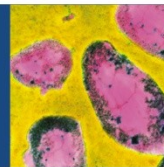
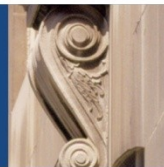
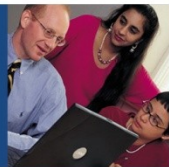




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Protecting Health, Saving Lives—*Millions at a Time*

# Processes and Tools: Measuring patient preferences

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# 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.



# The fundamentals of evaluation

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

# Getting it wrong

- In outcomes 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**

# Case study: Schizophrenia in Germany

- Schizophrenia is a **major public health problem**:
  - 24 million people globally (WHO, 2007)
  - 800,000 people in Germany affected (Kompetenznetz-Schizophrenie 2007)
- Economically **burdensome** treated or untreated
  - In Germany annual cost for the health and welfare service of schizophrenia is €4 to €9 billion per year
- Efficacy of treatment is mitigated by a multitude of factors including non-compliance, non-response and side-effects

# Choosing the wrong endpoints

- 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

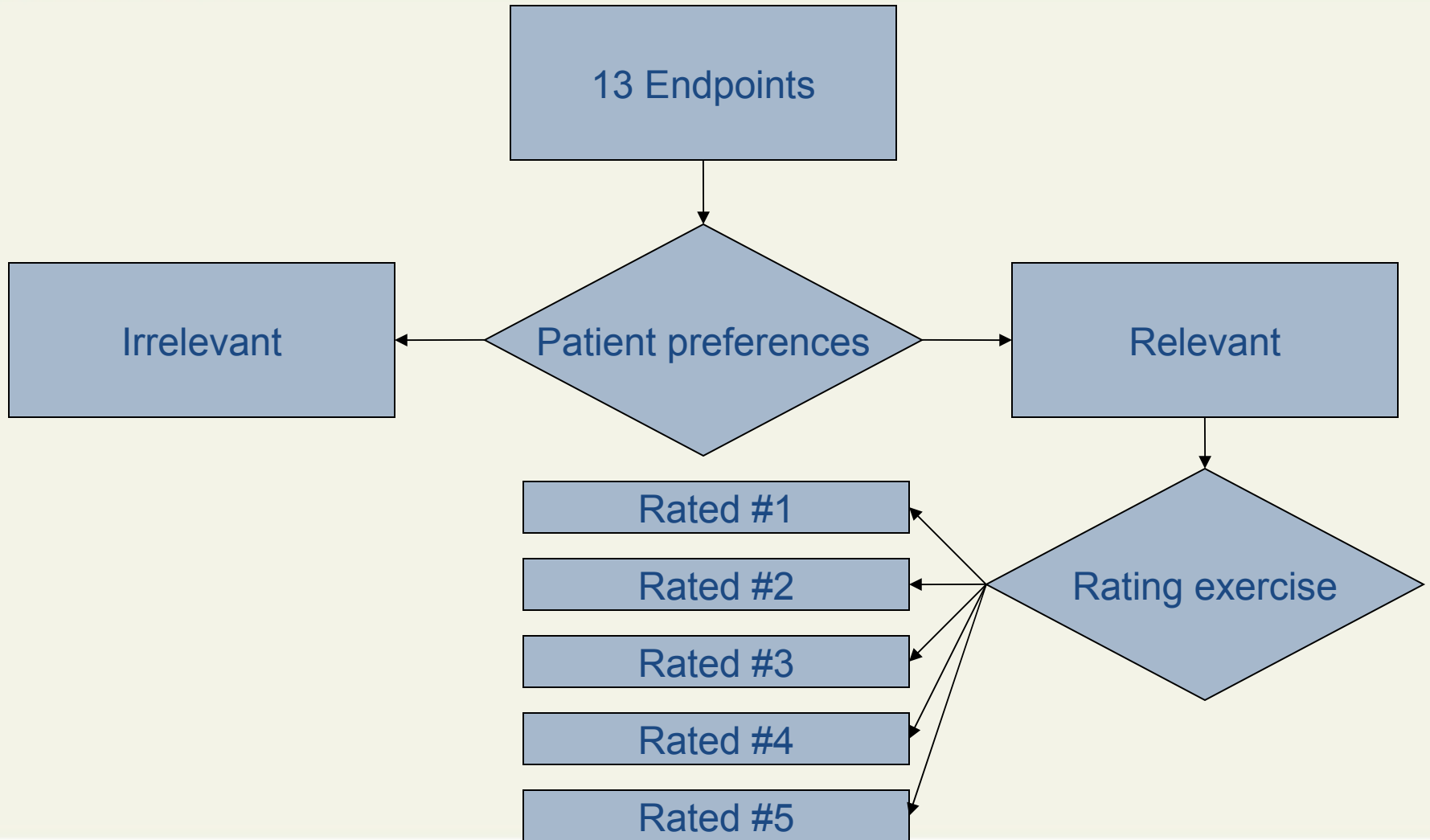
### Mein Körpergewicht

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

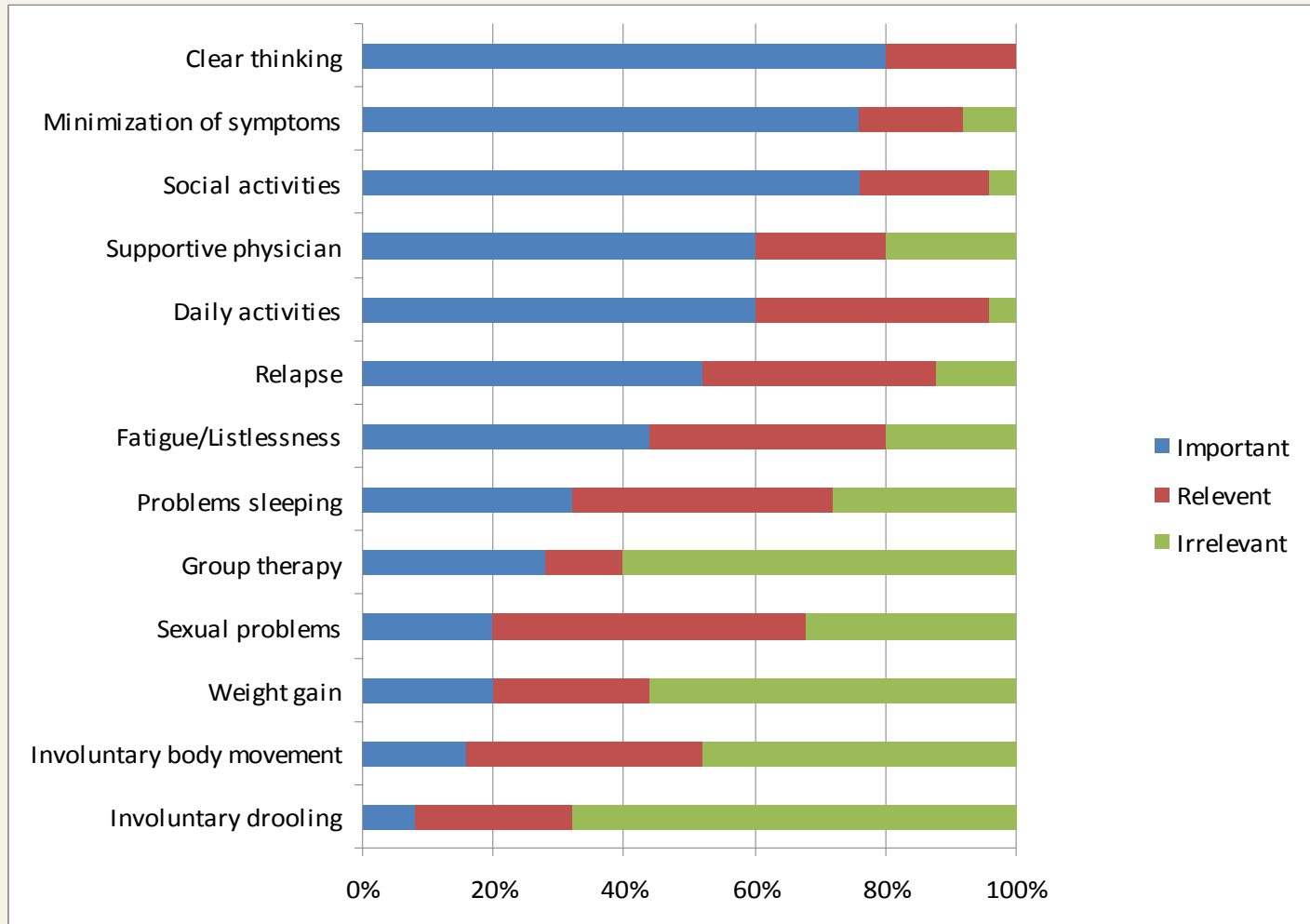
My Bodyweight

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

# Ranking Exercise



# Ranking Exercise Results



## Valuing outcomes – a case study

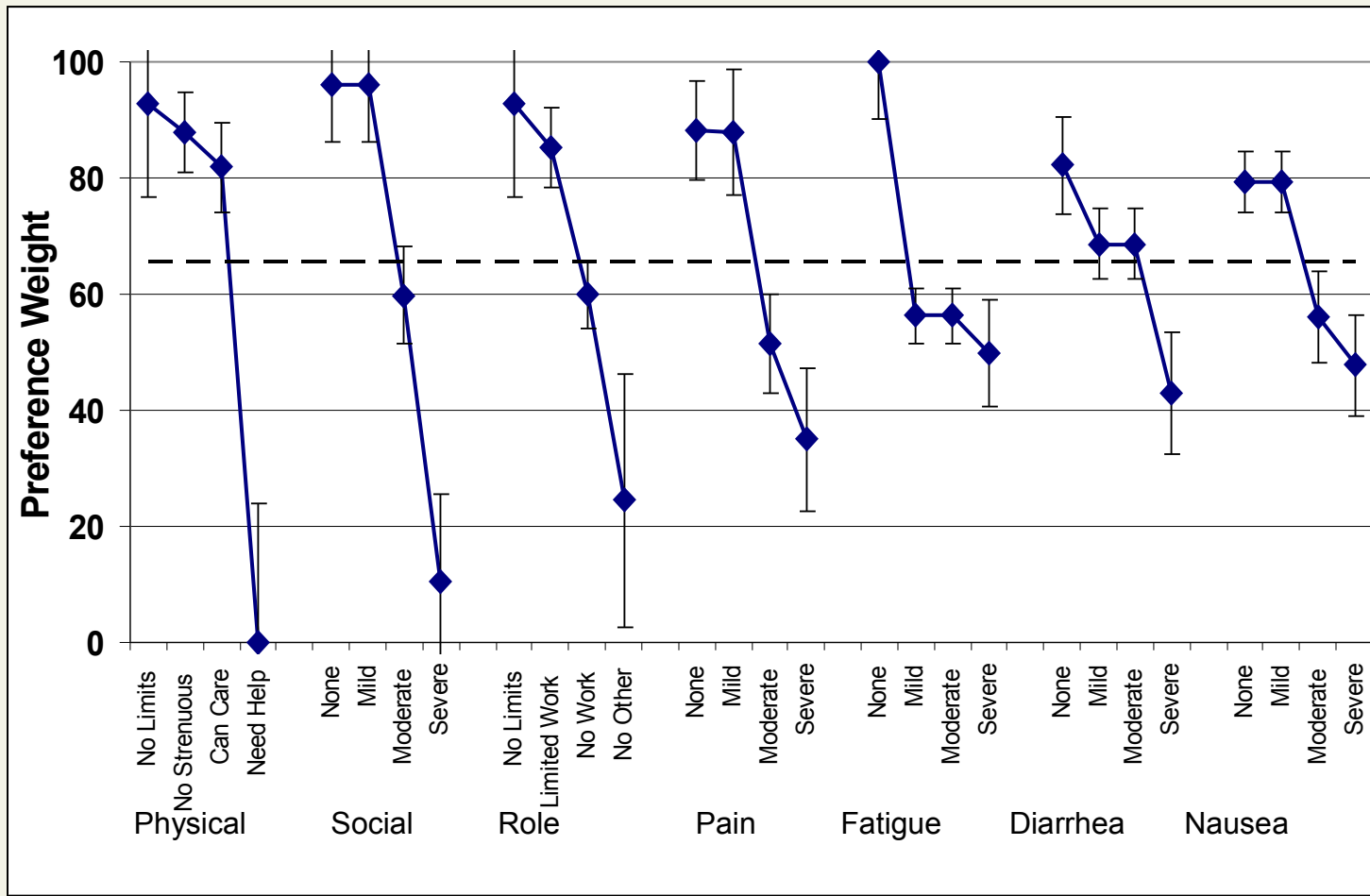
- Reed Johnson and colleagues recently examined QLQ-30 to assess the **assumption** regarding the **underlying valuation** of the scale.
- *“We have **no grounds** to believe that the EORTC QLQ items are **sufficiently non-linear** to warrant any correction before using them in summated scales. ... At 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
<b>Physical Limitations</b>	Unable to do strenuous activities	No physical limitations
<b>Limitations on work and household jobs</b>	Some limitations	Some limitations
<b>Tense, worried, irritable, or depressed</b>	Not at all	Very much
<b>Interference with family or social life</b>	A little	Somewhat
<b>Fatigue</b>	A little (mild)	Quite a bit (moderate)
<b>Nausea and Vomiting</b>	Quite a bit (moderate)	A little (mild)
<b>Trouble Sleeping</b>	A little (mild)	Very much (severe)
<b>Diarrhea</b>	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

# Conclusions

- HTA 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 in HTA needs to be supported by **scientific evidence**.
- Here qualitative, ranking, rating/ranking, conjoint analysis and other **preference based** methods are beneficial research tools.

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