



JOHNS HOPKINS
BLOOMBERG
SCHOOL *of* PUBLIC HEALTH



Protecting Health, Saving Lives—*Millions at a Time*

The economic and social value of innovation

John F P Bridges Ph. D.

***Department of Health Policy and Management
Johns Hopkins Bloomberg School of Public Health***

Founding Editor,

The Patient – Patient-Centered Outcomes Research

jbridges@jhsph.edu

The value of innovation

- State-of-the-art **medical technology** helps to **save lives** of European citizens, to regain and maintain their **productivity** and to improve their **quality of life** and wellness.
- When we measure the **economic and social returns** they are normally **far greater** than the investment costs made on innovation.
- Hence, we need valid metrics to:
 - Identify innovation
 - Value the social and economic returns



Acknowledgements/Transparency

- My research on this topic has been supported by a Johns Hopkins Bloomberg School of Public Health (JHSPH) Faculty Innovation Award, and through funding from USAID, Pfizer Inc., USA, Janssen Cilag GmbH, Germany and the **Institute for Health Technology Studies (InHealth)**, USA.
- Through **The Patient – Patient-Centered Outcomes Research**, Bridges and JHSPH has a financial relationship with Wolters Kluwer Health.

The evaluation revolution

- Over the past 30 years **we have come a long way** in the evaluation of medicines
 - Evidence based medicine (EBM)
 - Outcomes research
 - Pharmacoeconomics
 - Medical decision making
 - Health technology assessment (HTA).
- These five movements have **collectively changed** the quality of Medicine for the better.

Two hurdles in measuring value

- Hurdle 1: **The payers perspective**
 - Cost savings, value for money
 - Cost-effectiveness analysis
- Hurdle 2: **Methods in clinical sciences**
 - Large/lengthy clinical trial
 - Irrelevant/outcomes outcomes
- We need to **radically rethink** evaluation science to focus on the most important stakeholder
 - **The patient**



The fundamentals of evaluation

- 1. Identification:** What factors are important to patients and society?
- 2. Measurement:** How do these factors vary across interventions?
- 3. Valuation:** How do we rate, rank or score outcomes?
- 4. Deliberation:** How do we make decisions based on 1-3?

Getting it wrong

- In evaluation research, we have spent far **to much effort on measurement**, and too little on identification and valuation
- Measurement **dictates the terms** of our analysis
 - Generalizability = **societal perspective**
 - Aversion to uncertainty = **larger trials**
 - Analysis of variance = **single outcomes**
 - Psychometrics = **measuring functional status**

A case study: Schizophrenia

- CATIE: Examining effectiveness
 - Primary: All **cause treatment discontinuation**
 - Secondary: cognitive impairment, substance abuse, violence, adherence, side-effects, QoL, cost and use of services
- All endpoints measured by physician, **failing to capture patient perspective** (i.e. goals, priorities etc.) about their treatment (Manschreck and Boshes 2007)

Study 1: A qualitative approach

- Sequential **focus groups** conducted in 4 locations across Germany (n=30)
 - Review and analyze each groups results to identify themes
 - Refine the objective after each focus group for each subsequent group
- Key themes identified focused on the overall treatment experience
 - Summarized into 13 patient-relevant **“endpoints”**

Patient-relevant endpoints

Side-effects

- Weight gain
- EPS
- Increased saliva/drool
- Sleep disturbances

Functioning

- Social activities
- Sexual functioning
- Daily activities
- Clear thinking
- Listlessness

Processes

- Supportive physician
- Group therapy

Outcomes

- Minimization of symptoms
- Relapse

Study 2: A ranking approach

- Five locations across Germany (n=25)
 - Trained professional interviewer
 - Audio recorded
 - Vernacular changes across interviews
- Purpose: To determine if patients with schizophrenia have preferences for endpoints
 - Ranking exercise of 13 endpoints
 - Introduction to endpoints
 - Remove irrelevant endpoints
 - Of the relevant endpoints, rank the top 5 in order of priority

Ranking approach: Sample Card

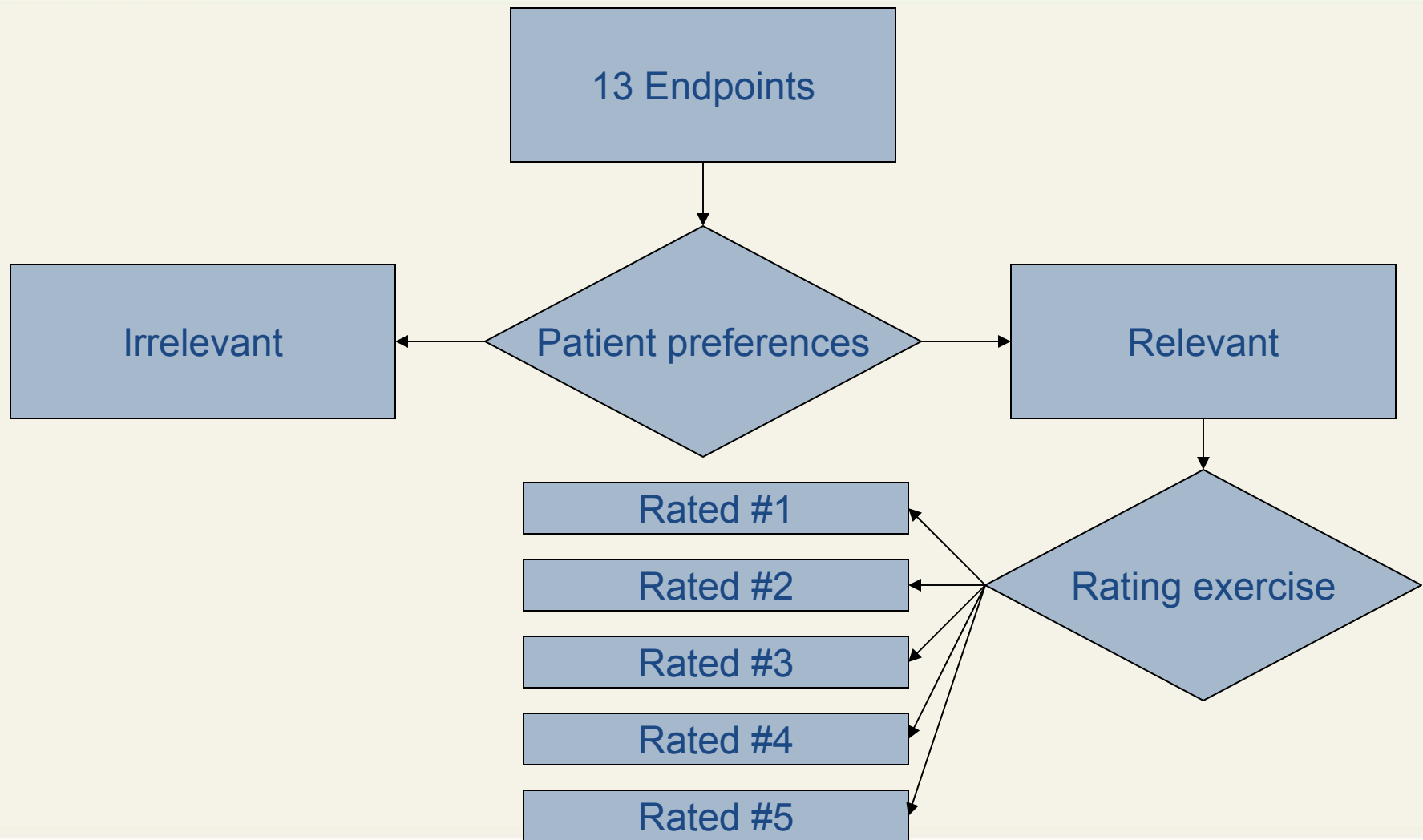
Mein Körpergewicht

Bei meiner medikamentösen Behandlung ist es schwer, mein Körpergewicht stabil zu halten.

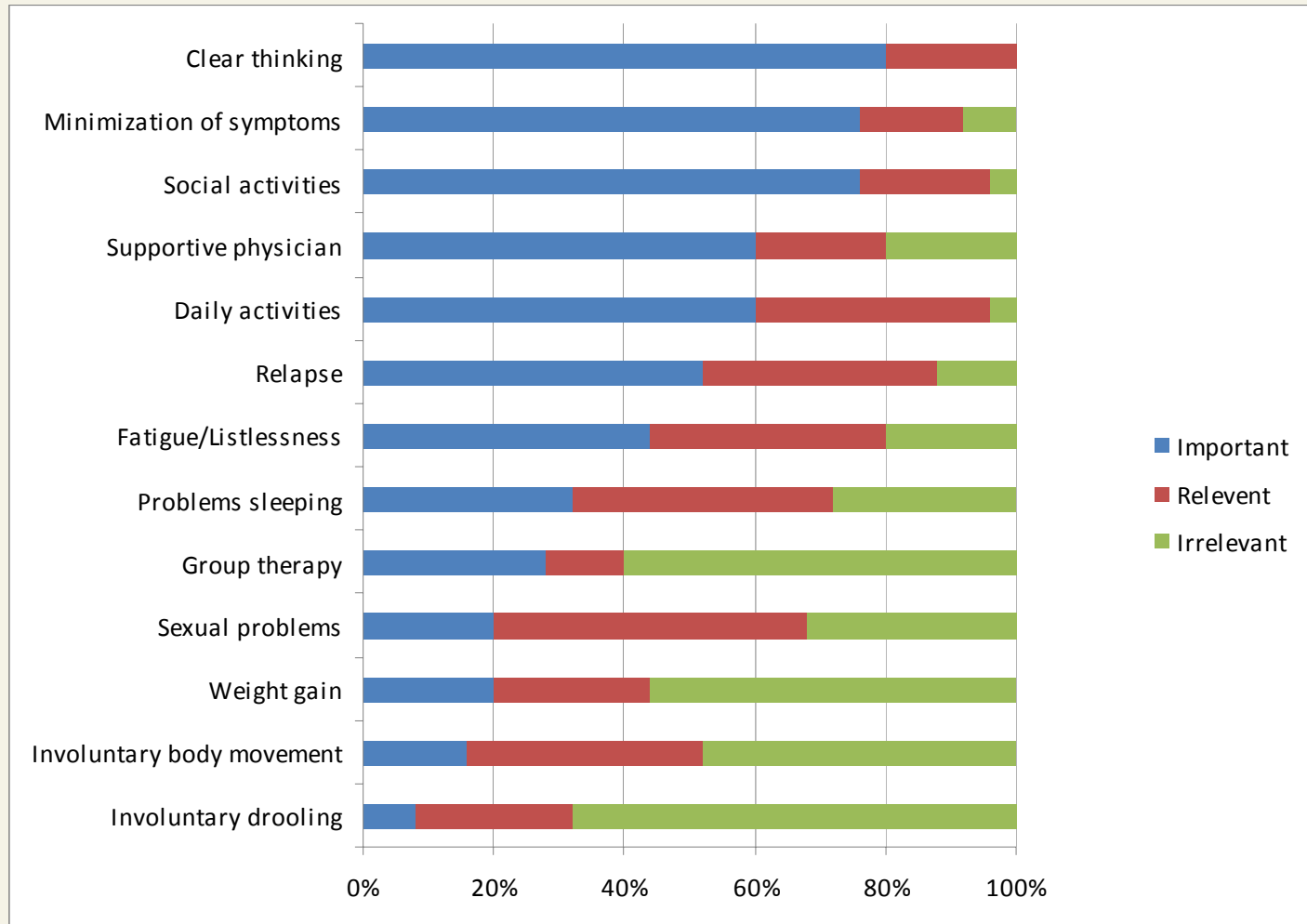
My Bodyweight

“With my medicine (medical treatment) it is hard to maintain my bodyweight.”

Ranking Exercise



Ranking Exercise Results



Case study 3: Valuing outcomes

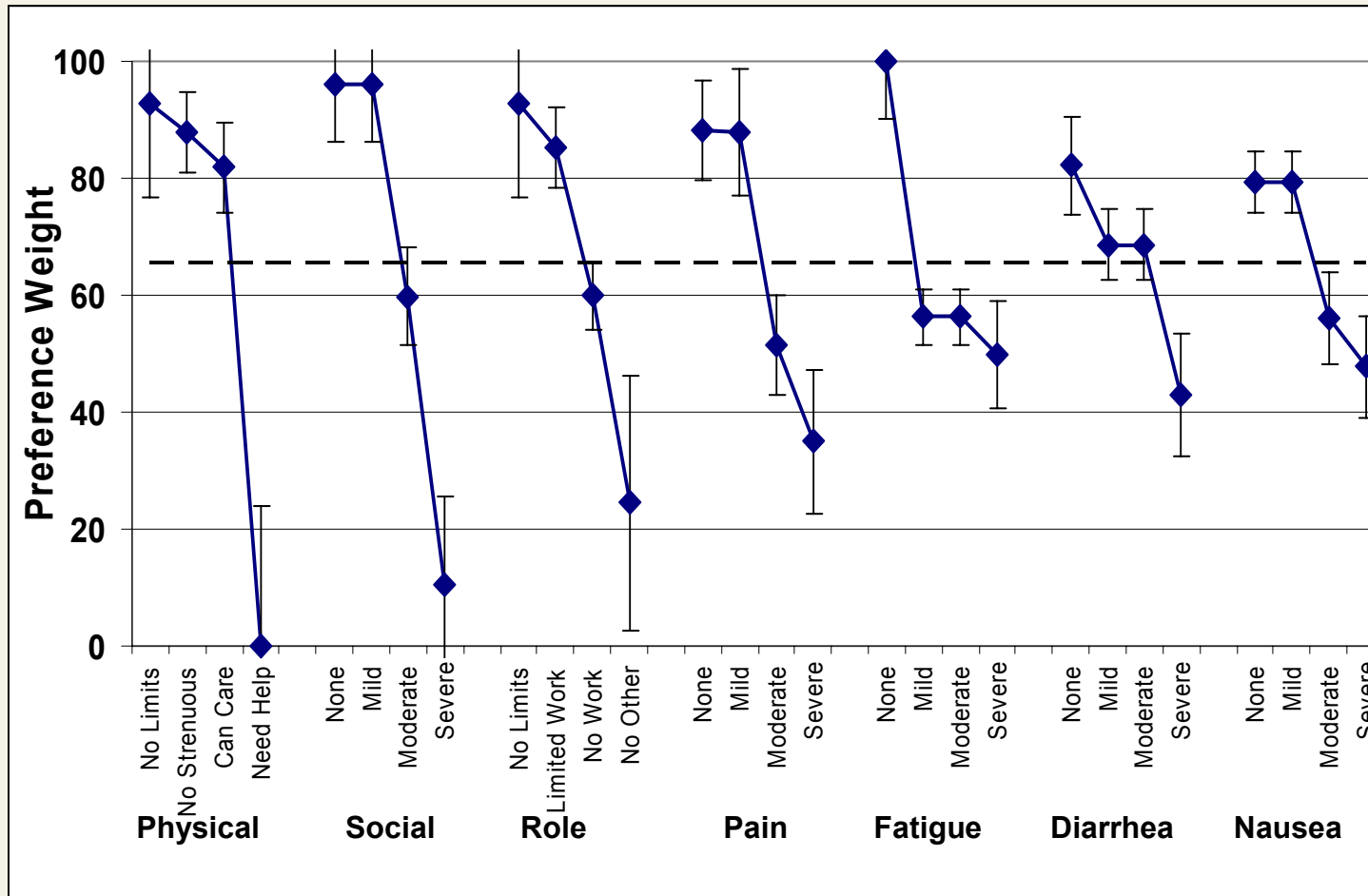
- Reed Johnson and colleagues recently examined QLQ-30 to assess the **assumption** regarding the **underlying valuation** of the scale.
- *“We have **no grounds** to believe that the EORTC QLQ items are **sufficiently non-linear** to warrant any correction before using them in summated scales. ... At the present time **we recommend** using scales based on **unweighted** summed scores.”* (EORTC QLQ-C30 Scoring Manual, 1999, p. 13)

Chemotherapy Outcome Preferences

Treatment Impact	Treatment A	Treatment B
Physical Limitations	Unable to do strenuous activities	No physical limitations
Limitations on work and household jobs	Some limitations	Some limitations
Tense, worried, irritable, or depressed	Not at all	Very much
Interference with family or social life	A little	Somewhat
Fatigue	A little (mild)	Quite a bit (moderate)
Nausea and Vomiting	Quite a bit (moderate)	A little (mild)
Trouble Sleeping	A little (mild)	Very much (severe)
Diarrhea	Quite a bit (moderate)	A little (mild)
Which treatment would you choose? (Please check <u>one</u> box.)	<i>Choose Treatment A</i>	<i>Choose Treatment B</i>

- Based on EORTC QLQ-C30 Instrument

Evaluation of weights



Sources: Johnson F.R., Hauber A.B., Osoba D., et al. Are chemotherapy patients' HRQoL importance weights consistent with linear scoring rules? A stated-choice approach. *Qual Life Res*, 2006, 15(2), 285-98.

Osoba D., Hsu M.A., Copley-Merriman C., et al. Stated preferences of patients with cancer for health-related quality-of-life (HRQOL) domains during treatment. *Qual Life Res*, 2006, 15(2), 273-83.

Why patient preferences?

- Focusing on patient preferences is **not easier**, it **more relevant** to those that we affect the most.
- Focusing on patient preferences we empower the patient (groups) by **giving them a voice**
- By measuring patient preferences using scientific methods, **we add validity** to what is normally considered the “soft” side of HTA
- By measuring patient preferences we produce **market like signals** to promote innovation in health care

The Patient

Patient-Centered Outcomes Research



JOHNS HOPKINS
BLOOMBERG
SCHOOL of PUBLIC HEALTH



Wolters Kluwer
Health

2007, Vol. 1, No. 1 (pp. 1-71)

ISSN: 1178-1653 (Print); 1178-1661 (Electronic)

The Patient

Patient-Centered Outcomes Research

Pioneer Profile

Lillie Shockney

Original Research Articles

Preferences for Prostate Cancer Screening

Patient Preferences for Exercise in Knee Osteoarthritis

Age and Choice in Health Insurance

Influence of Obesity and Race/Ethnicity on Medication Adherence

Patient Experiences with Waiting for Home/Residential Care



Wolters Kluwer | Adis
Health

Published in association with:



JOHNS HOPKINS
BLOOMBERG
SCHOOL OF PUBLIC HEALTH

Conclusions

- Evaluation metrics needs to become more **patient centered**.
- More attention has to be placed on the **identification** and **valuation** of patient endpoints.
- As such, the patients voice needs to be supported by **scientific evidence**.
- Here qualitative, ranking, rating/ranking, conjoint analysis and other **preference based** methods are beneficial research tools.

Protecting Health, Saving Lives—*Millions at a Time*

As a leading international authority on public health, the Johns Hopkins Bloomberg School of Public Health is dedicated to protecting health and saving lives. Every day, the School works to keep millions around the world safe from illness and injury by pioneering new research, deploying its knowledge and expertise in the field, and educating tomorrow's scientists and practitioners in the global defense of human life.