Scale was developed from a systematic literature review, focus groups of patients and primary care physicians, and patient cognitive interviews. A psychometric validation study was conducted based on two consultations in new (n = 100) or chronic (n = 105) gastro-oesophageal reflux disease patients.

Results
The Gastro-oesophageal Reflux Disease Impact Scale demonstrated internal consistency [Cronbach's alpha ranged from 0.68 to 0.82], reproducibility [intraclass correlation coefficient in stable patients ranged from 0.61 to 0.72] and construct validity [Spearman correlations with Quality of Life in Reflux and Dyspepsia instrument and Reflux Disease Questionnaire: 0.5–0.8 in both patient groups]. Effect sizes in new and chronic gastro-oesophageal reflux disease patients ranged from 0.9 to 1.5 and 0.32 to 0.42, respectively. Doctors reported altering their treatment decision based on information provided by the Gastro-oesophageal Reflux Disease Impact Scale in 39% of patients, and 77% of doctors found it to be useful.

Conclusions
The Gastro-oesophageal Reflux Disease Impact Scale demonstrated good psychometric properties in newly diagnosed gastro-oesophageal reflux disease patients and those already receiving treatment. This simple communication tool is a useful aid for managing primary care patients with gastro-oesophageal reflux disease.

Aliment Pharmacol Ther **25**, 1451–1459

doi:10.1111/j.1365-2036.2007.03343.x

PRO MEASURES OFFER A WIDE RANGE OF PUBLICATIONS OPPORTUNITIES (4/5)

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review
2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews
3	Confirm conceptual framework and assess properties of PRO measure	Validation study in relevant patient samples
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs
5	Modify PRO measure for wider usage	Cultural adaptations, translations, evaluations in related diseases

JAMA Ophthalmology

Formerly *Archives of Ophthalmology*

Original Investigation | CLINICAL TRIAL

Patient-Reported Visual Function Outcomes Improve After Ranibizumab Treatment in Patients With Vision Impairment Due to Diabetic Macular Edema

Randomized Clinical Trial

PRO MEASURES OFFER A WIDE RANGE OF PUBLICATIONS OPPORTUNITIES (5/5)

	What do we need to do?	How do we do it?
1	Find out what PRO measures and concepts are already available	Systematic literature review
2	Develop conceptual framework and draft PRO measure	Patient and physician focus groups and cognitive interviews
3	Confirm conceptual framework and assess properties of PRO measure	Validation study in relevant patient samples
4	Collect, analyze and interpret PRO data in clinical studies	Use PRO measure in clinical studies alongside other PROs
5	Modify PRO measure for wider usage	Cultural adaptations, translations evaluations in related diseases

South African GASTROENTEROLOGY Review

RESEARCH

Psychometric Validation of the Afrikaans Translation of Two Patient-Reported Outcomes Instruments for Reflux Disease

Christo J. van Rensburg[1], Károly B. Kulich[1], Jonas Carlsson[2], Ingela K. Wiklund[2]
[1]Gastroenterologist Unit, Tygerberg Hospital, South Africa
[2]Clinical Science, AstraZeneca R&D Mölndal, Sweden

ABSTRACT
Objectives: To verify the validity and reliability of two Afrikaans patient-reported outcomes instruments: a disease-specific and a health-related quality of life instrument in patients with reflux disease. **Design:** Psychometric validation study. **Setting:** South African, major referral gastroenterology clinic. **Subjects:** Consecutive patients with predominant symptoms of heartburn. **Outcome measures:** Patients completed the Afrikaans versions of the Gastrointestinal Symptom Rating Scale (GSRS), the Quality of Life in Reflux and Dyspepsia questionnaire (QOLRAD) and the Short Form Health-36 (SF-36). The frequency and severity of heartburn during the previous 7 days were recorded. **Results:** 128 patients (aged of 46.0 ± 12.3 years; 74.4% female, 87% mixed race) completed the assessments. Most patients (82%) had severe symptoms and half (54%) had symptoms on more than 5 days in the previous week. Patients were most bothered by symptoms of reflux (mean GSRS score of 4.9), indigestion (4.0) and abdominal pain (4.0). These symptoms caused patients problems with food and drink (mean QOLRAD scores of 3.8), emotional distress (3.9), impaired vitality (3.7) and sleep disturbances (3.8). The internal consistency of the GSRS symptom clusters was between 0.69 and 0.86 and, for QOLRAD dimensions, it was in the range 0.80–0.94. Test–retest reliability was 0.61–0.76 (GSRS) and 0.71–0.82 (QOLRAD). Relevant domains of GSRS and QOLRAD were significantly correlated. GSRS domains of abdominal pain and indigestion, and relevant QOLRAD domains showed negative correlation with related SF-36 domains. **Conclusions:** The Afrikaans translations of GSRS and QOLRAD are valid and reliable instruments for use in clinical trials for the assessment of reflux symptoms and their impact on South African patients' health-related quality of life.

Suggested keywords: Reliability, Validity, Reflux, Health-Related Quality of Life, South Africa

INTRODUCTION

Heartburn is the primary symptom of gastroesophageal reflux disease (GERD), a common condition, associated with a range of esophageal and extra-esophageal symptoms and complications. GERD affects many aspects of patients' lives, including their sleep and enjoyment of meals and social occasions.[1,2] As GERD can be diagnosed on the basis of symptoms alone, patient-reported outcomes instruments are needed to quantify GERD symptoms and their effect on patients' lives. Generic patient-reported outcomes instruments are comprehensive, and allow comparisons between diseases, treatments and populations.[3] Disease-specific instruments, on the other hand, capture details about the disease activity and symptom patterns, and are generally more responsive to changes than generic instruments. Taking into consideration their complementary nature, disease-specific and generic instruments are, in practice, often used in tandem.

Two of the best-characterized disease-specific patient-reported outcomes instruments for patients with GERD are the Gastrointestinal Symptom Rating Scale (GSRS) and the Quality of Life in Reflux and Dyspepsia questionnaire (QOLRAD).[4] These were originally developed in English, and have been subsequently translated and cross-culturally adapted for use in international studies. This paper reports the development and psychometric validation of Afrikaans versions of GERS and QOLRAD in patients with GERD.

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METHODS

Patient selection

Consecutive patients with predominant symptoms of heartburn attending a major referral clinic, the Gastroenterology Unit of Tygerberg Hospital between

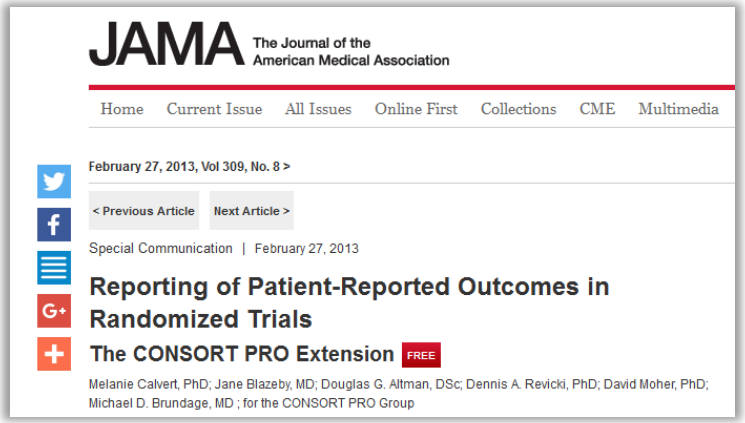
WRITING UP THE STUDIES – MAKING THE MOST OF THE AVAILABLE GUIDANCE

- There is less guidance on the reporting of PRO studies than RCTs
- Reporting standards are available for describing PRO data in an RCT
  - CONSORT PRO
  - ISOQOL
- Regulatory guidance provides a framework for the elements that a PRO validation should cover
  - FDA and EMA guidelines
  - ISPOR PRO Special Interest Group

Qual Life Res (2013) 22:1161–1175
DOI 10.1007/s11136-012-0252-1

Patient-reported outcomes in randomized clinical trials: development of ISOQOL reporting standards

Michael Brundage · Jane Blazeby · Dennis Revicki · Brenda Bass · Henrica de Vet · Helen Duffy · Fabio Efficace · Madeleine King · Cindy L. K. Lam · David Moher · Jane Scott · Jeff Sloan · Claire Snyder · Susan Yount · Melanie Calvert



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February 27, 2013, Vol 309, No. 8 >

< Previous Article Next Article >

Special Communication | February 27, 2013

Reporting of Patient-Reported Outcomes in Randomized Trials

The CONSORT PRO Extension FREE

Melanie Calvert, PhD; Jane Blazeby, MD; Douglas G. Altman, DSc; Dennis A. Revicki, PhD; David Moher, PhD; Michael D. Brundage, MD ; for the CONSORT PRO Group

equator network

SIMPLE STEPS TO MAKING PRO ARTICLES EASIER FOR THE NON-SPECIALIST

- How can I convey the meaning to a non-PRO specialist among all this technical detail?

Use the abstract to place the study in a clinical context

Preface each section with one sentence that tells the non-specialist what it means (e.g. what is construct validity)

Use the conclusion to convey how the results might affect healthcare decision-making

Make use of **supplementary tables/figures/methods**

WHEN TO TARGET MAINSTREAM CLINICAL VERSUS SPECIALIST JOURNALS AND MEETINGS

- Specialist journals for PRO studies exist
  - But most of your key audiences are not PRO or psychometrics specialists
- Effective publication planning is essential
- Mainstream clinical journals and meetings
  - Core PRO papers – can be top-tier specialist journals
- Specialist journals and meetings
  - Technical and methodology papers (e.g. psychometric validation)

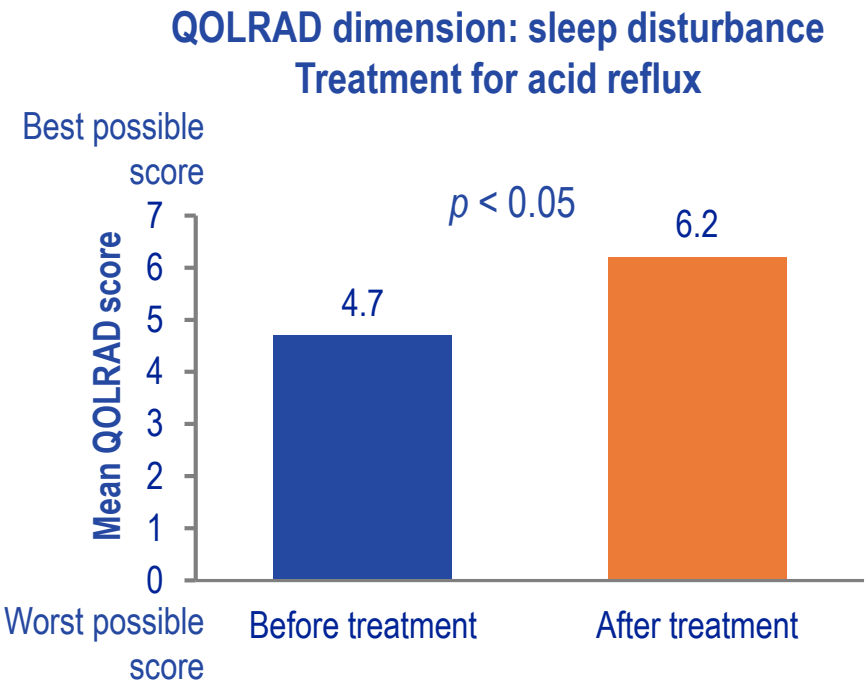
ISOQOL
International Society for Quality of Life Research

INTERNATIONAL SOCIETY FOR PHARMACOECONOMICS AND OUTCOMES RESEARCH
ISPOR

COMMUNICATING PATIENT-REPORTED OUTCOME DATA EFFECTIVELY

EFFECTIVE COMMUNICATION OF PRO DATA: APPLY THE 'SO WHAT?' FACTOR

- Effective communication involves clearly describing the data and then relating it to relevant measures of patient function
- The QOLRAD domain scores don't tell us anything about:
  - what the scale relates to in terms of **patient outcomes**
  - whether this difference in score is **clinically** meaningful



RELEVANCE OF CHANGES IN PRO SCORE: MINIMAL CLINICALLY IMPORTANT DIFFERENCE

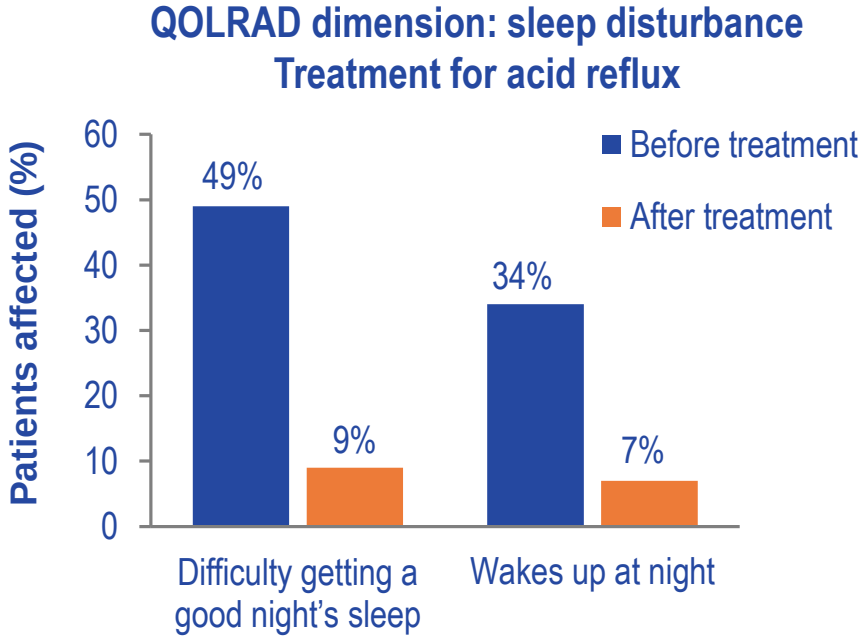
Minimal clinically important difference (MCID) is the smallest difference in score that patients perceive as beneficial and that is significant enough to result in a change to the patient's management[1,2]

- When a change in PRO score is less than the MCID it is unlikely to have a meaningful impact on the patient
  - Even if the difference is statistically significant

- Changes and differences in PRO scores should therefore be interpreted relative to the MCID for the instrument

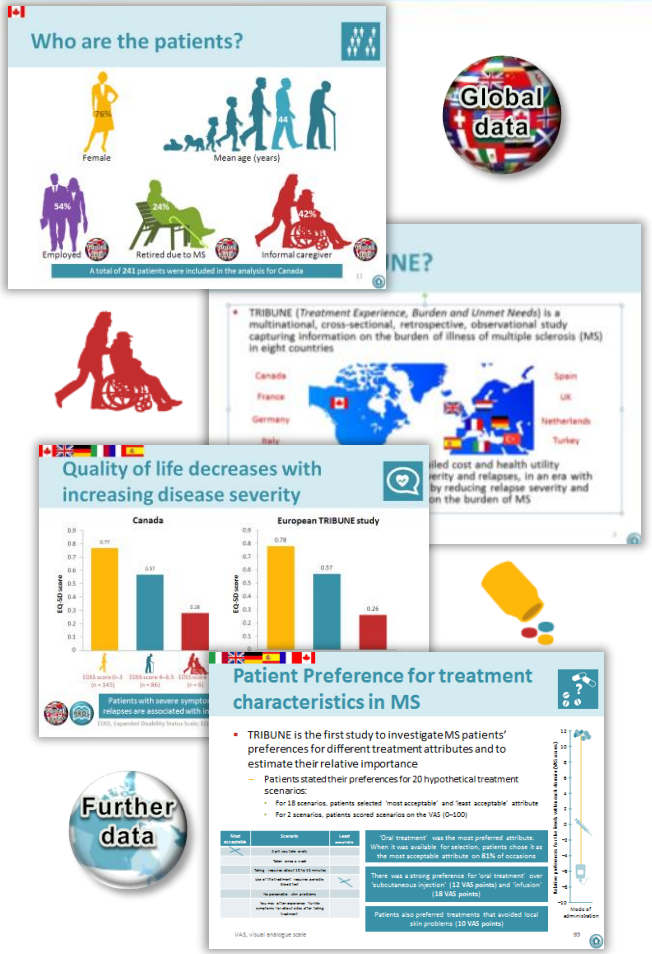
INTERPRETABILITY: DESCRIBING PRO DATA IN TERMS OF MEANINGFUL PATIENT OUTCOMES

- Mean data for the overall PRO measure are often only the starting point
- Consideration should be given to:
  - Significant differences in **individual domains or items**
  - How **threshold scores** correspond to **meaningful patient outcomes**
  - Differences across **relevant patient subsets** (e.g. disease severity)



HELP YOUR AUDIENCES – GO BEYOND THE INITIAL PRO STUDY PUBLICATION

- Most of your internal and external audiences for PRO publications will **not understand the technical details of PRO studies**
- Develop **simple, non-technical tools** to accompany PRO publications
  - One-page evidence summaries of key PRO study publications
  - Infographics-driven, visually stimulating interactive slide decks

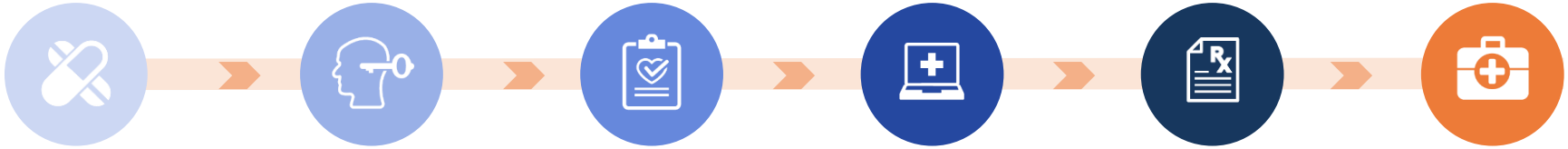


WHY DO WE CARE ABOUT PATIENT ENGAGEMENT?

WE'RE ON THE CUSP OF CHANGE

- Enhanced Patient Voice in Medicines Lifecycle (IMI2 Call 2)
- The European Patients' Academy (EUPATI)
- Adapt Smart
- European Medicines Research Training Network (EMTRAIN)
- Patient-Centred Outcomes Research Institute (PCORI)
- International Consortium for Health Outcomes Measurement (ICHOM)
- Patient Focused Medicines Development (PFMD)
- National Health Council (NHC)
- Faster Cures
- Clinical Trials Transformation Initiative (CTTI)
- TransCelerate
- DIA-Tufts initiative on Return on Engagement

UNDERSTANDING WHAT MATTERS TO PATIENTS ACROSS THE DRUG DEVELOPMENT CONTINUUM



Product development

- Input on interest in research question
- Provide key insights on unmet needs that matter to patients

Study design and protocol development

- Help to design study protocols (study visits, procedures, eligibility criteria, etc.)
- Advise on and review the development of patient materials (e.g. ICFs) (click here for more information)
- Design and advise on feasibility of patient recruitment strategies
- Input on meaningful PRO endpoints for inclusion in the pivotal trial

Study start and ongoing monitoring

- Assist in selecting/ recruiting trial sites
- Educate and motivate patients for clinical trial accrual
- Help with retention strategies
- Report feedback on participant experience
- Serve on Data Safety and Monitoring Board

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THE INTERNAL JOURNEY TOWARDS A BETTER UNDERSTANDING OF PATIENTS

I can think like a patient, so I already understand the patient perspective

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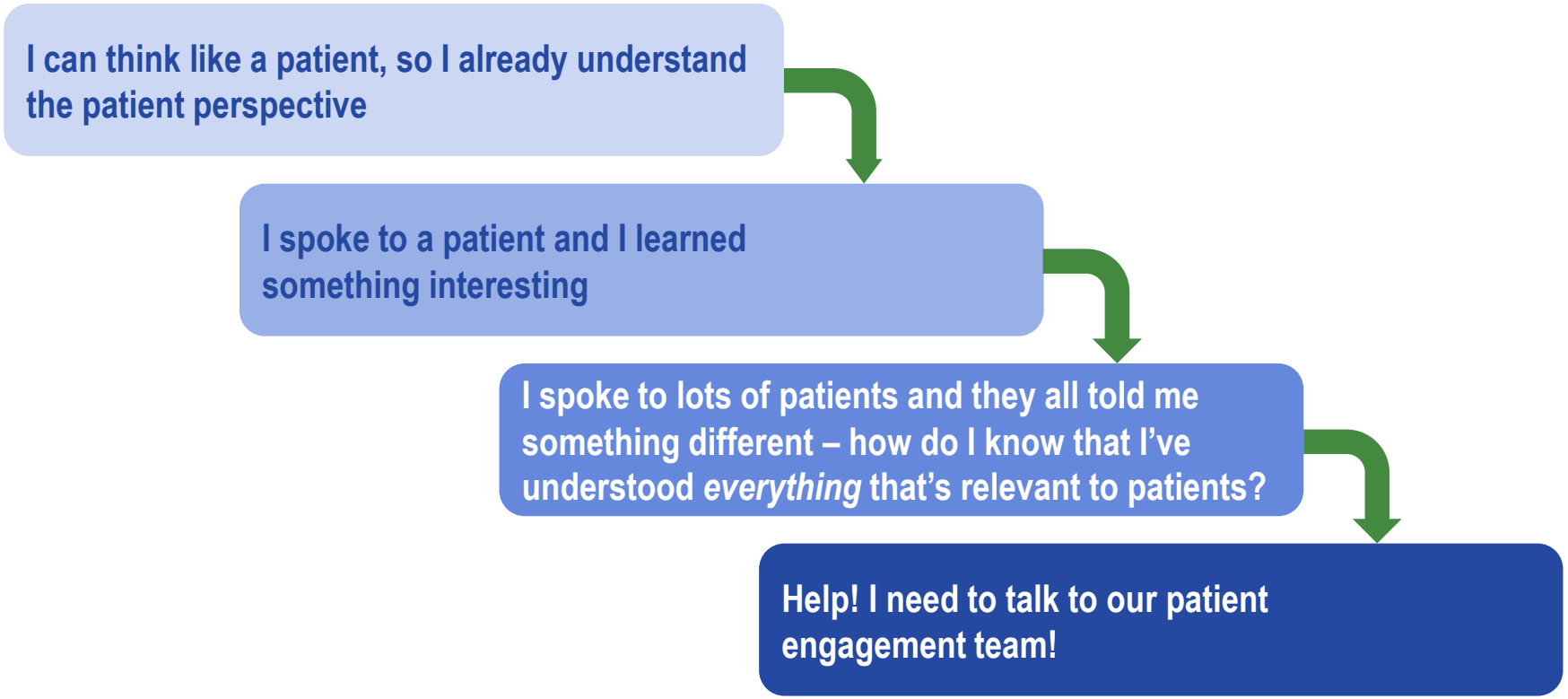
I spoke to a patient and I learned something interesting

I spoke to lots of patients and they all told me something different – how do I know that I've understood *everything* that's relevant to patients?

THE INTERNAL JOURNEY TOWARDS A BETTER UNDERSTANDING OF PATIENTS



UNDERSTANDING HEALTH LITERACY

Health literacy … entails people's knowledge, motivation and competences to access, understand, appraise and apply health information in order to make judgements and take decisions in everyday life concerning health care, disease prevention and health promotion to maintain or improve quality of life during the life course

World Health Organization Regional Office for Europe

Health literacy report 2013

WHAT DOES HEALTH LITERACY MEAN?

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"Health literacy means empowerment"

WHY DOES HEALTH LITERACY MATTER?

- Worse health[1]
- Finds medication difficult to manage[1]
- Higher hospitalization rate[1]
- Higher mortality[1]

- High healthcare costs[2]

AUDIENCE QUESTION

What reading age should you write your communications for, if you want > 90% of the public to understand what you've written?

A. 5–6 years
B. 7–8 years
C. 9–11 years
D. 12–14 years
E. 15–17 years

WHAT DOES THIS MEAN FOR COMMUNICATORS?

Proportion of readers able to understand, %	National Qualifications Framework age equivalent
93%	9–11 years
85%	GCSE grades D–G
57%	GCSE grades A*–C or higher qualifications

The single biggest problem in communication is the illusion it has taken place

George Bernard Shaw, playwright and author

PATIENT INFORMATION NEEDS TO IMPROVE

- Researchers user-tested leaflets written by charities and the NHS
  - 64 leaflets (50 included numerical information)
  - 4767 UK residents aged 16–65 years, sampled to reflect the socioeconomic demographics of the UK population

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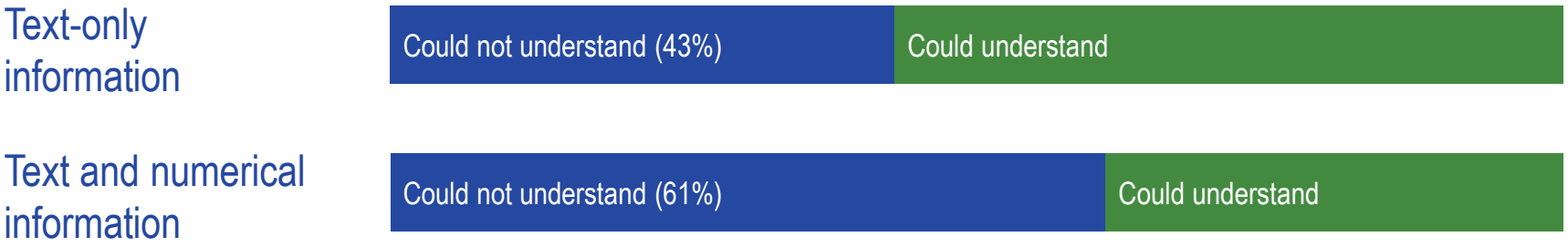
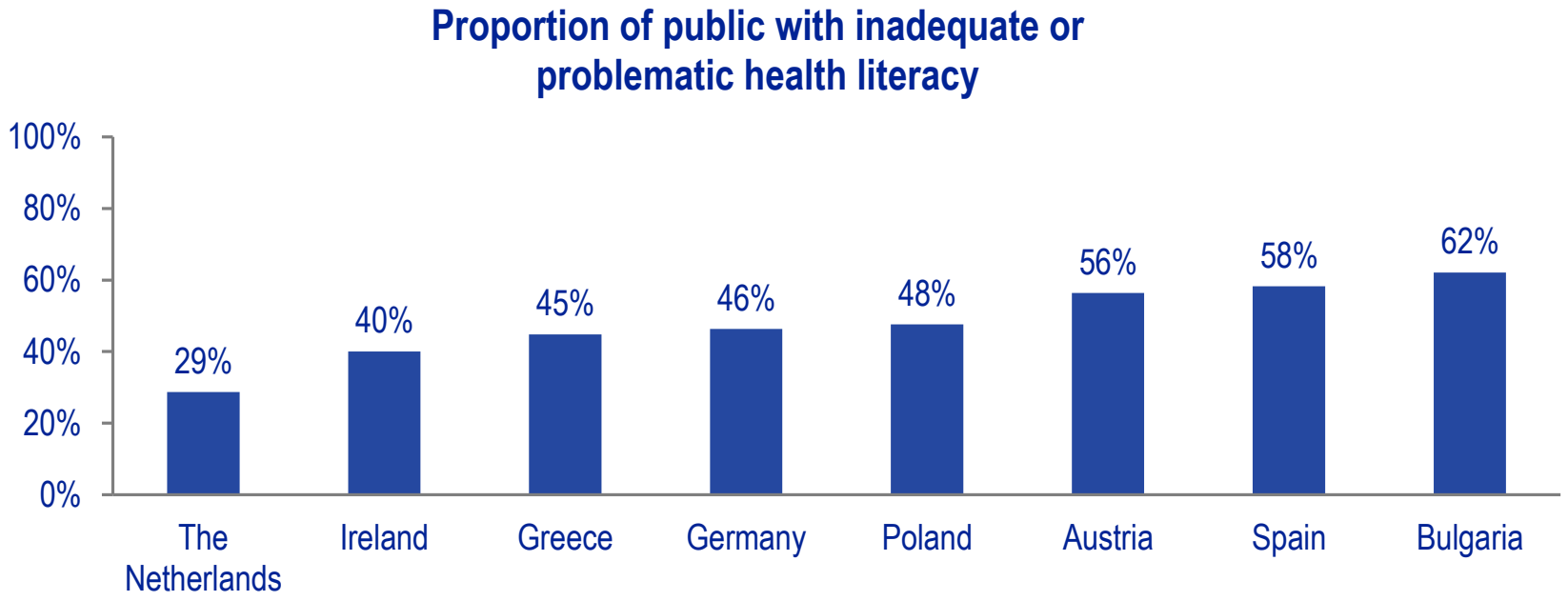


HEALTH LITERACY IN EUROPE

- Health literacy varies across Europe



HEALTH LITERACY IN THE US

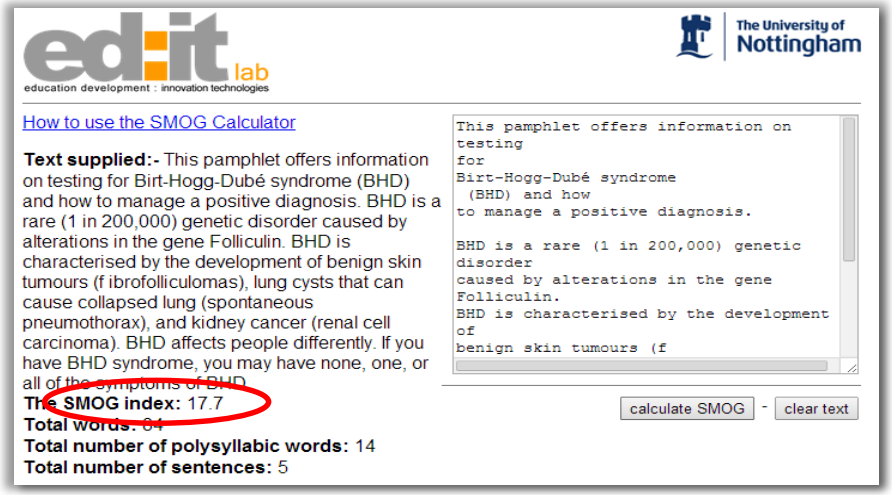
- Approximately 36% of adults have limited health literacy[1]
- Only 12% of the population has proficient health literacy[1]

"Nearly 9 out of 10 adults have difficulty using the everyday health information that is routinely available in our health care facilities, retail outlets, media and communities"[2]

BRIEF TIPS ON HOW TO WRITE IN A WAY THAT PATIENTS WILL UNDERSTAND

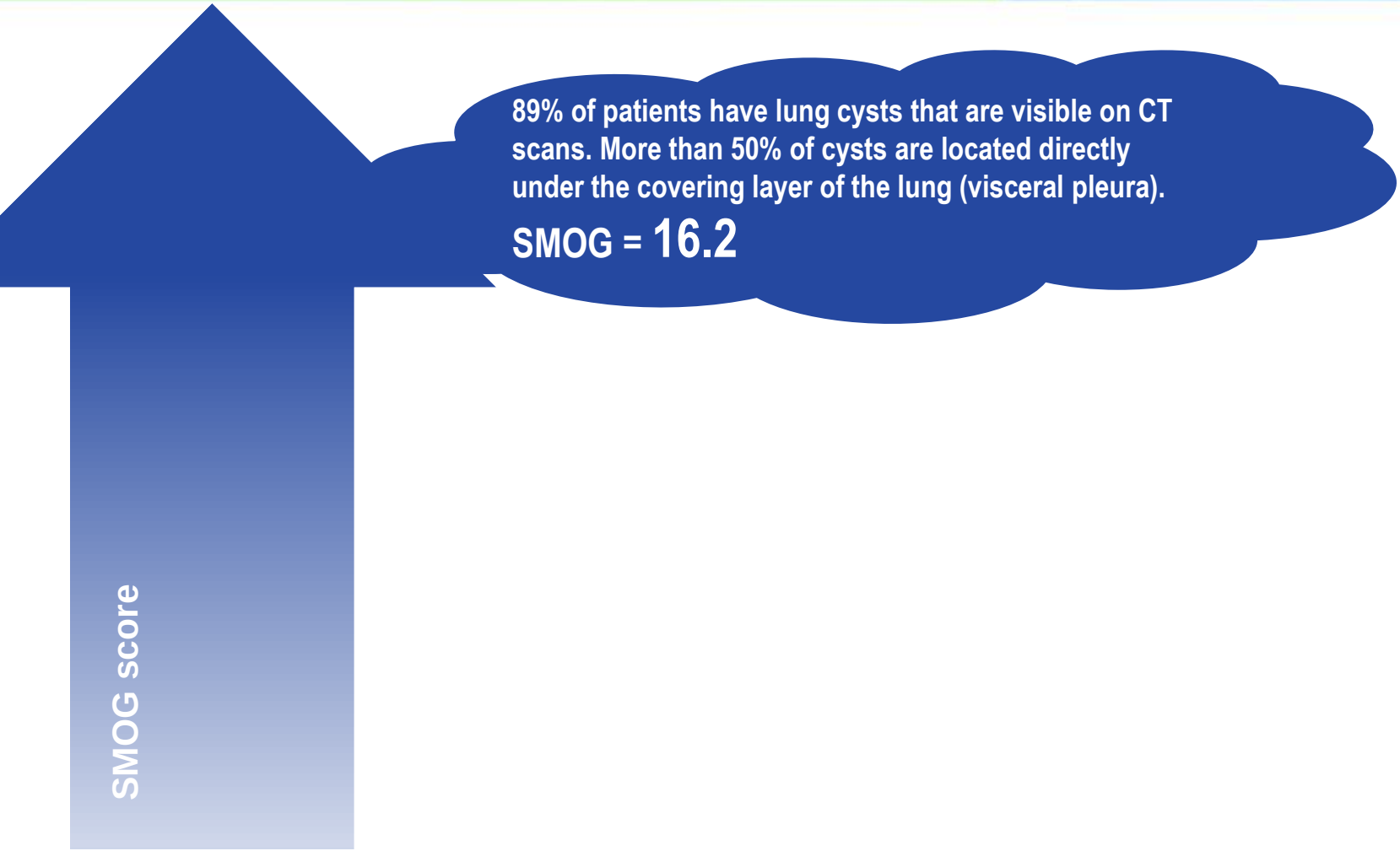
- Simple language
- Short sentences
- Simplify numerical information
- Larger fonts with plenty of white space
- Use **bold lowercase letters** for emphasis (not CAPITALS, *italics* or underlined)
- Left-align rather than fully justify
- Use only pictures that are directly relevant to the text
- User-test everything

Simple Measure of Gobbledegook (SMOG)



http://www.niace.org.uk/misc/SMOG-calculator/smogcalc.php

SMOG SCORE EXAMPLE – TALKING ABOUT LUNG CYSTS



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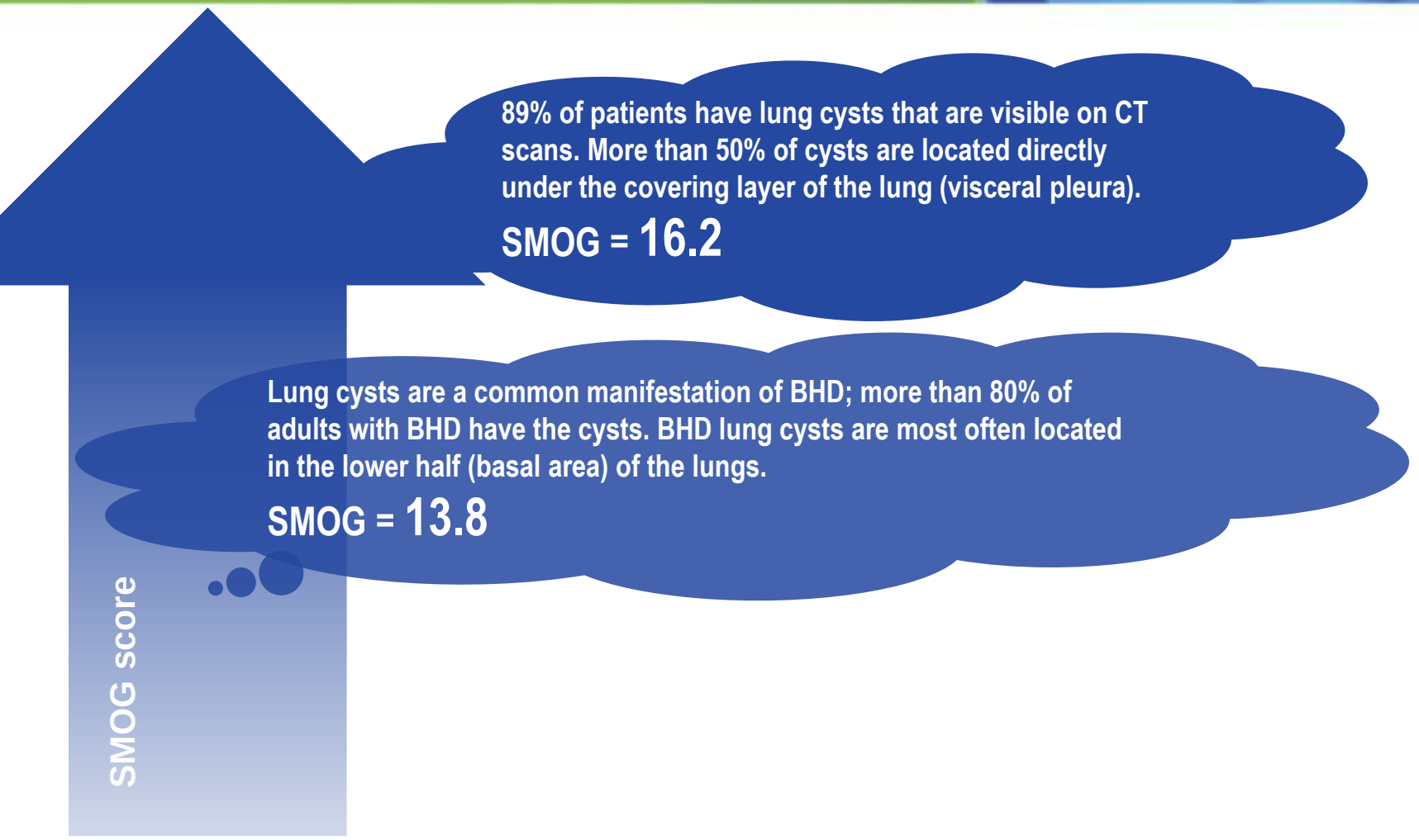

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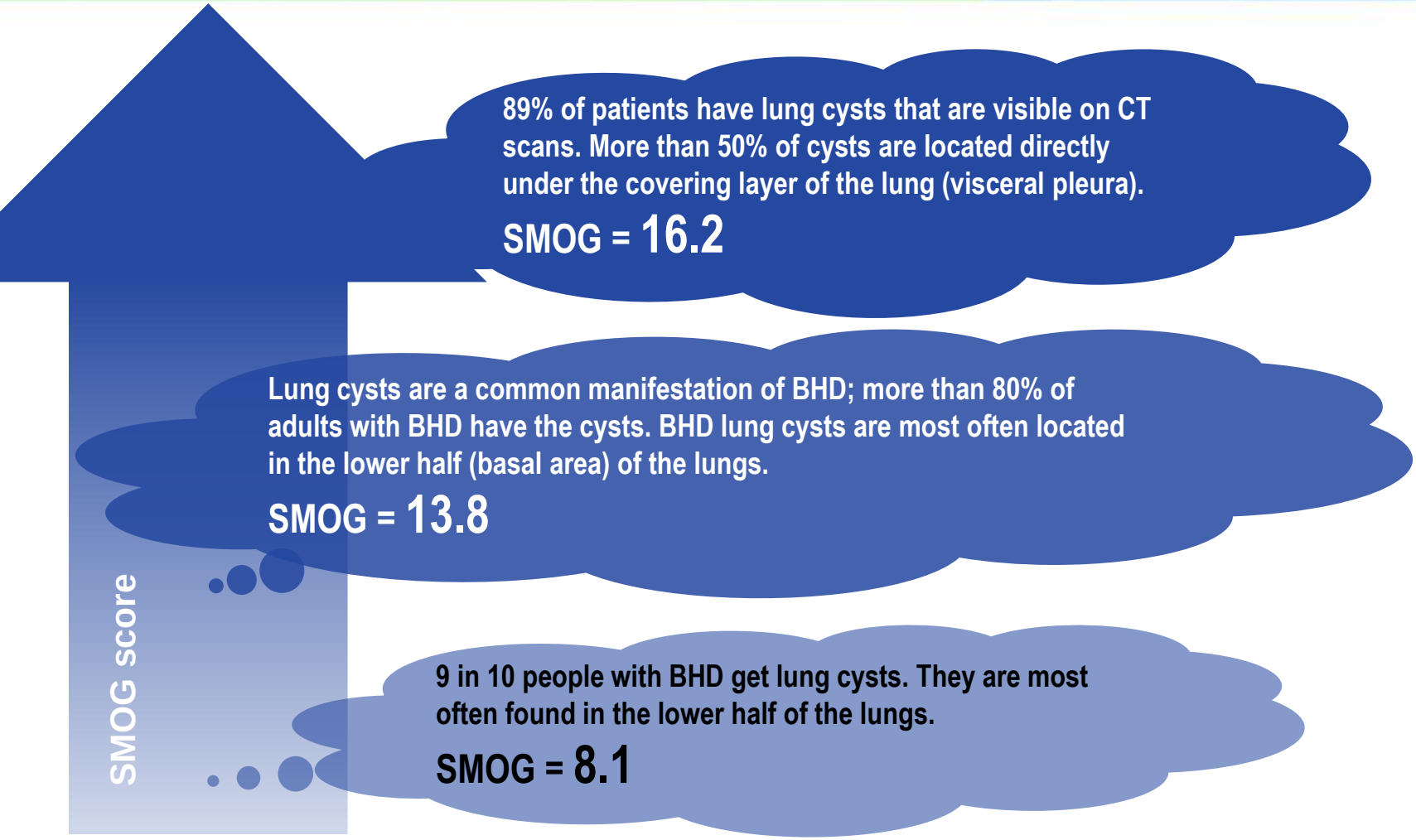

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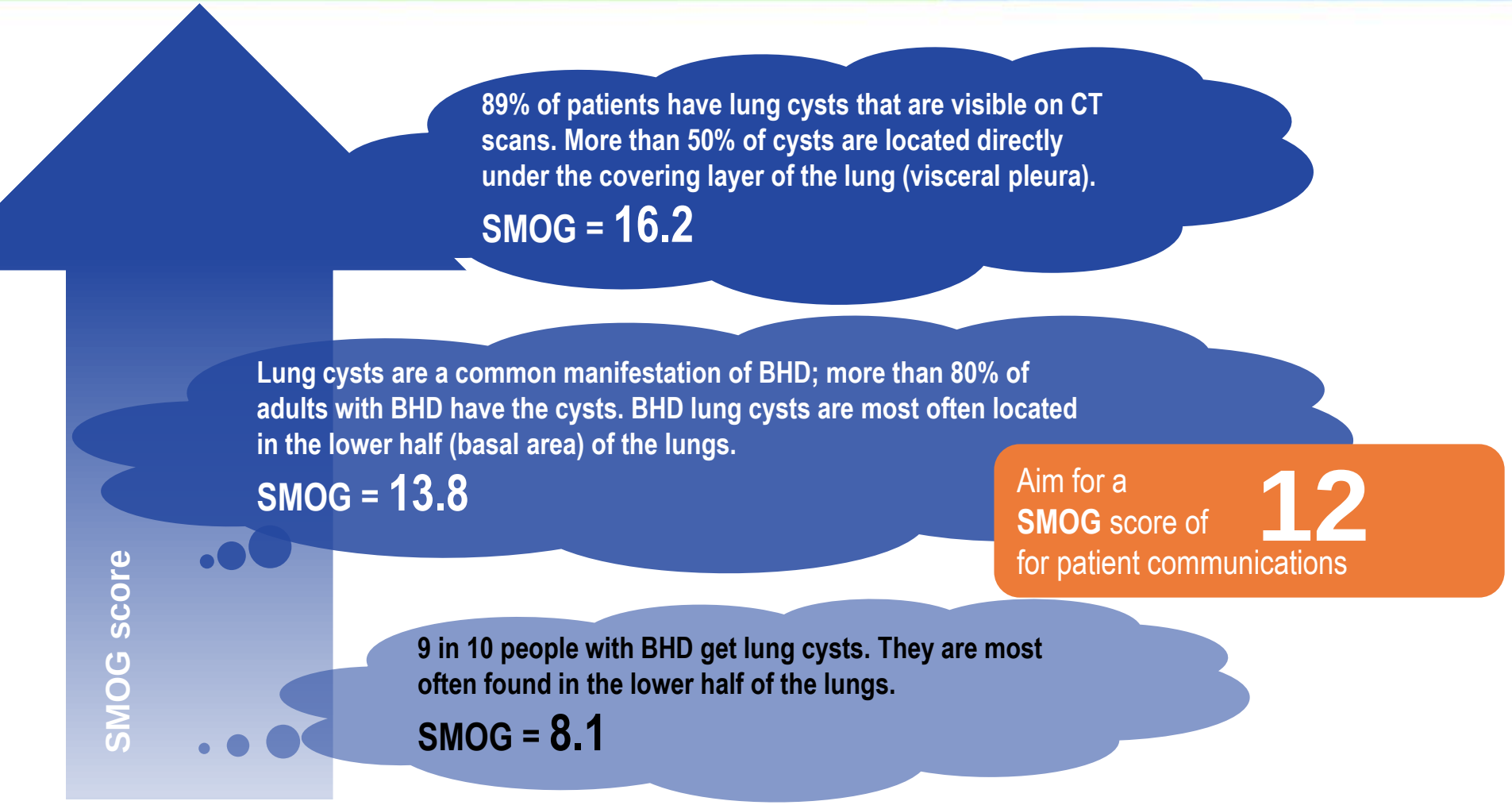
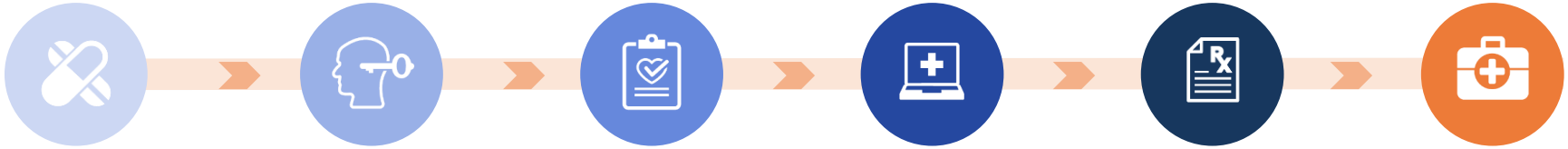


Aim for a **SMOG** score of **12** for patient communications

BEING PATIENT-CENTRIC AS A PUBLICATIONS PROFESSIONAL

UNDERSTANDING WHAT MATTERS TO PATIENTS ACROSS THE DRUG DEVELOPMENT CONTINUUM



Product development

- Input on interest in research question
- Provide key insights on unmet needs that matter to patients

Study design and protocol development

- Help to design study protocols (study visits, procedures, eligibility criteria, etc.)
- Advise on and review the development of patient materials (e.g. ICFs) (click here for more information)
- Design and advise on feasibility of patient recruitment strategies
- Input on meaningful PRO endpoints for inclusion in the pivotal trial

Study start and ongoing monitoring

- Assist in selecting/ recruiting trial sites
- Educate and motivate patients for clinical trial accrual
- Help with retention strategies
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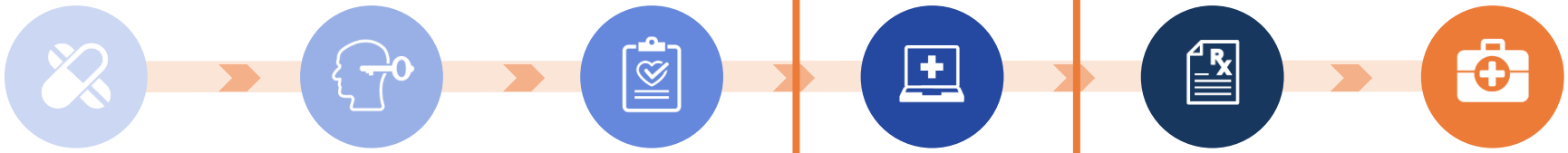

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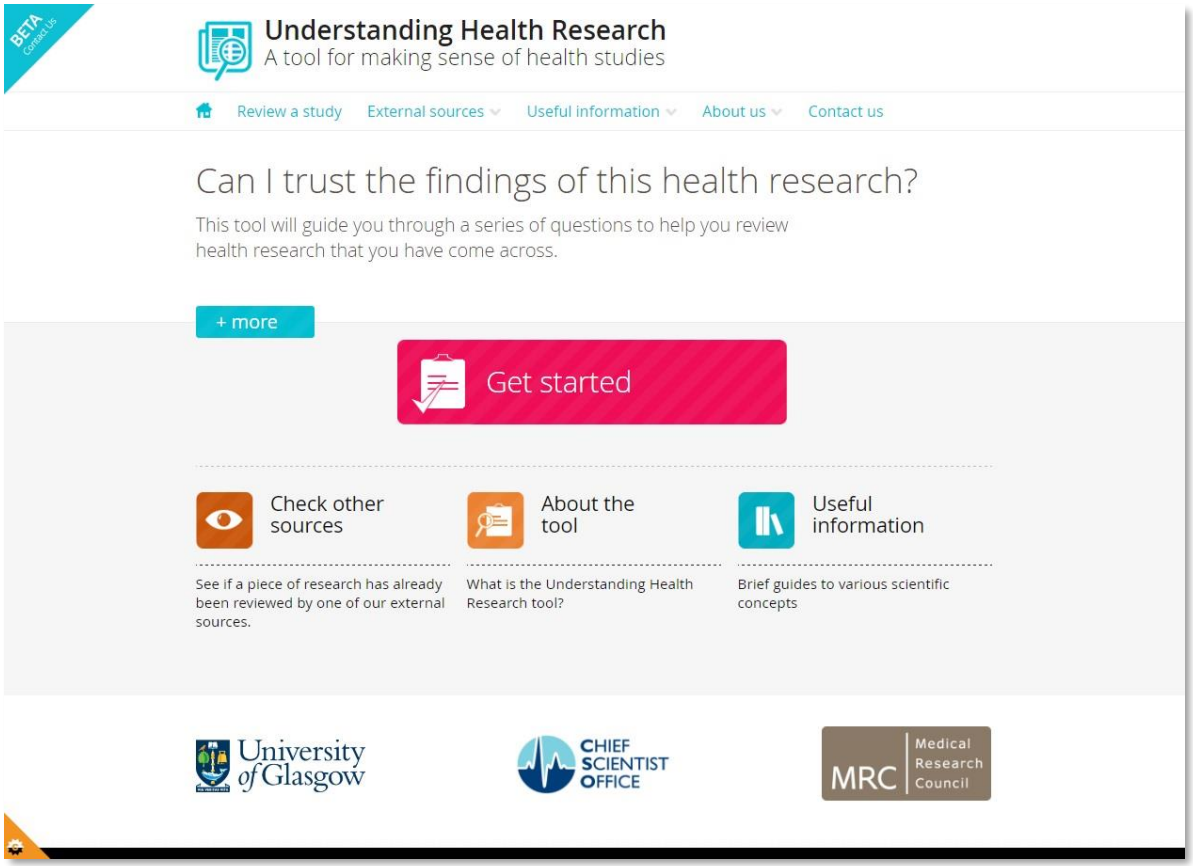
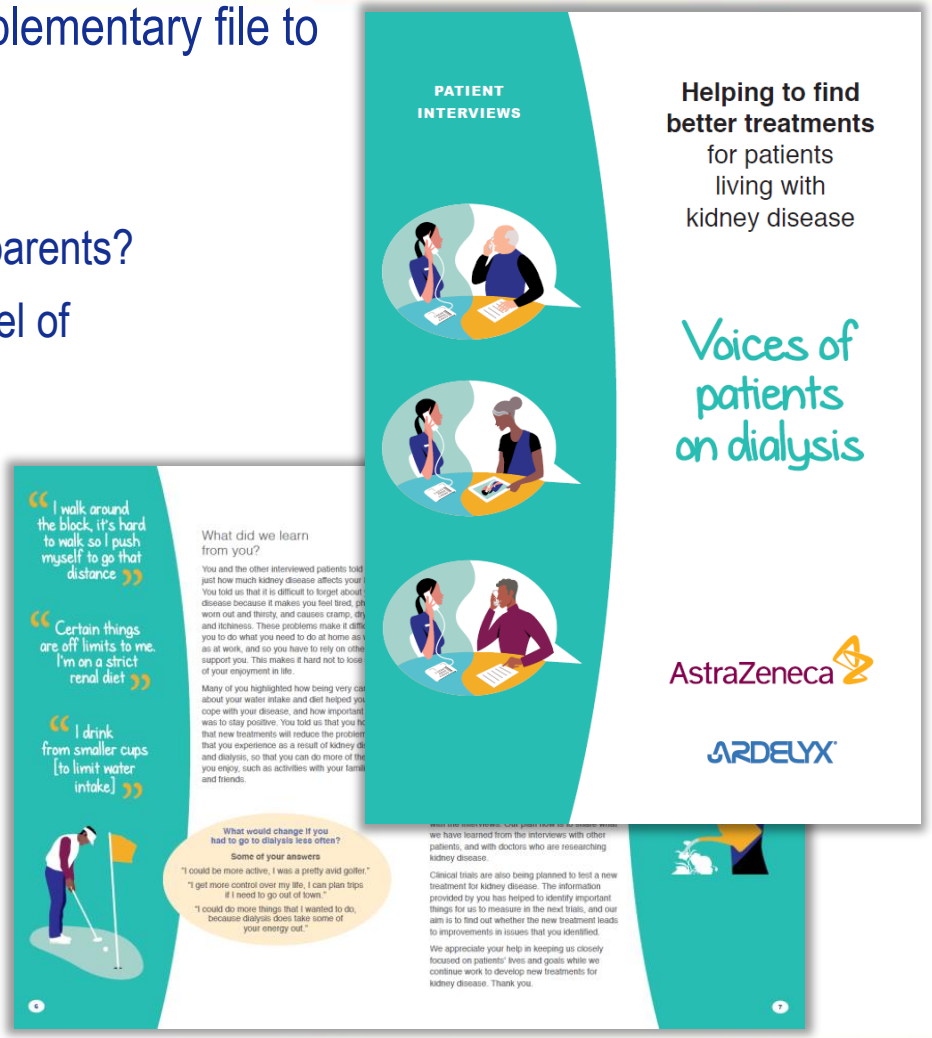
PATIENTS ARE ALREADY READING PAPERS



(1) PUBLISH OPEN ACCESS, SO PATIENTS CAN FIND YOUR PAPER

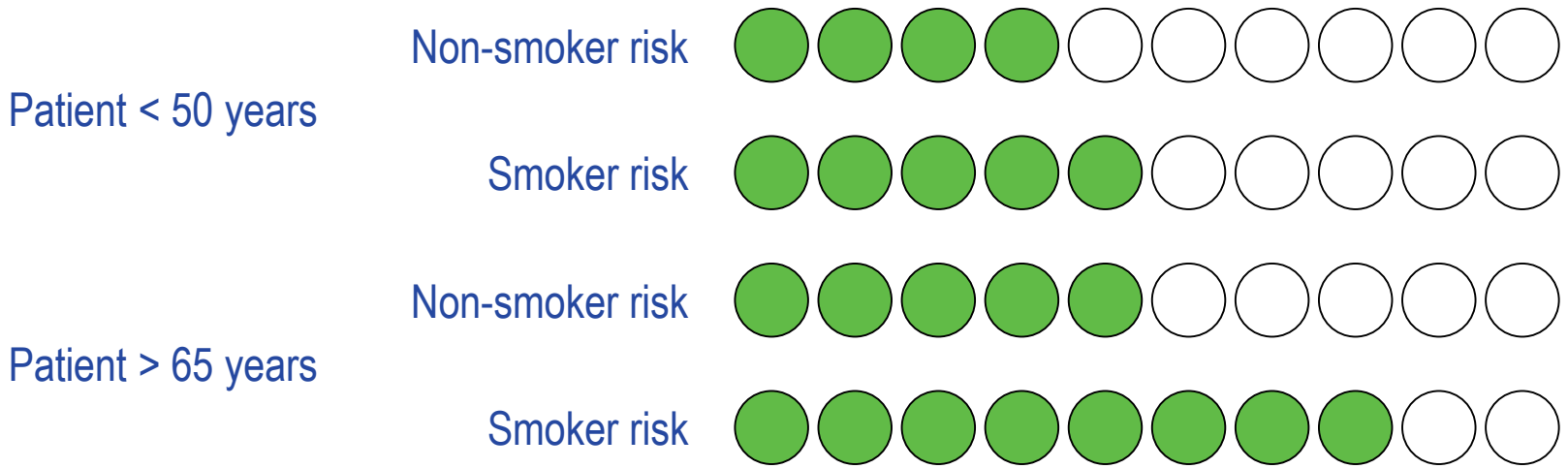
(2) PROVIDE A (PATIENT) LAY SUMMARY, WRITTEN AT THE APPROPRIATE LEVEL

- Can be a **standalone** document, or a supplementary file to a published journal article
- Pitch the document at the **correct level**
  - How would you describe the study to your parents?
  - Work from the patient's perspective and level of understanding
  - Don't treat a patient like a scientist who doesn't understand big words



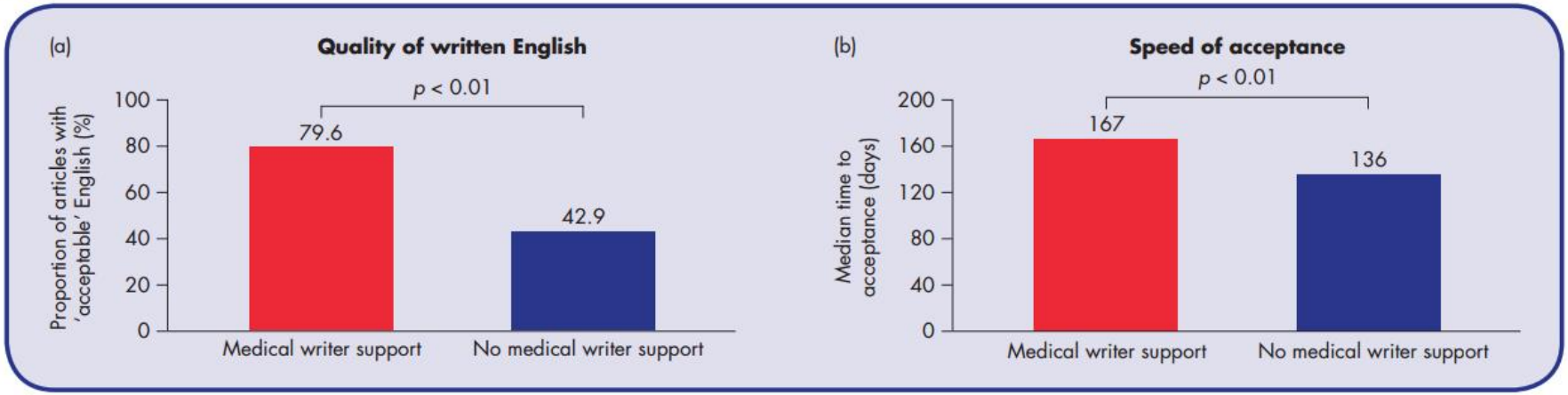
(3) INCLUDE SECONDARY ANALYSES OF KEY PATIENT POPULATIONS IN PUBLICATION PLANNING

- Be clear about the patient population the research relates to
- Clearly communicate information for each group



(4) USE CLEAR, INTUITIVE GRAPHICS WHERE POSSIBLE

Figure 4. (a) Quality of written English, as assessed by peer reviewers, and (b) time from submission to editorial acceptance for articles with and without acknowledged medical writer support.



Gattrell W *et al*., Professional medical writing support improves the quality but not the speed of reporting of randomized controlled trials [poster]. Presented at the 2015 European Meeting of the International Society for Medical Publication Professionals, 20–21 January 2015, London, UK. Available from: http://www.ismpp.org/assets/docs/Education/EuropeanMeeting/2015EM/Posters/2015%20eu%20meeting%20gattrell%20poster.pdf (Accessed 13 September 2016)

A lie will go round the world while the truth is pulling its boots on

C. H. Spurgeon, *Gems from Spurgeon* (1859)

(5) INCLUDE PATIENTS AS CO-AUTHORS

ORPHANET JOUNRAL OF RARE DISEASES

Phenotype and natural history in 101 individuals with Pitt-Hopkins syndrome through an internet questionnaire system

Channa F. de Winter[1†], Melanie Baas[2†], Emilia K. Bijlsma[3], John van Heukelingen[4], Sue Routledge[5] and Raoul C. M. Hennekam[2*]

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[4]Pitt-Hopkins Parents Support Group, Leidschendam, The Netherlands. [5]Pitt-Hopkins Parents Support Group UK, London, UK

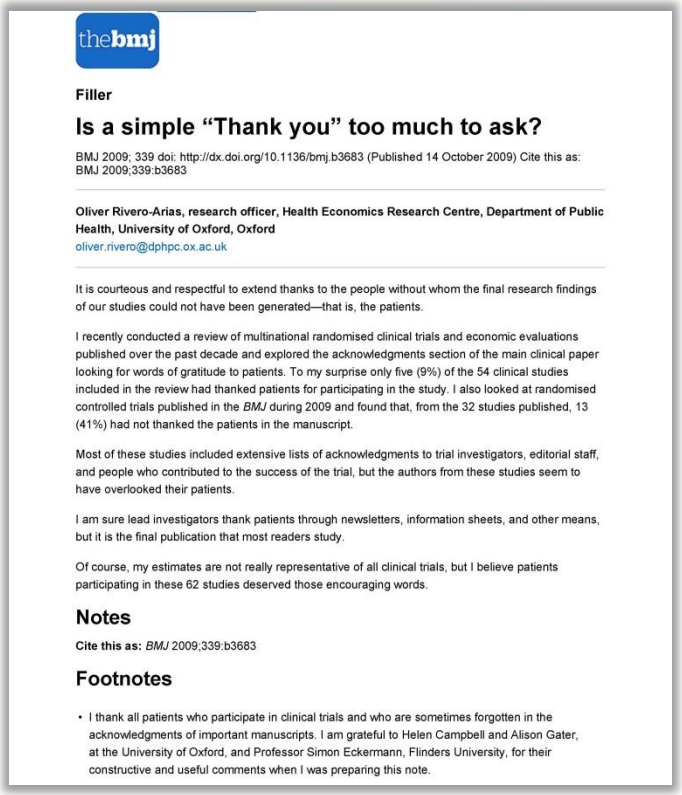
BMJ PATIENT PARTNERSHIP STRATEGY

- Report patient involvement in:
  - Choice of research question
  - Study design
  - Outcome measures
  - Dissemination of results
- Patients or carers (caregivers) should be involved as contributors or authors

"In future we are likely to include clinical research papers only if the authors can demonstrate partnership with patients in their study"

And finally…
(6) Thank patients for their contribution

- Clinical study acknowledgments sections routinely thank the investigators, the medical centre staff, the professional medical writer …
- … but in articles published by the *BMJ* that used patient data, **patients were acknowledged only ~50% of the time**
- Why not routinely thank patients in any study that involves them?



thebmj

Filler

Is a simple "Thank you" too much to ask?

BMJ 2009; 339 doi: http://dx.doi.org/10.1136/bmj.b3683 (Published 14 October 2009) Cite this as: BMJ 2009;339:b3683

Oliver Rivero-Arias, research officer, Health Economics Research Centre, Department of Public Health, University of Oxford, Oxford
oliver.rivero@dphpc.ox.ac.uk

It is courteous and respectful to extend thanks to the people without whom the final research findings of our studies could not have been generated—that is, the patients.

I recently conducted a review of multinational randomised clinical trials and economic evaluations published over the past decade and explored the acknowledgments section of the main clinical paper looking for words of gratitude to patients. To my surprise only five (9%) of the 54 clinical studies included in the review had thanked patients for participating in the study. I also looked at randomised controlled trials published in the *BMJ* during 2009 and found that, from the 32 studies published, 13 (41%) had not thanked the patients in the manuscript.

Most of these studies included extensive lists of acknowledgments to trial investigators, editorial staff, and people who contributed to the success of the trial, but the authors from these studies seem to have overlooked their patients.

I am sure lead investigators thank patients through newsletters, information sheets, and other means, but it is the final publication that most readers study.

Of course, my estimates are not really representative of all clinical trials, but I believe patients participating in these 62 studies deserved those encouraging words.

Notes

Cite this as: *BMJ* 2009;339:b3683

Footnotes

- I thank all patients who participate in clinical trials and who are sometimes forgotten in the acknowledgments of important manuscripts. I am grateful to Helen Campbell and Alison Gater, at the University of Oxford, and Professor Simon Eckermann, Flinders University, for their constructive and useful comments when I was preparing this note.


THANK YOU!

QUESTIONS . . .

- To ask a question, please type your query into the Q&A box
- To ensure anonymity, before sending please choose the drop-down box option, "Hosts, Presenters and Panelists." Otherwise, ALL audience members will be able to see your submitted question

UPCOMING ISMPP U'S

DATE	TOPIC	FACULTY
October 26	*Reproducibility: A Tragedy of Errors*	**David Allison & Richard Sarver** University of Alabama at Birmingham
November 30	*Practices and Challenges in Publication Peer Review*	**Martin Delahunty,** Nature Partner Journals **Mary Beth DeYoung**, AstraZeneca **Rosamund Snow**, *BMJ* *Ann Davis, Moderator*

THANK YOU FOR ATTENDING!

- We hope you enjoyed today's presentation. **Please check your email for a link to a survey** that should take a few minutes to complete. We depend on your feedback and take your comments into account as we develop future educational offerings. Thank you in advance for your participation!