

PROMIS: the US experience, or how not to reinvent the wheel

**Patient Reported Outcome Measures (PROMS)
in Cancer and Palliative Care
July 2010
Manchester, UK**

**Matthias Rose
for the PROMIS Collaborative Group**

What' s Wrong with Today' s
Outcomes Measures ?



What is Wrong with Today 's Measurement?

- not practical
 - measurement of multiple constructs may need hundreds of items & provider reports are often not available



What is Wrong with Today 's Measurement?

- not practical

- measurement of multiple constructs may need hundreds of items & provider reports are often not available



- not comparable

- same constructs are often measured with different instruments without being comparable



Not Comparable

<i>Concepts and Characteristics</i>	<i>Psychometric</i>							<i>Utility Related</i>				
	SIP	HIE	NHP	QLI	COOP	DUKE	MOS FWBP	MOS SF-36	QWB	EURO -QOL	HUI	SF-6D
CONCEPTS												
Physical functioning	●	●	●	●	●	●	●	●	●	●	●	●
Social functioning	●	●	●	●	●	●	●	●	●	●		●
Role functioning	●	●	●	●	●	●	●	●	●	●		●
Psychological distress	●	●	●	●	●	●	●	●		●	●	●
Health perceptions (general)		●	●	●	●	●	●	●				
Pain (bodily)		●	●		●	●	●	●		●	●	●
Energy/fatigue		●	●			●	●	●	●			●
Psychological well-being		●				●	●	●				
Sleep	●		●			●	●					
Cognitive functioning	●					●	●				●	
Quality of life		●			●		●					
Reported health transition					●		●	●				

SIP = Sickness Impact Profile (1976)

HIE = Health Insurance Experiment surveys (1979)

NHP = Nottingham Health Profile (1980)

QLI = Quality of Life Index (1981)

COOP = Dartmouth Function Charts (1987)

DUKE = Duke Health Profile (1990)

MOS FWBP = MOS Functioning and Well-Being Profile (1992)

MOS SF-36 = MOS 36-Item Short-Form Health Survey (1992)

QWB = Quality of Well-Being Scale (1973)

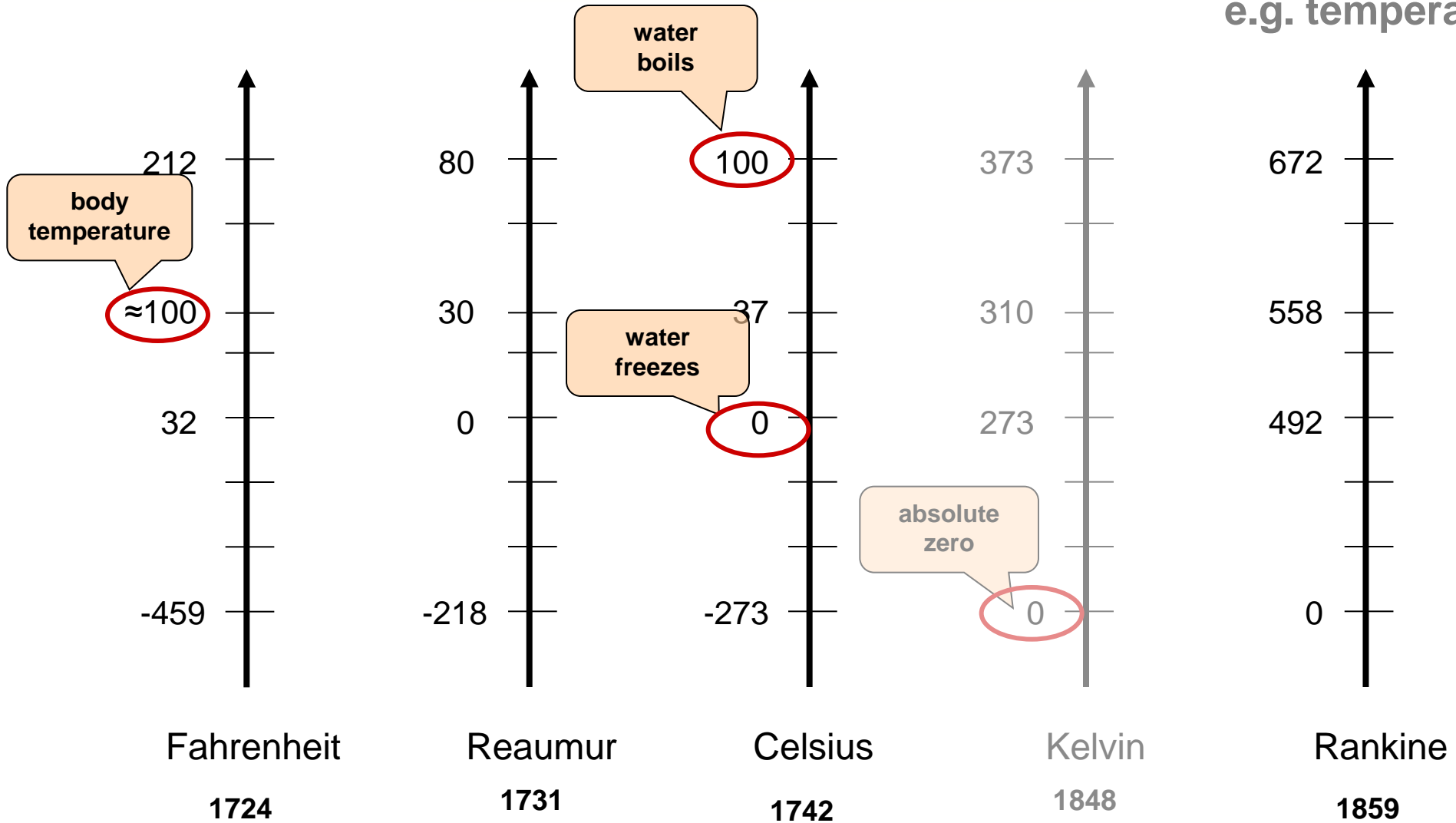
EUROQOL = European Quality of Life Index (1990)

HUI = Health Utility Index (1996)

SF-6D = SF-36 Utility Index (Brazier, 2002)

Not Anchored

e.g. temperature



What is Wrong with Today 's Measurement?

- **not practical**

- measurement of multiple constructs may need hundreds of items & provider reports are often not available



- **not comparable, not anchored**

- same constructs are often measured with different instruments without being comparable



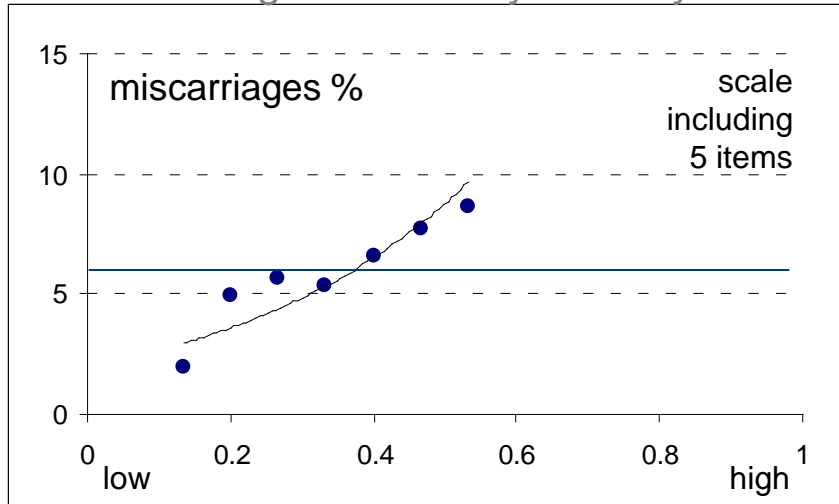
- **restricted measurement range**

- clinical instruments do not provide information for the general population

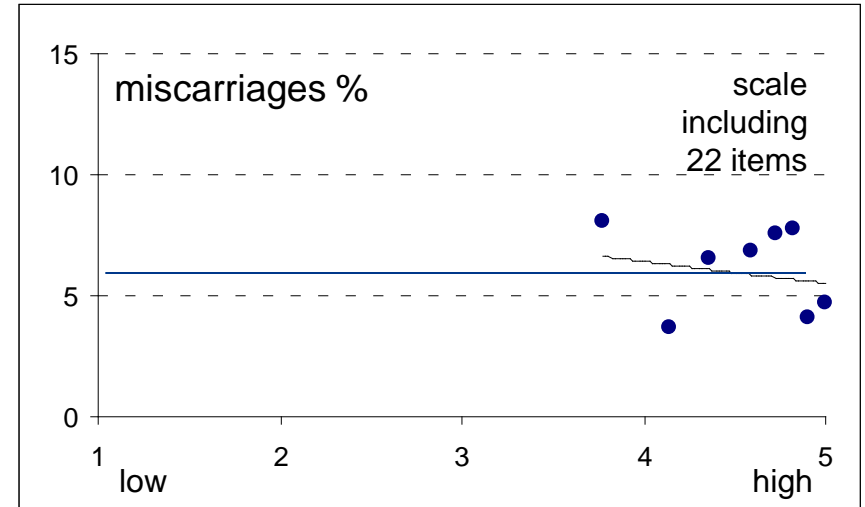


Restricted Range

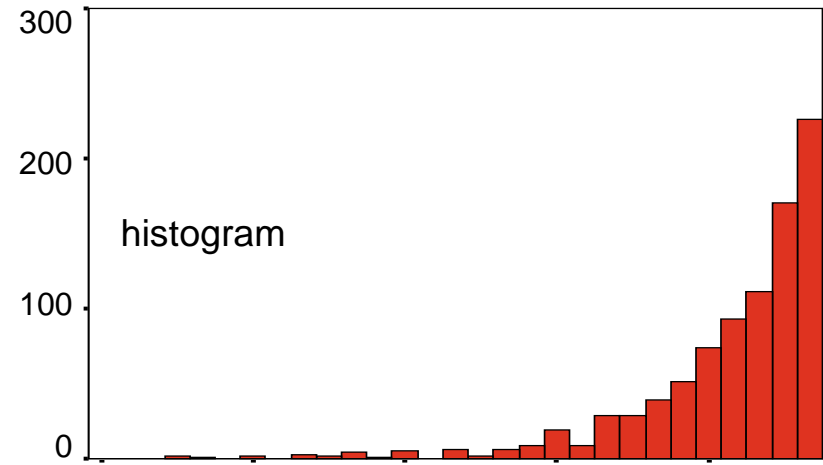
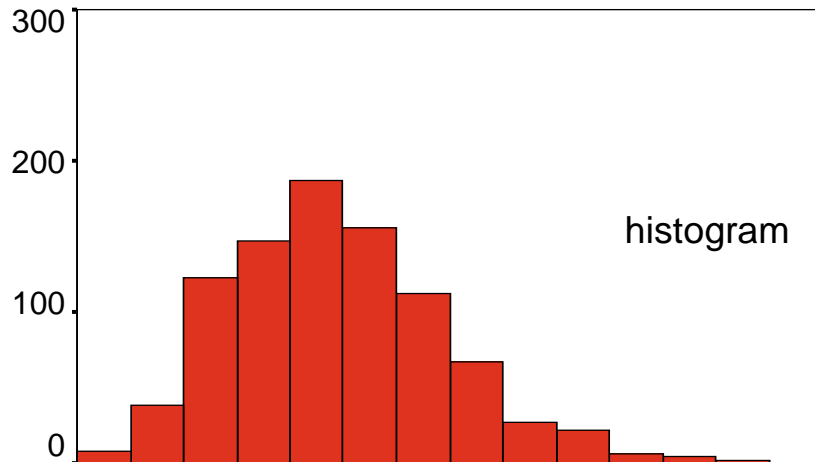
n=1.052 longitudinal study over 5 years



perceived stress



perceived social support



What is Wrong with Today 's

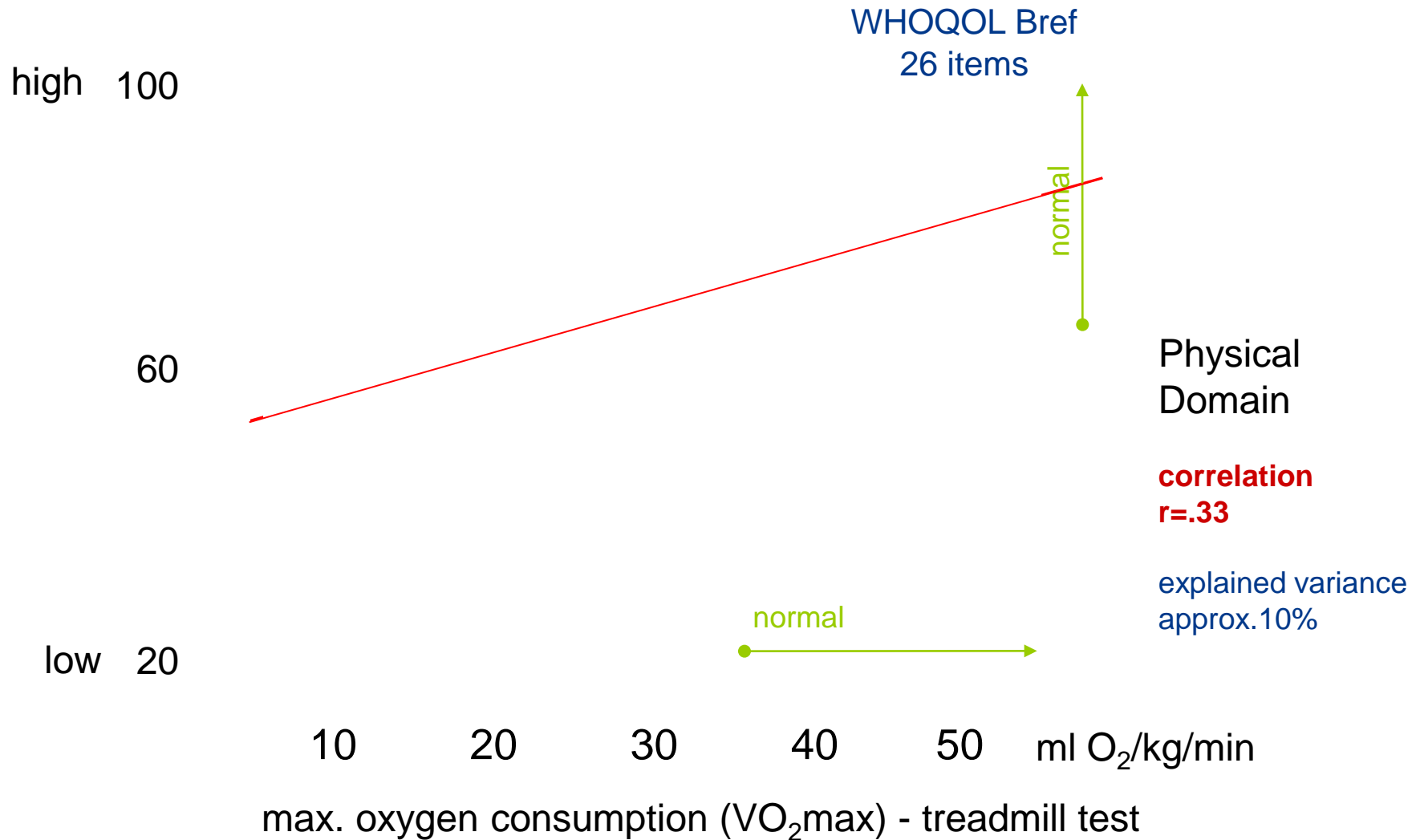
Measurement?

- not practical
 - measurement of multiple constructs may need hundreds of items & provider reports are often not available
- not comparable
 - same constructs are often measured with different instruments without being comparable
- restricted measurement range
 - clinical instruments do not provide information for the general population
- not precise
 - measurement precision does not meet required standards for individual clinical decision making

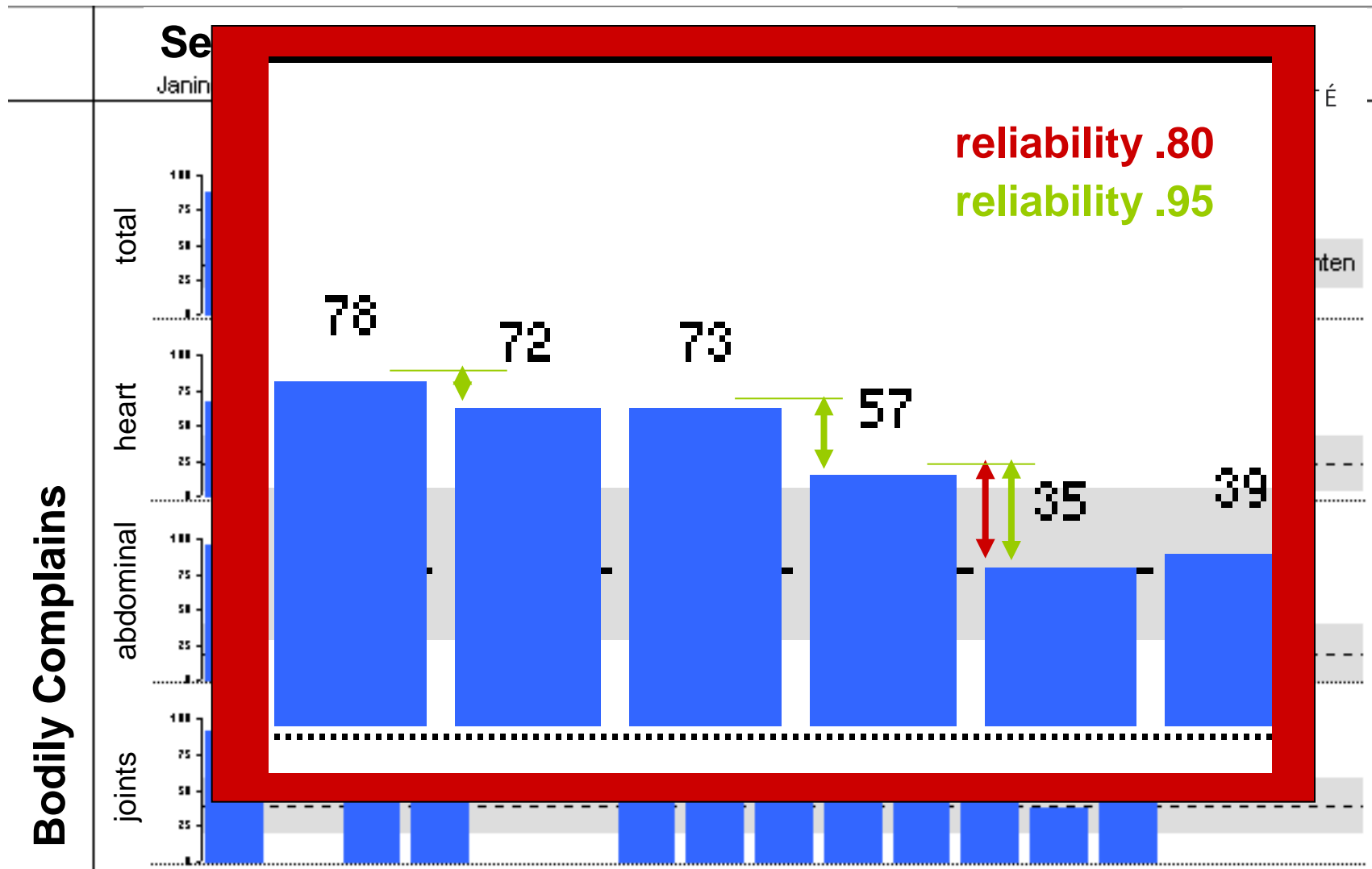


Cardio/Pulmonary Function & Well-Being

n=112 patients with congenital heart failure



Precision & Clinical Decision Making



$SEM = SD * (1-\rho)^{1/2}$ $CI = x \pm (1.96 * SEM)$

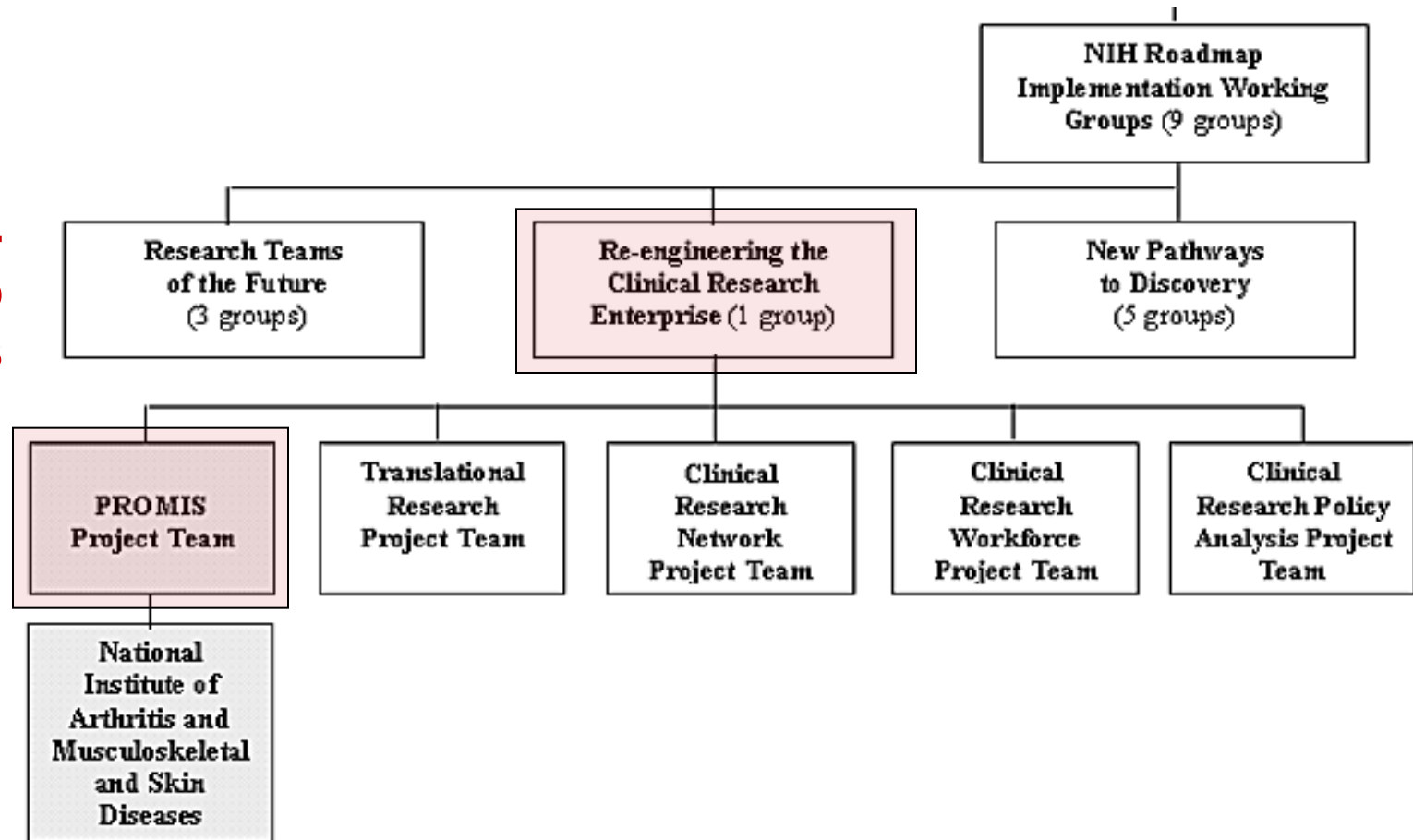
reliability .80 vs .95: Twice the change score is required to indicate true change

Can We Do Better ?
Without Building Just Another
Tool !



Position of PROMIS at the NIH

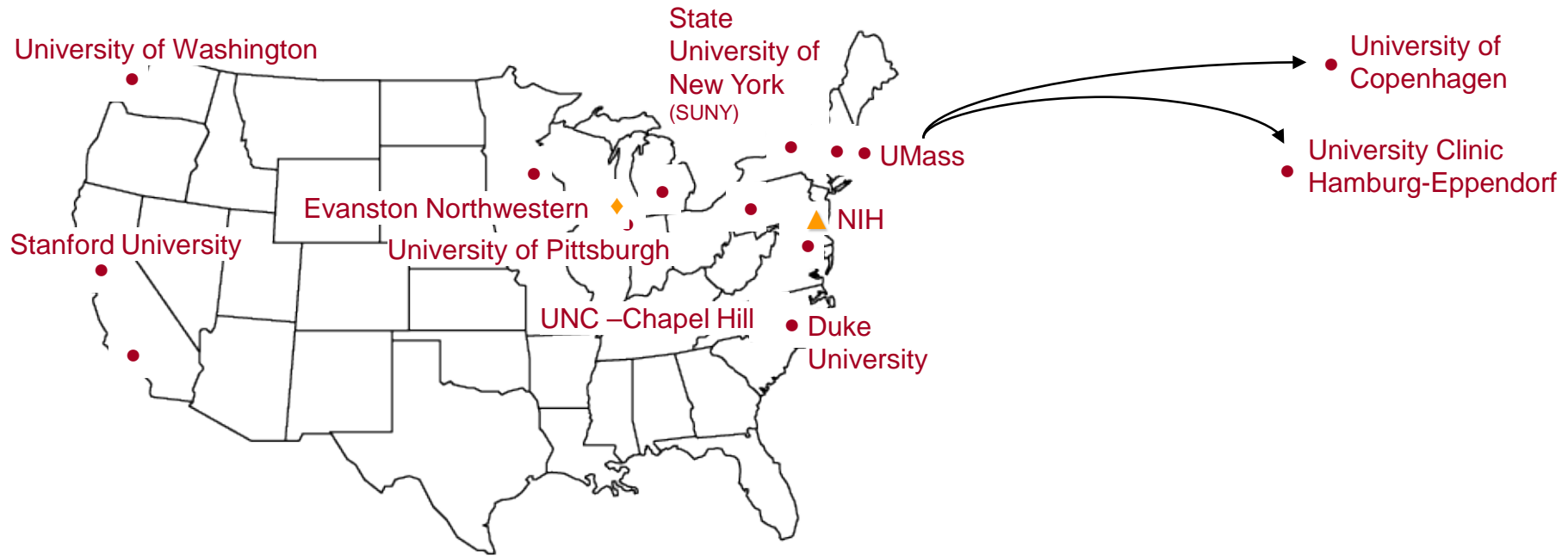
**2004
Key Roadmap
Initiatives**





Patient-Reported Outcomes Measurement Information System

Dynamic Tools to Measure Health Outcomes From the Patient Perspective



US-National Institutes of Health (NIH)
 Key-Road-Initiative
 “Re-engineering the Clinical Research Enterprises”

Phase 1
 2004-2009
 Phase 2
 2010-2013

\$60+ mio

www.nihpromis.org/

Rational of the NIH Initiative

NIH RFA RM04-011

Long-term objective ...

- Facilitate a better integration of PROMs for the assessment of health outcomes ...

Mid-term objective ...

- More precise and less burdensome PROMs
- Standardized assessments
- Up- and Downwards
- Tools useful for epidemic practice at the same

Item Response Theory (IRT)
Item Banks
&
Computer Adaptive Tests (CAT)

Steps towards an IRT Item Bank

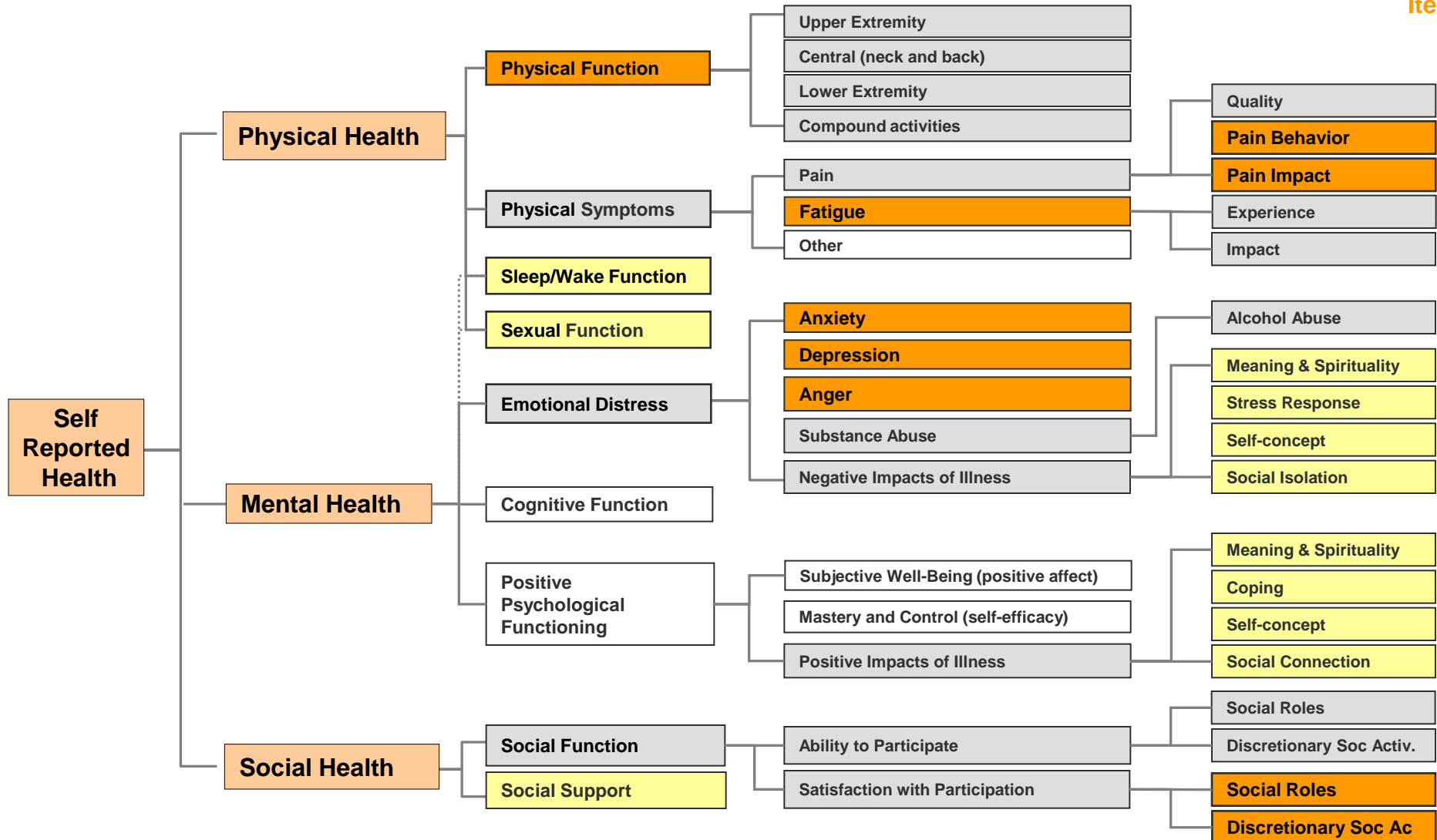
Construct Definition

1

Literature Review/Consensus

PROMIS Health Domain Framework

Available PROMIS
Item Banks



Steps towards an Item Bank

Construct Definition

1

Literature Review/Consensus

Item Construction

2

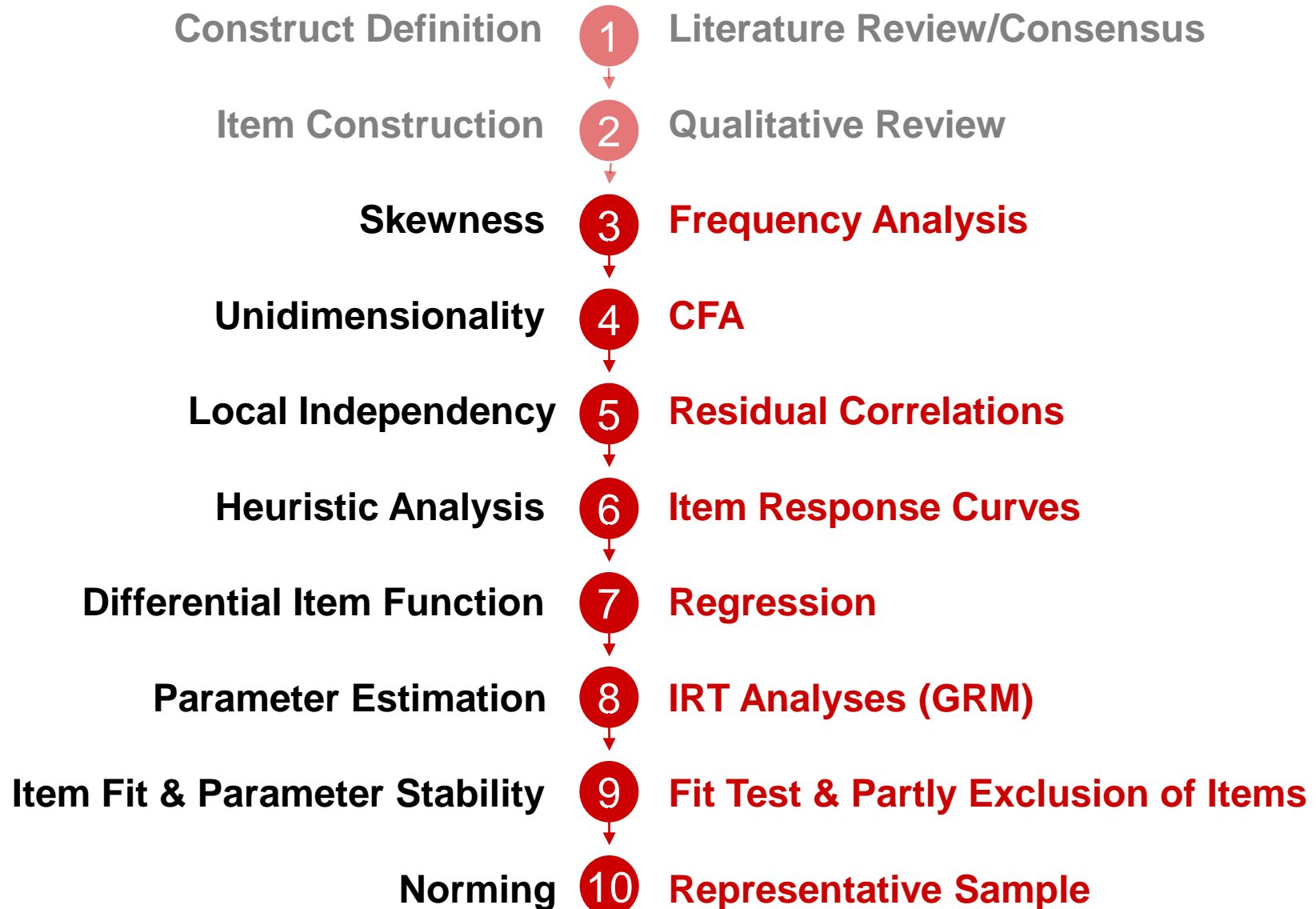
Qualitative Review

- @ 8,000 items reviewed
- binning and winnowing to 1,064 items
- 28 focus groups
- 784 cognitive interviews

Steps towards an Item Bank



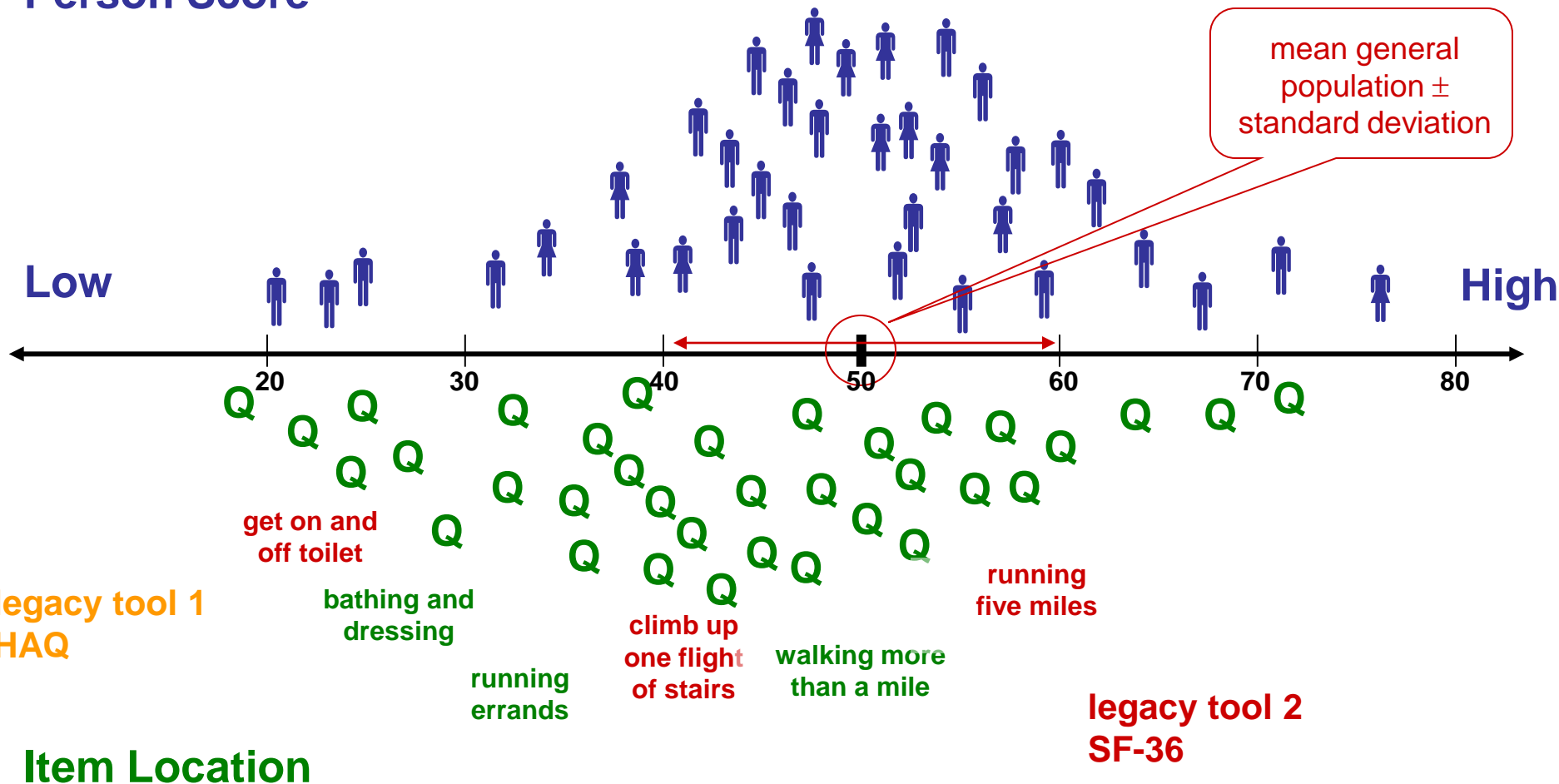
Steps towards an Item Bank



“Item Response Theory” (IRT) Item Bank

e.g. for Physical Function

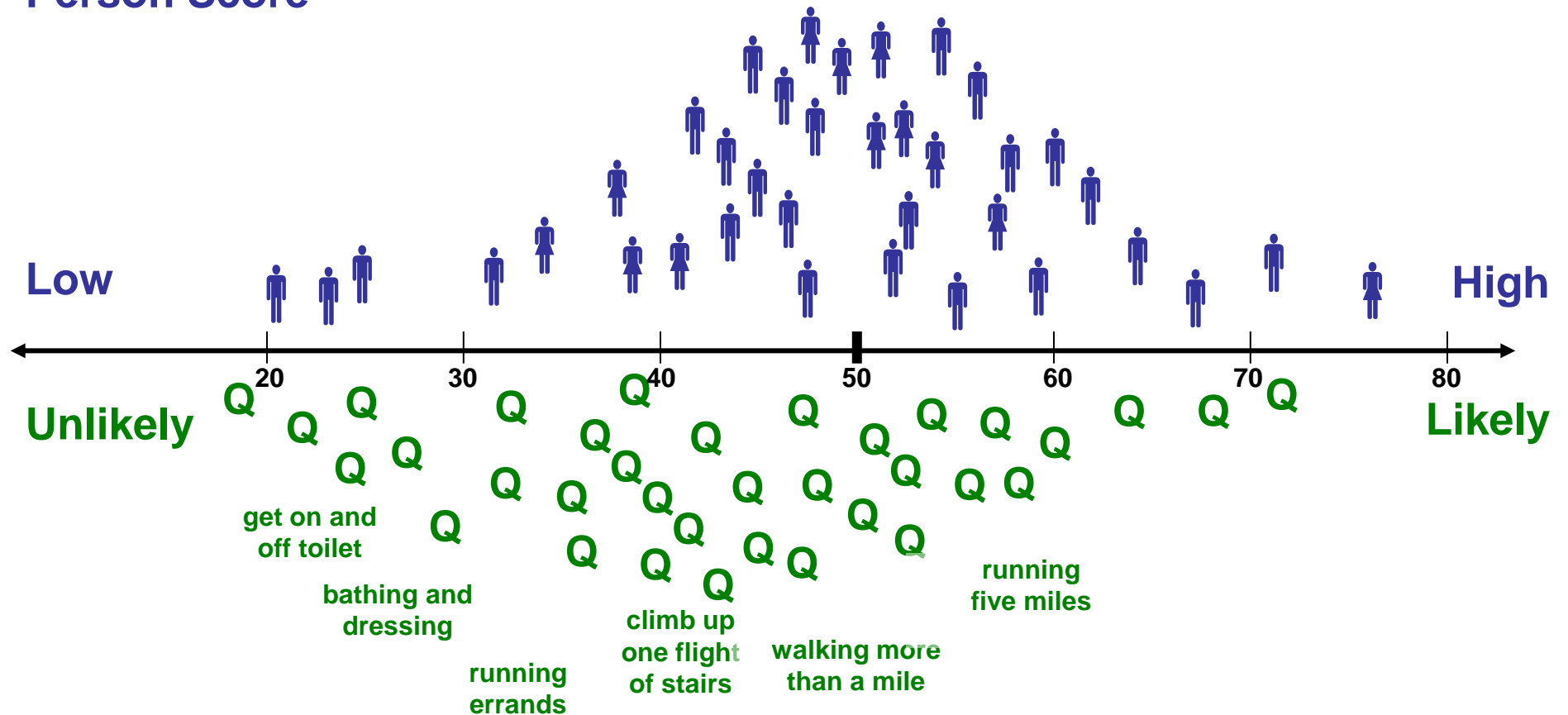
Person Score



“Item Response Theory” (IRT) Item Bank

e.g. for Physical Function

Person Score

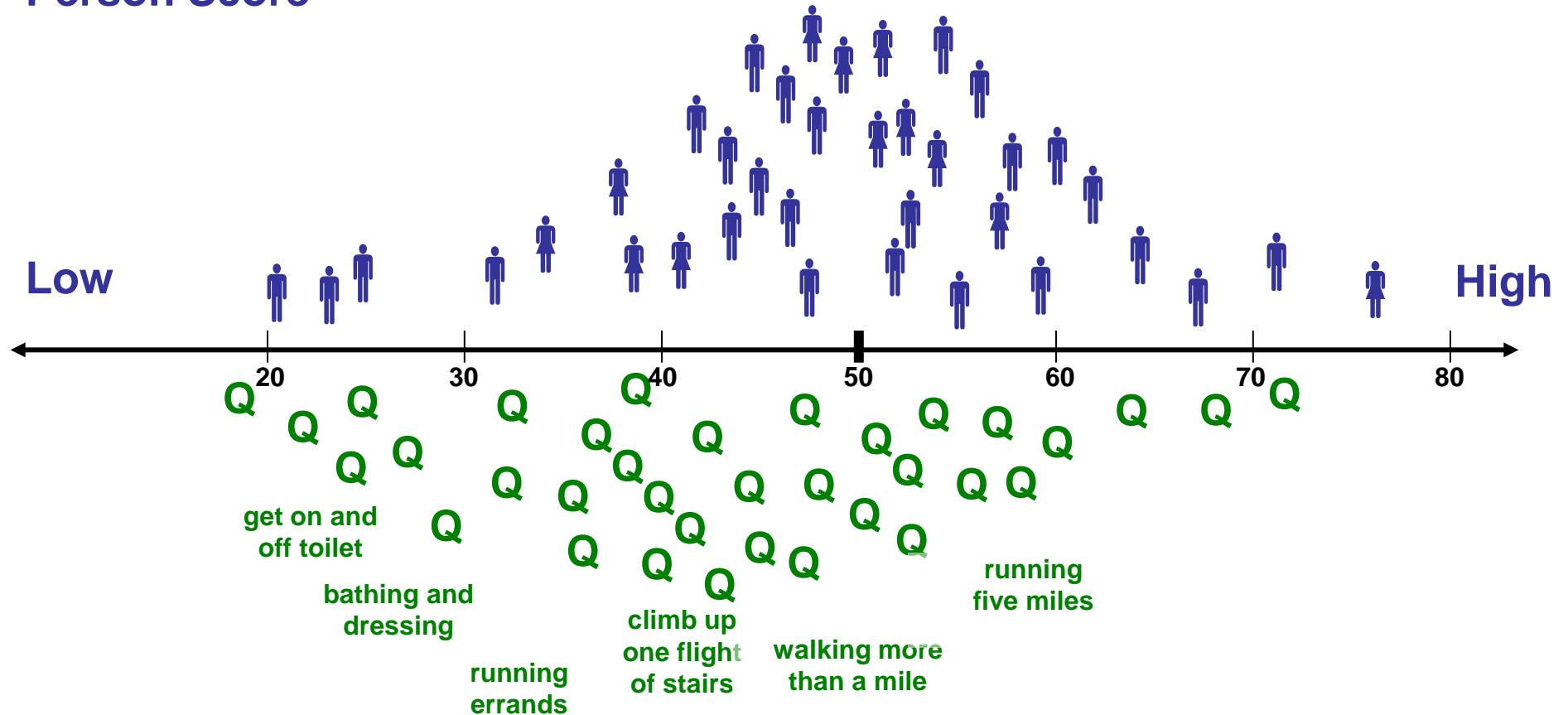


The patient with a score of **60** has a significant higher physical function than the average.
Persons with those score typically have no problem running five miles ...

“Item Response Theory” (IRT) Item Bank

e.g. for Physical Function

Person Score

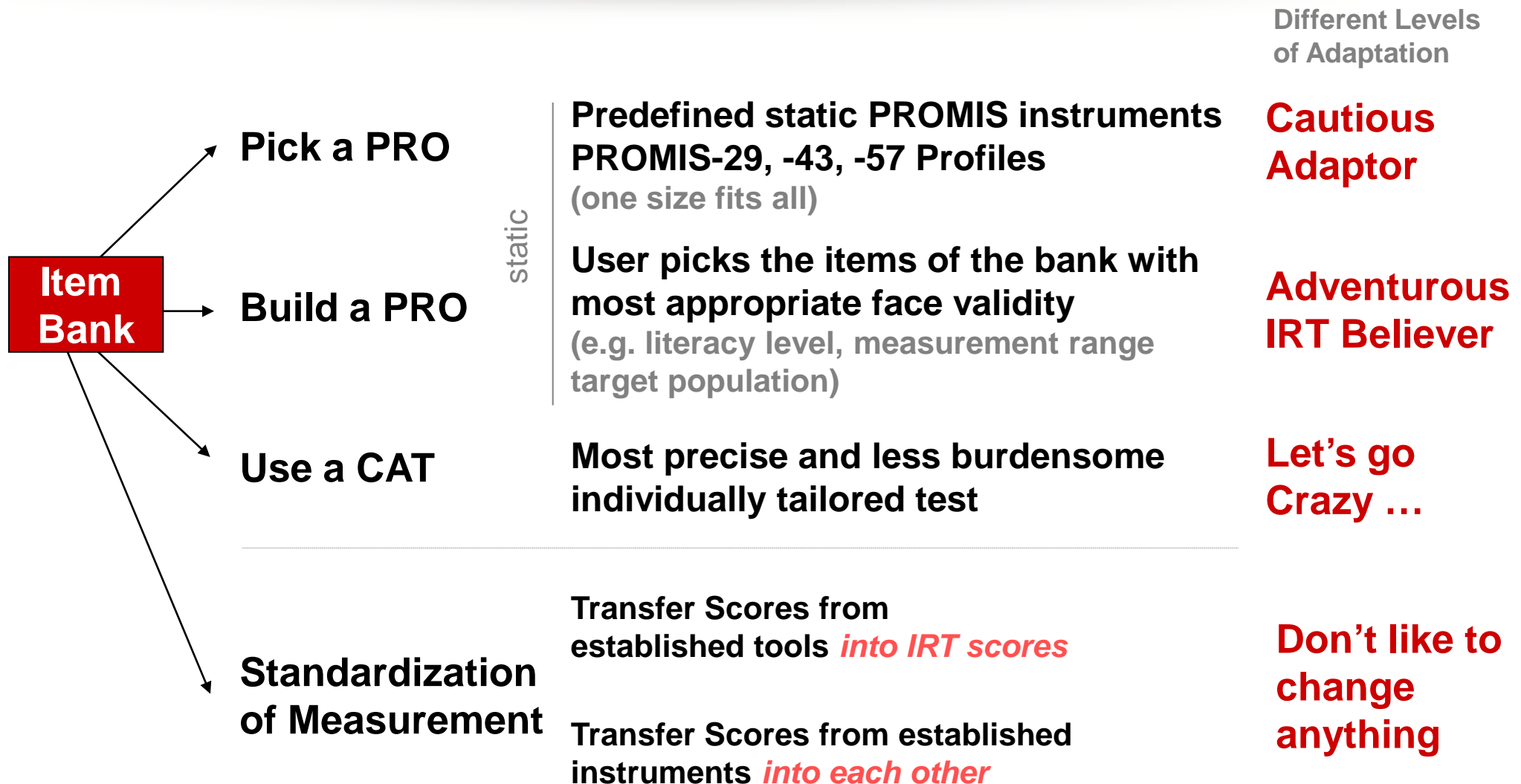


The patient with a score of **32** has a highly significant lower physical function than the average person. Persons with those score typically have difficulties bathing and dressing, running errands ...

PROMIS Approach



PROMIS Tools



Pick a PRO

PROMIS 29 Profile

Please respond to each question or statement by marking one box per row.

		Without any difficulty	With a little difficulty	With some difficulty	With much difficulty	Unable to do
1	Are you able to do chores such as vacuuming or yard work?.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2	Are you able to go up and down stairs at a normal pace?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3	Are you able to go for a walk of at least 15 minutes?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4	Are you able to run errands and shop?.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	In the past 7 days...	Never	Rarely	Sometimes	Often	Always
5	I felt fearful.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
6	I found it hard to focus on anything other than my anxiety	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
7	My worries overwhelmed me.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
8	I felt uneasy	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Pick a PRO

PROMIS 29 Profile

Please respond to each question or statement by marking one box per row.

	In the past 7 days...	Never	Rarely	Sometimes	Often	Always
9	I felt worthless	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
10	I felt helpless	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
11	I felt depressed	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
12	I felt hopeless	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
	In the past 7 days...	Not at all	A little bit	Somewhat	Quite a bit	Very much
13	I feel fatigued	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
14	How fatigued were you on average?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
15	How run-down did you feel on average? ...	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
16	I have trouble <u>starting</u> things because I am tired.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Pick a PRO

PROMIS 29 Profile

	In the past 7 days...	Not at all	A little bit	Somewhat	Quite a bit	Very much						
21	I am satisfied with how much work I can do (include work at home).....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
22	I am satisfied with my ability to work (include work at home).....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
23	I am satisfied with my ability to do regular personal and household responsibilities	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
24	I am satisfied with my ability to perform my daily routine	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
	In the past 7 days...											
25	How would you rate your pain on average?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	
		0	1	2	3	4	5	6	7	8	9	10
		No pain										Worst imaginable pain
	In the past 7 days...	Not at all	A little bit	Somewhat	Quite a bit	Very much						
26	How much did pain interfere with your day to day activities?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
27	How much did pain interfere with work around the home?.....	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						
28	How much did pain interfere with your ability to participate in social activities?	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>						

Build a PRO

Depression Item Bank 28 items

ID	Stem
EDDEP03	I felt that I had no energy
EDDEP04	I felt worthless
EDDEP05	I felt that I had nothing to look forward to
EDDEP06	I felt helpless
EDDEP07	I withdrew from other people
EDDEP09	I felt that nothing could cheer me up
EDDEP13	I felt that other people did not understand me
EDDEP14	I felt that I was not as good as other people
EDDEP16	I felt like crying
EDDEP17	I felt sad
EDDEP19	I felt that I wanted to give up on everything
EDDEP21	I felt that I was to blame for things
EDDEP22	I felt like a failure
EDDEP23	I had trouble feeling close to people
EDDEP26	I felt disappointed in myself
EDDEP27	I felt that I was not needed
EDDEP28	I felt lonely
EDDEP29	I felt depressed
EDDEP30	I had trouble making decisions
EDDEP31	I felt discouraged about the future
EDDEP35	I found that things in my life were overwhelming
EDDEP36	I felt unhappy
EDDEP39	I felt I had no reason for living
EDDEP41	I felt hopeless
EDDEP42	I felt ignored by people
EDDEP44	I felt upset for no reason
EDDEP45	I felt that nothing was interesting
EDDEP46	I felt pessimistic
EDDEP48	I felt that my life was empty
EDDEP50	I felt guilty
EDDEP54	I felt emotionally exhausted
EDDEP56	I had trouble enjoying things that I used to enjoy

Build a PRO

Three PROMIS Domains and Associated Main Subdomains

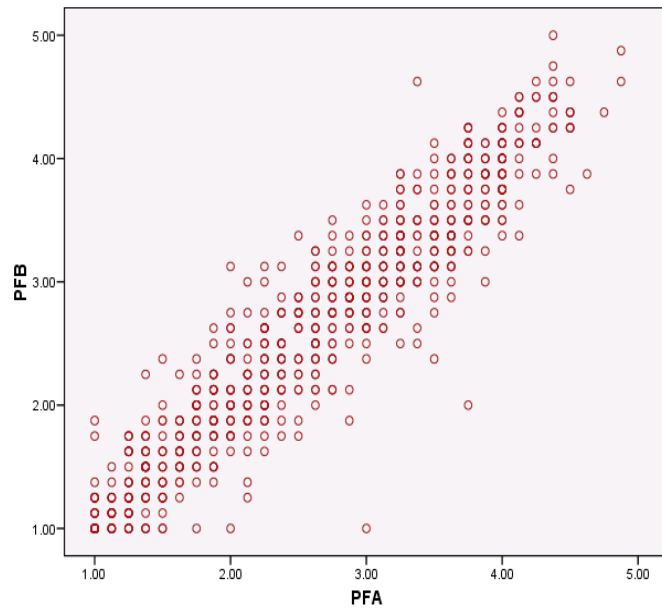
Domain	Subdomain	Number of Items	
		Item Bank	Short form
Emotional Distress- Depression	Mood	12	4
	Cognition	8	2
	Self worth	8	2
Fatigue	Experience	36	4
	Impact	47	4
Physical Functioning	Central	5	1
	IADL	40	3
	Lower	40	2
	Upper	40	2

created parallel forms (A and B) using IRT methods for each domain

Build a PRO

n=912 chronically ill patients

Physical Function

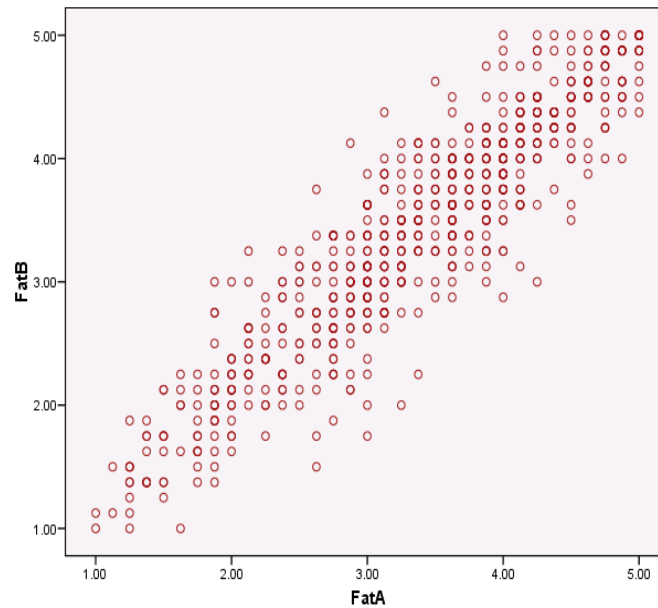


ICC = .92

r = .92

Cronbach α Form A/B = .93/.93

Fatigue

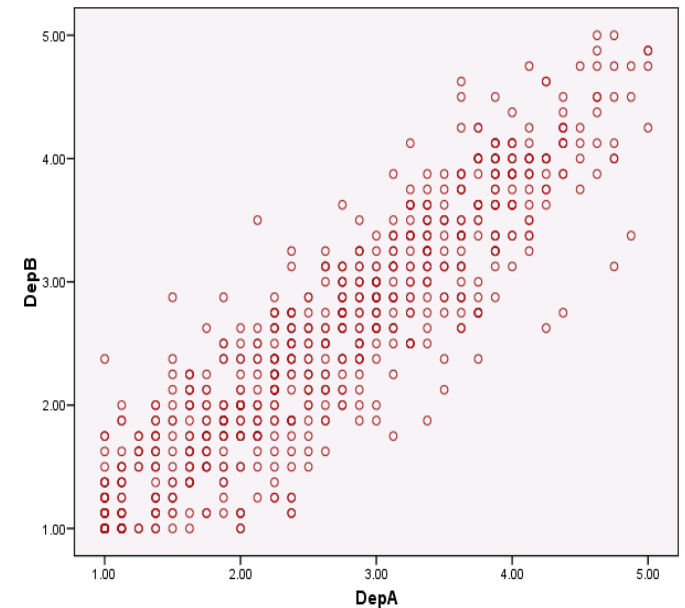


ICC = .92

r = .92

Cronbach α Form A/B = .96/.96

Depression



ICC = .90

r = .90

Cronbach α Form A/B = .95/.94

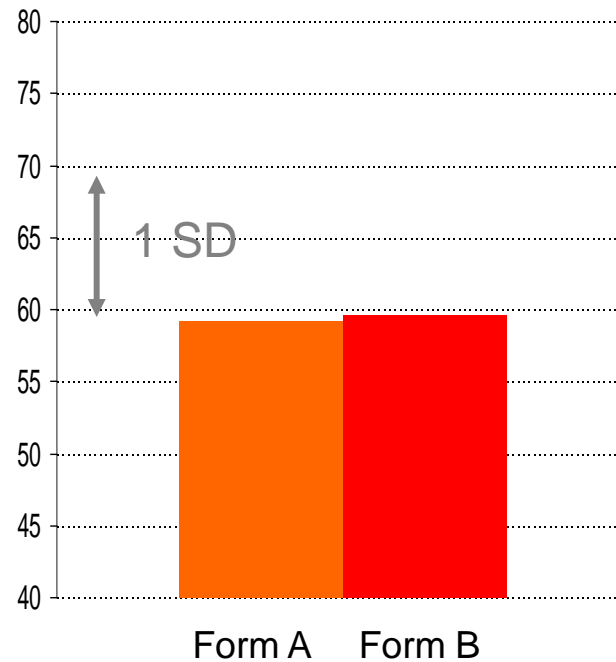
Build a PRO

n=912 chronically ill patients

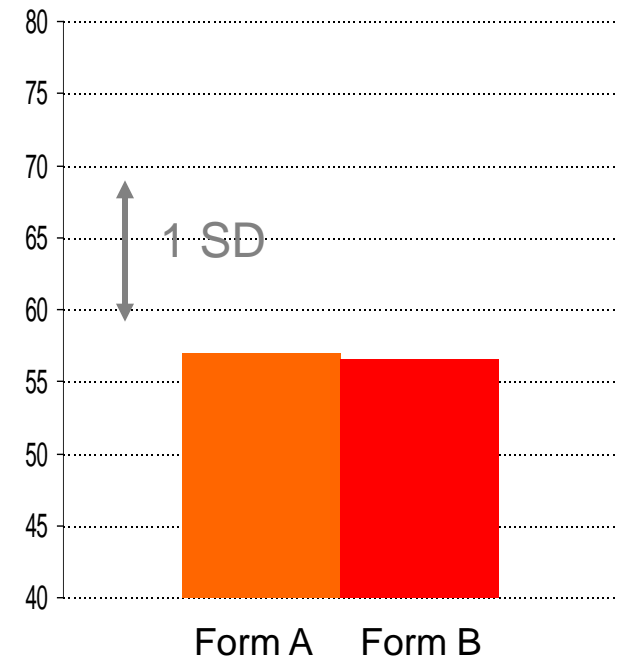
Physical Function



Fatigue



Depression

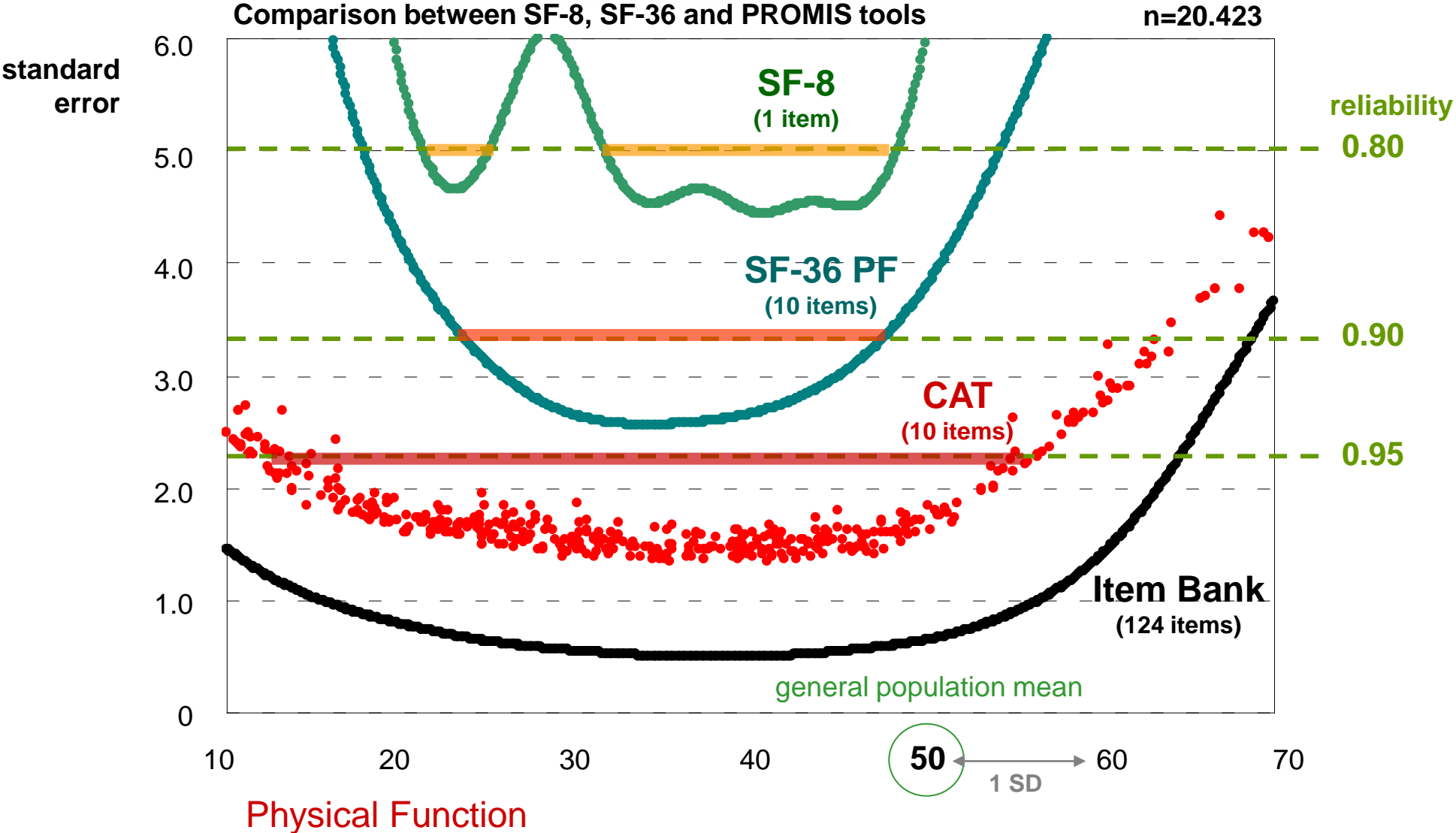


no relevant mean differences
between different short forms

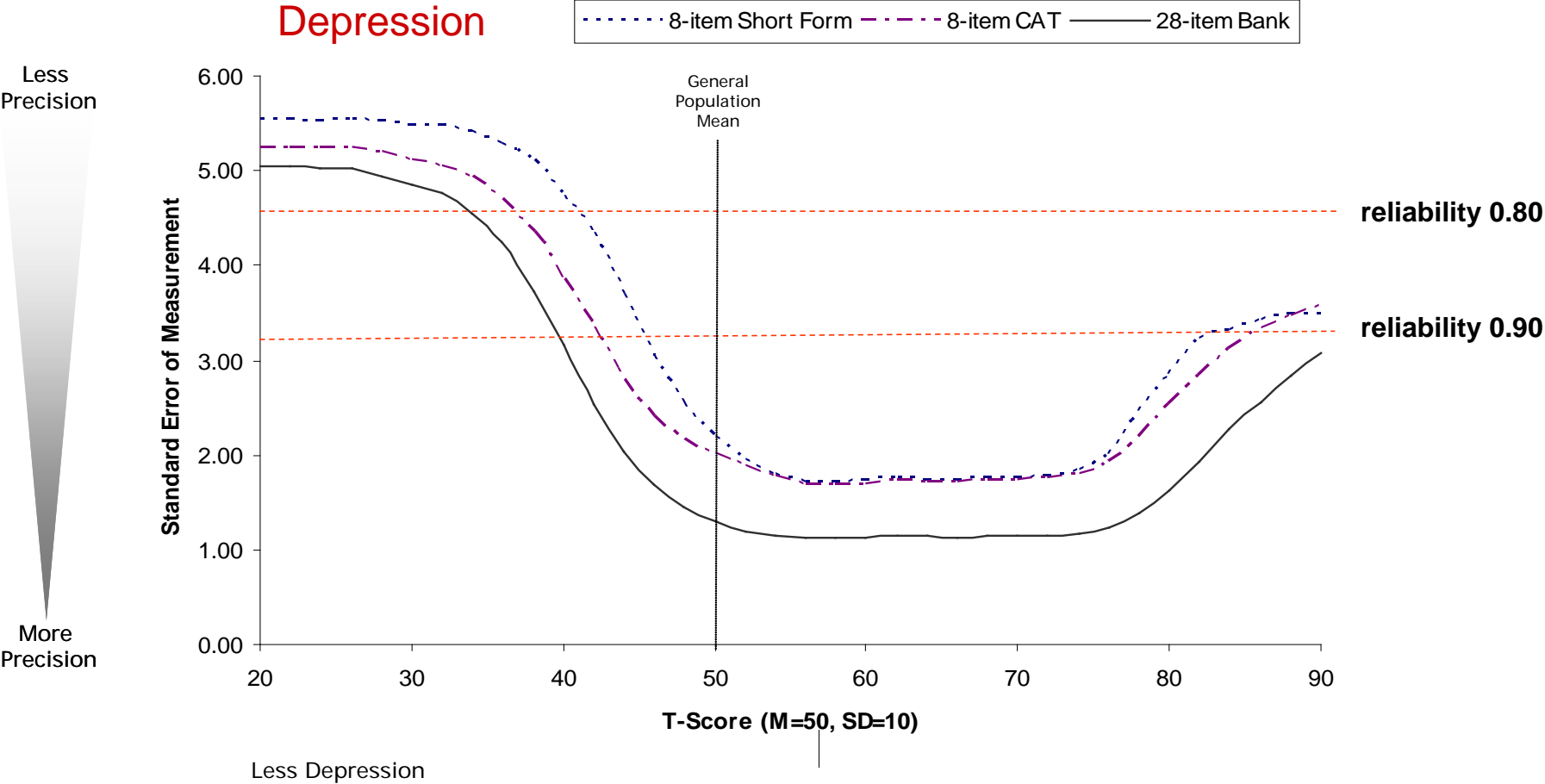
Computer Adaptive Tests



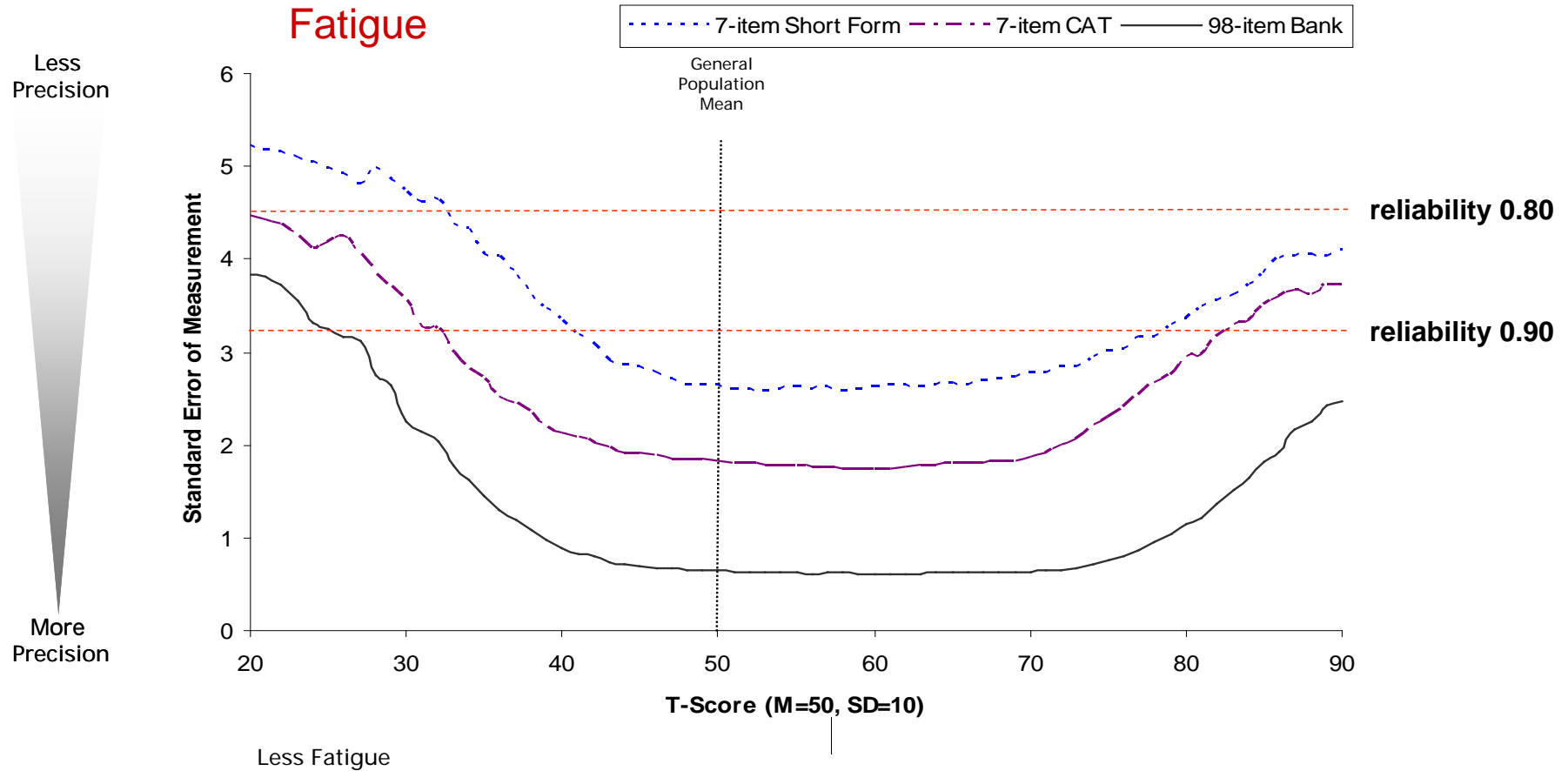
Measurement Precision and Range



Measurement Precision and Range

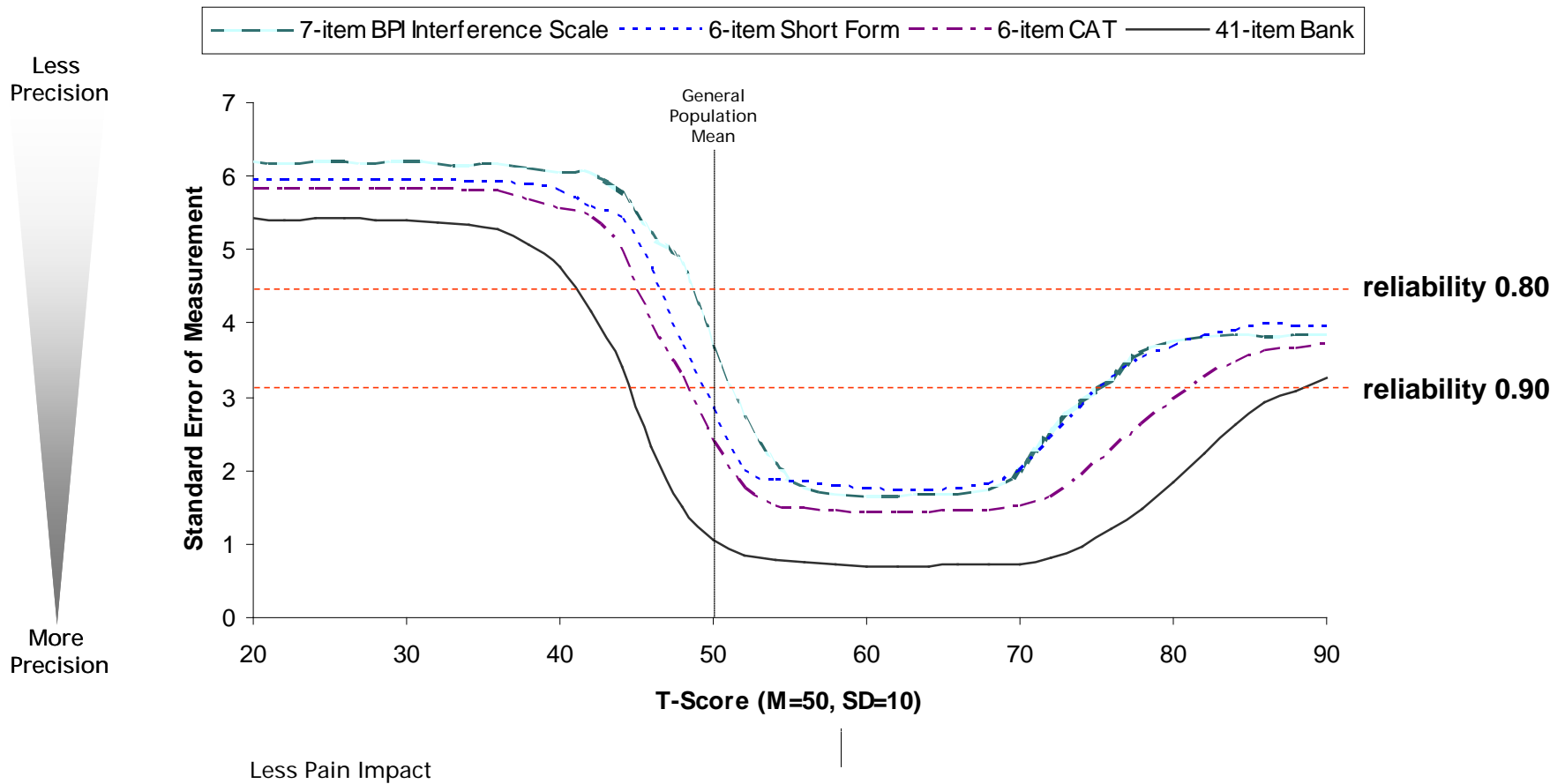


Measurement Precision and Range

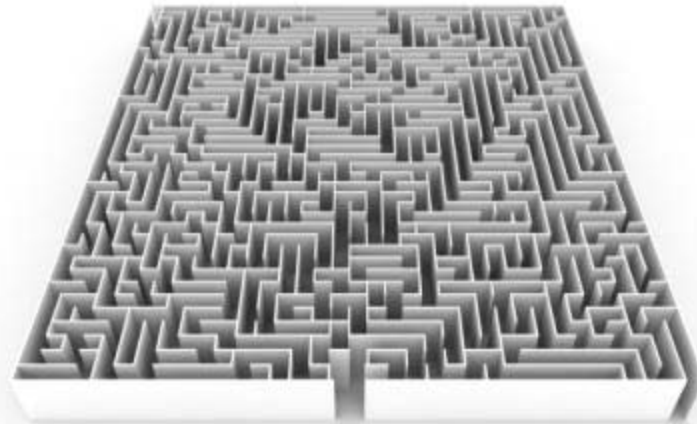



Measurement Precision and Range

Pain Impact



How to Access PROMIS Tools ?






Patient-Reported Outcomes Measurement Information System

Dynamic Tools to Measure Health Outcomes From the Patient Perspective

This Site



HOME

About PROMIS

- NIH Roadmap and PROMIS Goals
- PROMIS Network

PROMIS Domains

- Domain Framework
- Domain Definitions

Item Bank Development

- Literature Review for Items
- Archival Data Analysis
- Item Classification
- Qualitative Item Review
- Preliminary Item Pools

Item Bank Testing

- Wave I Testing
- Wave II Testing

Item Banks and Tools

- Version 1.0 Item Banks

Announcements

Title

PROMIS: The First Four Years

The Patient Reported Outcomes Measurement Information System (PROMIS): A Walk Through the First Four Years
[Click here to view document](#)

Assessment Center Training Workshops


April 20, 2009 (New York, NY)
8:00-5:00 PM. **To register go to: www.regonline.com/63367_694367**
[Click here for lodging and transportation information](#)

July 17, 2009 (Chicago, IL)
8:00-5:00 PM. **To register go to: www.regonline.com/63368_695556**
[Click here for lodging and transportation information](#)

FDA on PROMIS

"FDA joins NIH in the goal of ensuring that PROMIS results in meaningful measurement of the impact of medical product treatment."

Laurie Burke, MPH, RPh
Director, Study Endpoint and Label Development
Center for Drug Evaluation and research
U.S. Food and Drug Administration



Assessment Center Links

- ENTER ASSESSMENT CENTER
- Assessment Center User Manual
- Assessment Center Workshop - April 20th in NYC
- Lodging and transportation for April 20th workshop
- Assessment Center Workshop, July 17th in Chicago
- Lodging and transportation for July 17th workshop
- Introduction to PROMIS, Item Response Theory, Computerized Adaptive Testing, and Assessment Center Slide Presentation (Richard Gershon)

Assessment Center

 **PROMIS Assessment Center**SM

Studies Instruments **Set-up** Administration

My Account Help Logout

[Basic](#) | [Advanced](#) | [Consent](#) | [Registration](#) | [Launch](#) |

Basic Set-up of Study (Curious George V1)

Basic Set-up of Study

You may define participant registration forms below.

Start Date*


End Date*


Close data collection when sample size equals*

Contact information for participant questions*

URL Extension*

Login screen welcome message (maximum 10,000 characters)





Welcome to Curious George V1

Assessment Center

The screenshot shows the Assessment Center web application interface. The main header features the PROMIS logo and the text 'Assessment Center' with a trademark symbol. Navigation tabs for 'Studies', 'Instruments', 'Set-up', and 'Administration' are visible, along with links for 'My Account', 'Help', and 'Logout'. The main content area is titled 'Study Content (Curious George V1)' and includes instructions for adding and ordering instruments. A table lists the selected instrument: 'PROMIS Bank-Depression 1.0'. A 'Preview Instrument Options' dialog box is overlaid on the right, titled 'Assessment Center - Preview Instrument Options -- Webpage Dialog'. The dialog contains the following options:

- Preview items in order
- Simulate instrument administration
- Simulate CAT administration
- Show Execution Detail (e.g. Item ID and Ability Estimate)

Buttons for 'Continue' and 'Cancel' are located at the bottom right of the dialog.

Assessment Center



Assessment CenterSM

[EDDEP04]

In the past 7 days

I felt worthless

- Never
- Rarely
- Sometimes
- Often
- Always

[EDDEP05]

In the past 7 days

I felt that I had nothing to look forward to

- Never
- Rarely
- Sometimes
- Often
- Always

Assessment Center

[Custom Instruments \(D. Cella's first project\)](#) >> [Instrument Detail \(PROMIS ED Short Form\)](#) >> Item Statistics

Item Statistics

ID: SDS_OUTL

Domain: Domain B

[Back](#)

In the last week, OUTLOOK

Responses

Sample Population <input type="text" value="Breast Stage III"/>											Update				
N-Size	Mean Score	SD Score	Minimum Score	Maximum Score	Alpha Reliability	Adj. Alpha Reliability	Item Total Correlation	Adj. Item Total Correlation	Percent Omit	% Option 1	% Option 2	% Option 3	% Option 4	% Option 5	
750	2.50	1.00	1	5	0.97	0.96	0.57	0.54	1.2	35.0	20.9	17.2	15.9	10.4	

Differential Item Functioning (DIF) Information					Update
DIF Groups (Reference/Focal)	Uniform	Uniform Prob.	Non-Uniform	Non-Uniform Prob.	
<input type="text" value="Breast Stage III - Breast Stage IV"/>	50.01	0.0010	20.75	0.0010	

Description: Adanced Breast Ca.

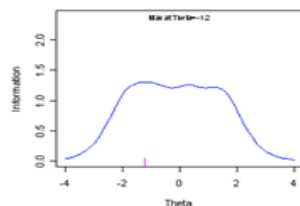
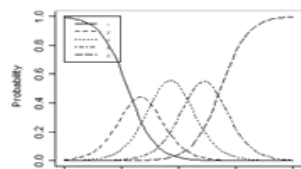
Sample Population

[Update](#)

IRT Model

Category Response Function

Item Information Function



Model Fit & Scalability Indices		
	Value	Prob.
S-X ²	6.30	0.7894
S-G ²	6.33	0.7868
X ²	1.06	0.7473
G ²	1.18	0.7544
Loevinger H Scalability	0.52	
Location At Max Information	0	

IRT Parameters					
Slope (a)	Guessing (c)	Threshold (CB) 1	Threshold (CB) 2	Threshold (CB) 3	Threshold (CB) 4
2.05000	0	0.7800	0.9500	0.0200	1.0400

Conclusion



Conclusions

- 1 The PROMIS initiative may help to harmonize and standardize PRO measures
- 2 CAT instruments promise to provide more precise measurement without increased respondent burden
- 3 IRT based instruments can estimate the precision for each assessment and tailor effectiveness cut offs to each patient



U.S. Department of Health
and Human Services



This work was supported in part by the National Institutes of Health through the NIH Roadmap for Medical Research, Grant U01 AR052158-01



**National
Institutes
of Health**



NIAMS

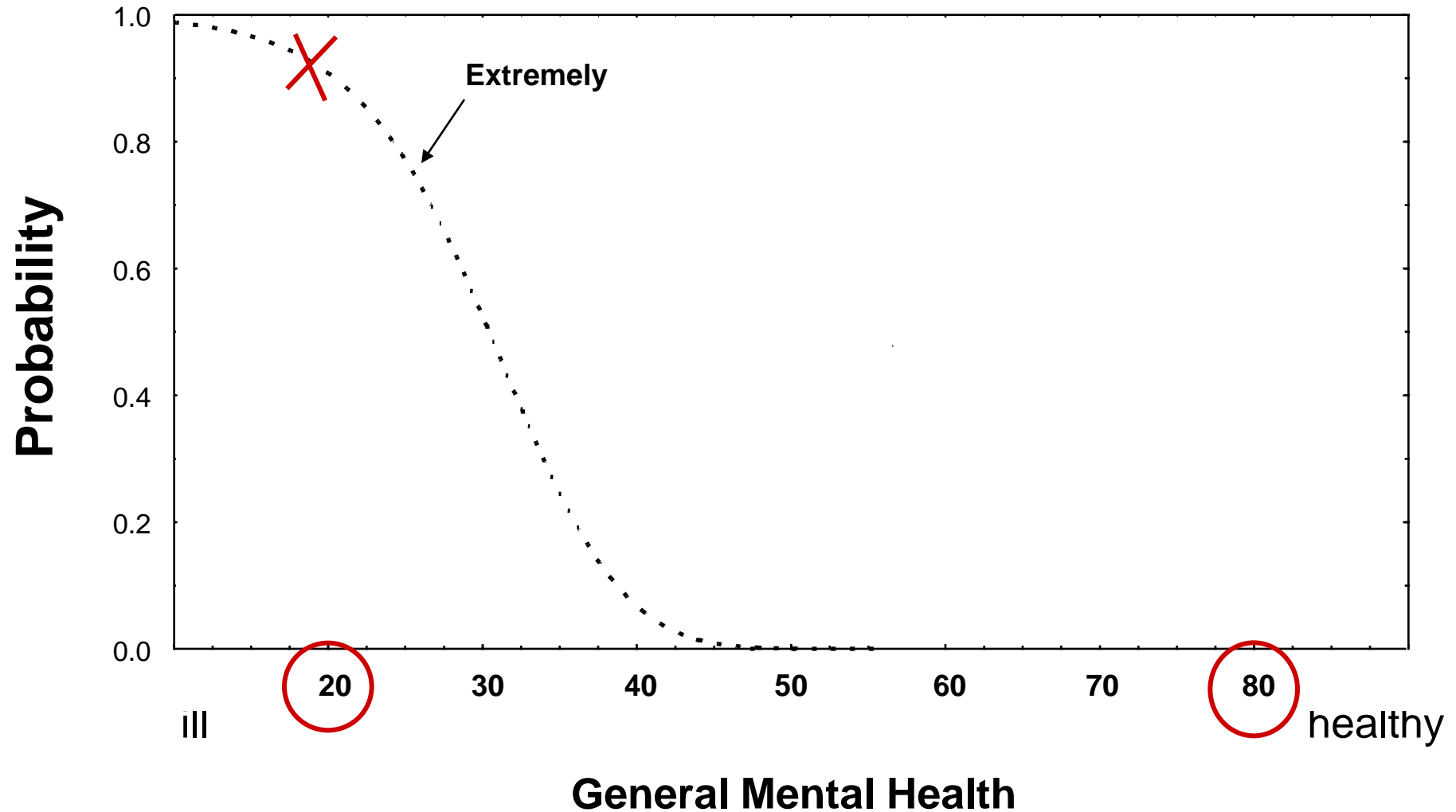
National Institute of Arthritis and
Musculoskeletal and Skin Diseases

Parking Lot

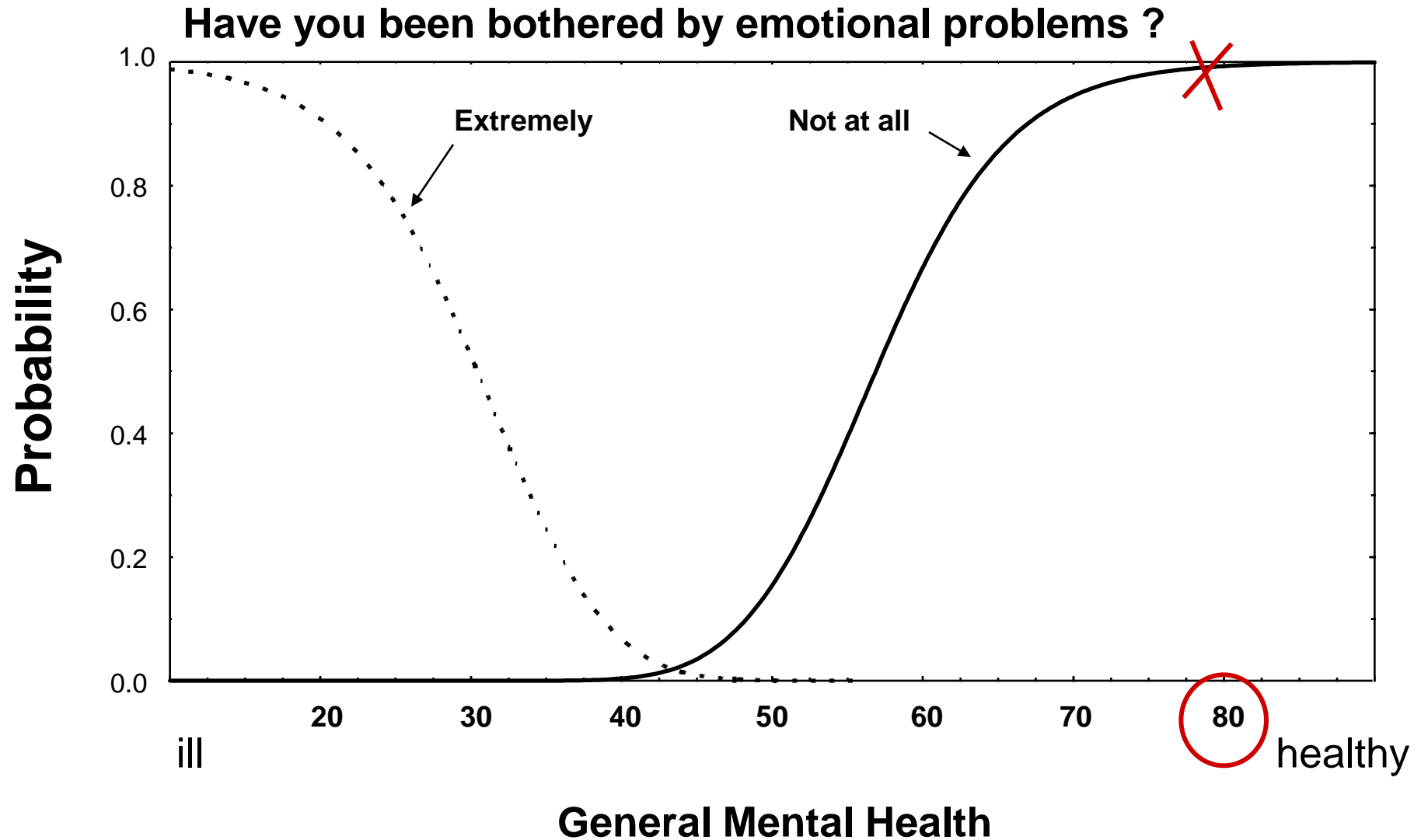
Item Response Theory

Item Response Theory

Have you been bothered by emotional problems ?

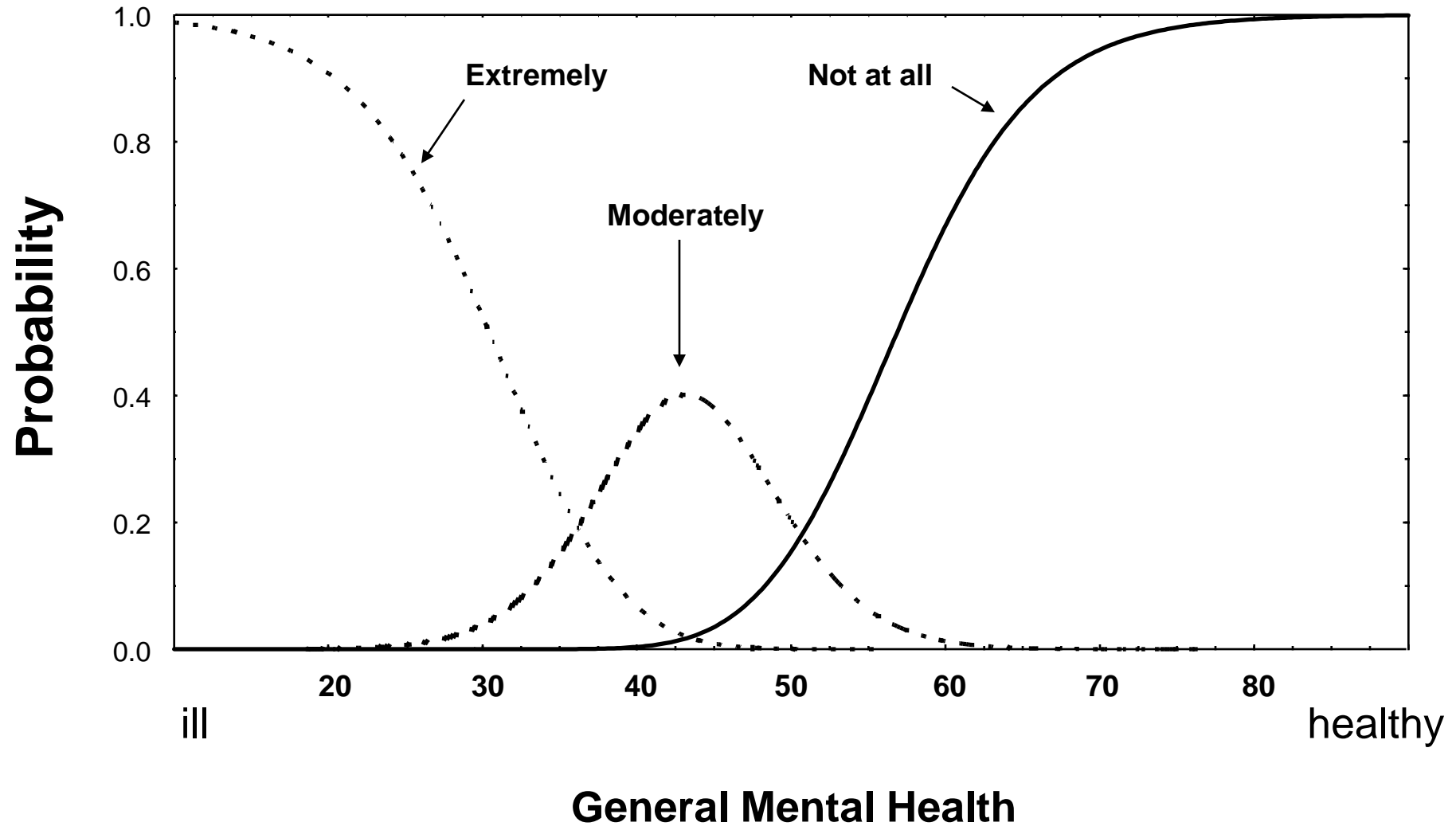


Item Response Theory



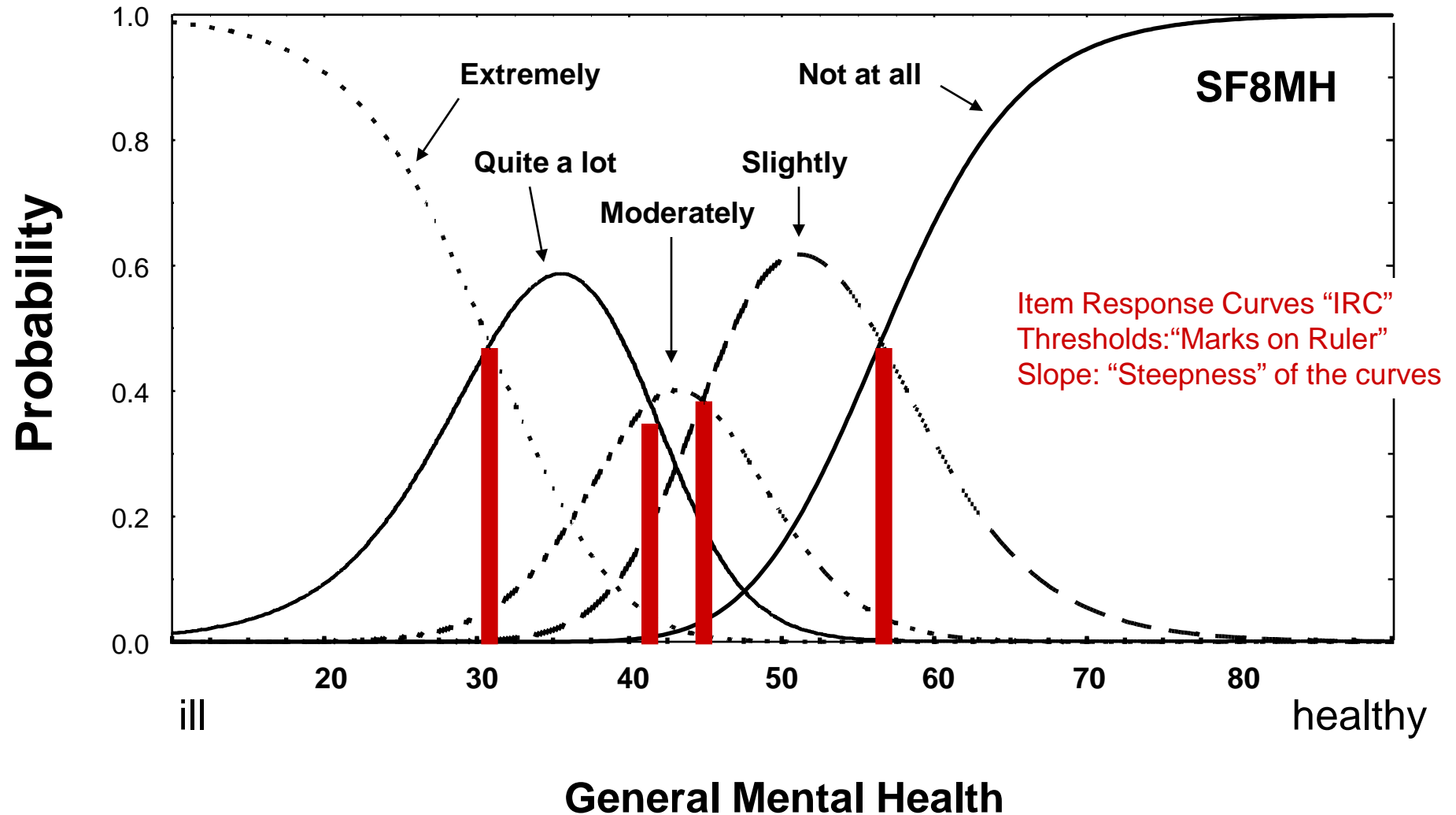
Item Response Theory

Have you been bothered by emotional problems ?

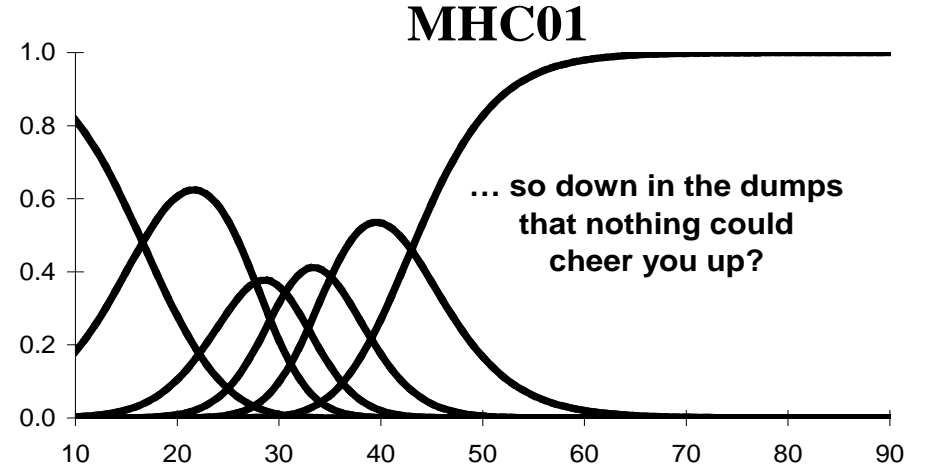
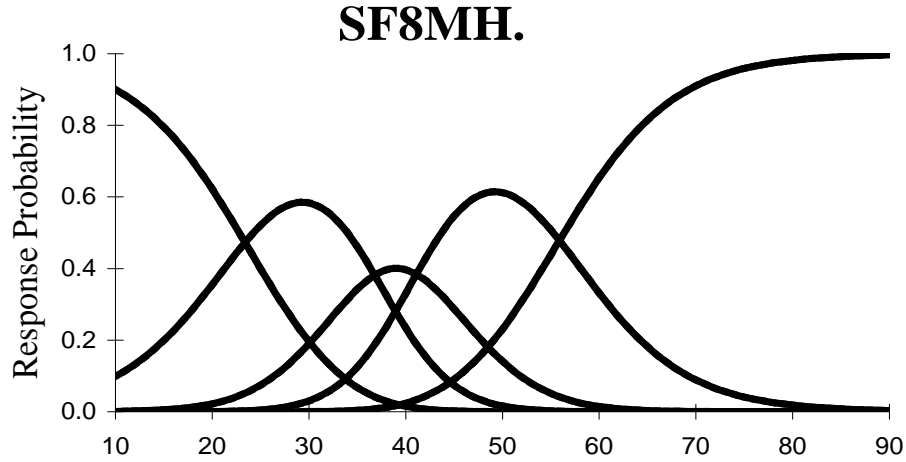


Item Response Theory

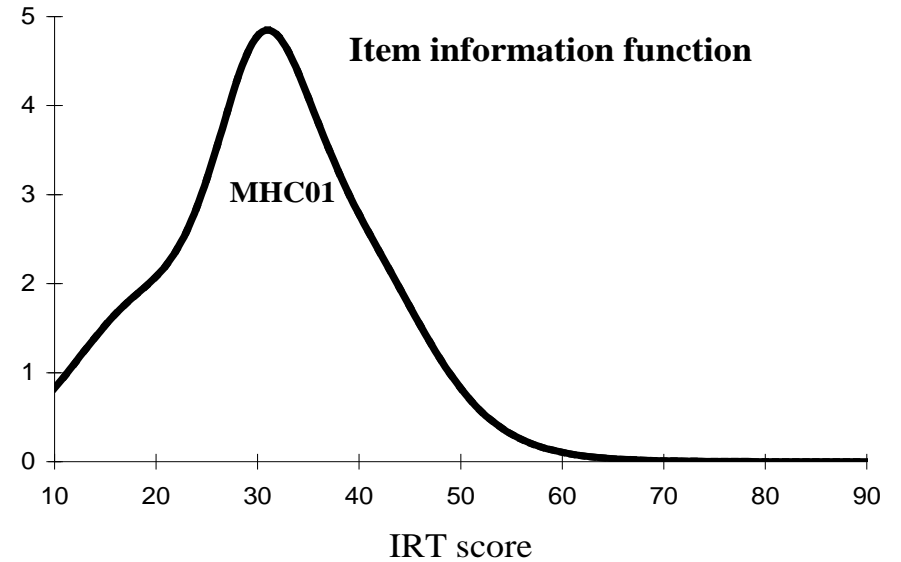
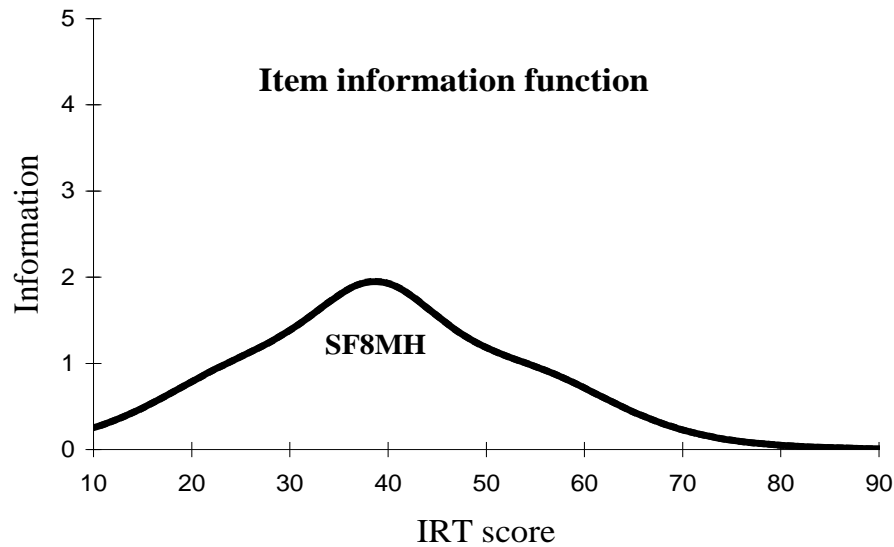
Have you been bothered by emotional problems ?



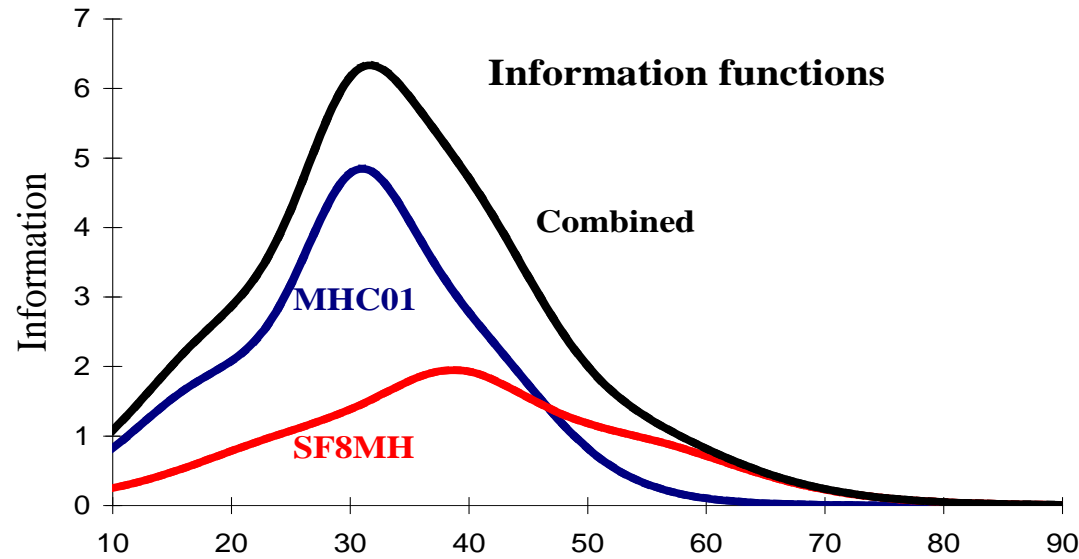
Item Information



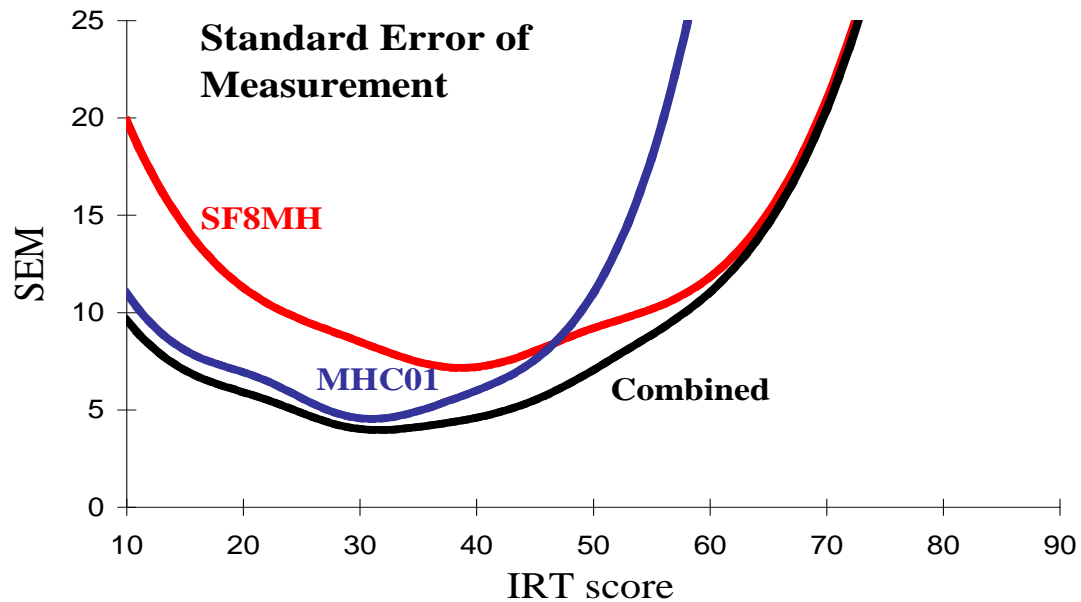
$$I_i(\theta) = a_i^2 \left[\sum_{k=0}^{m_i} k^2 P_{ik}(\theta) - \left(\sum_{k=0}^{m_i} k P_{ik}(\theta) \right)^2 \right] = a_i^2 \text{var}(X_i)$$



Item and Test Information



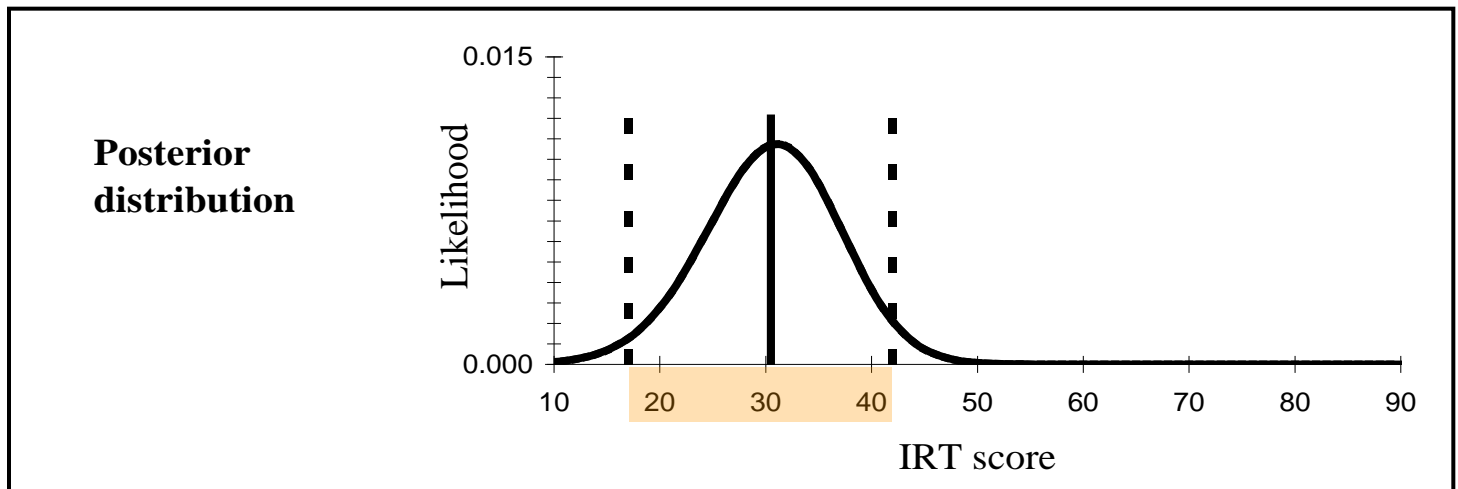
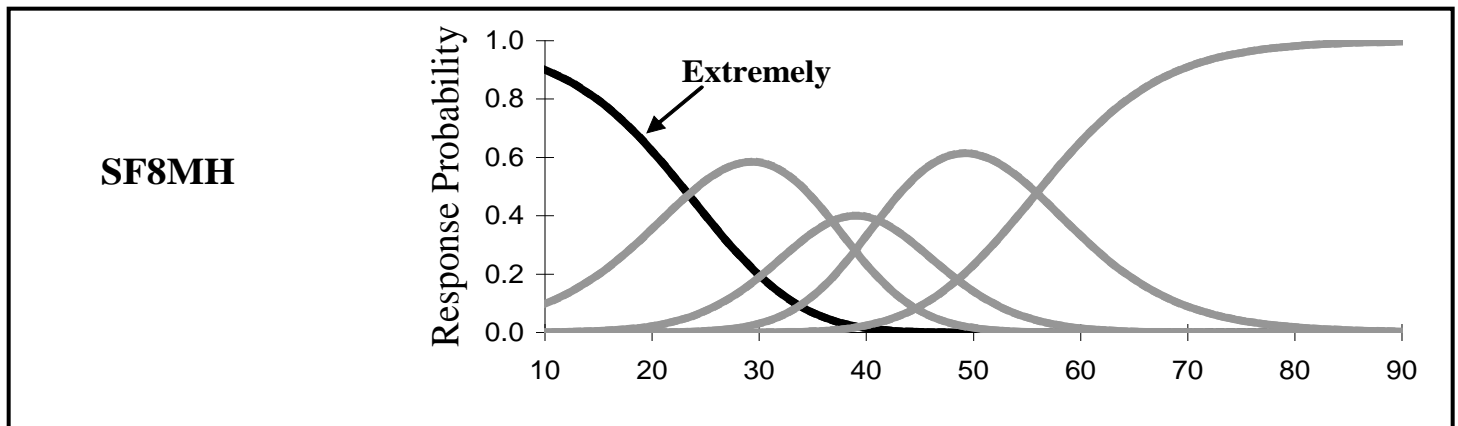
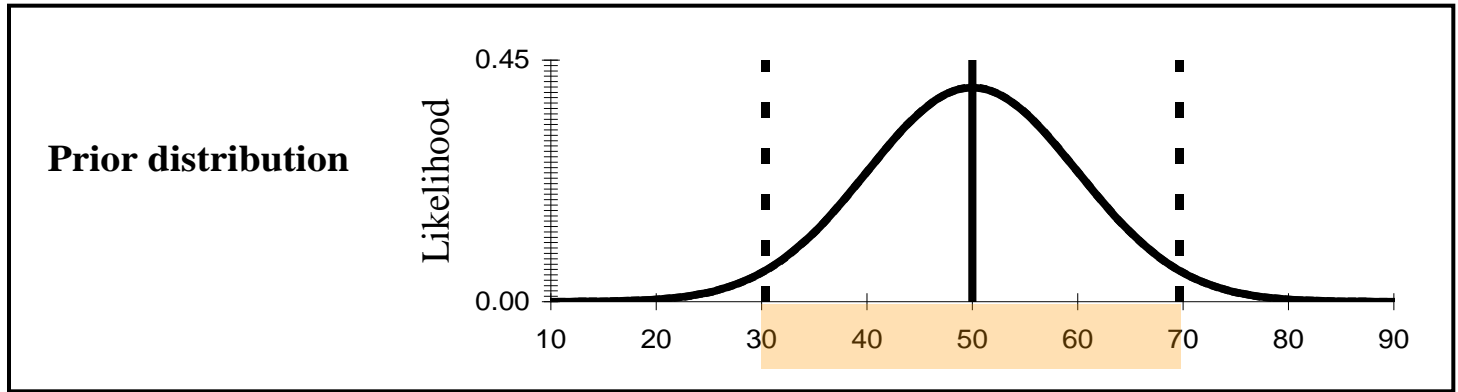
$$I_t = \sum I_i$$



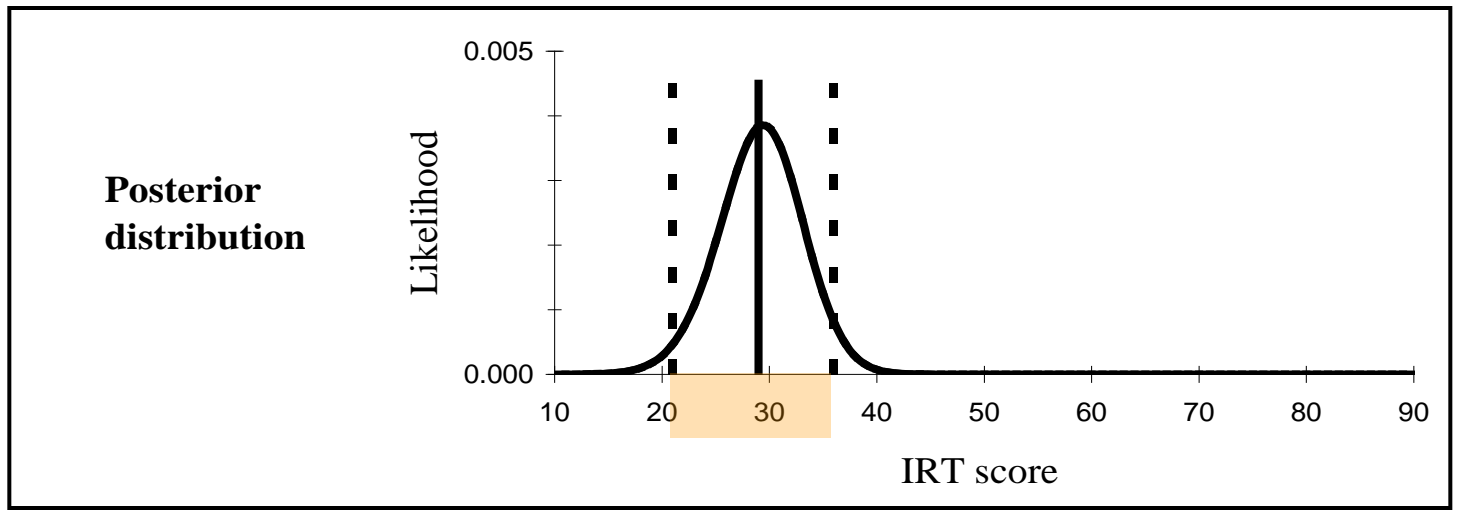
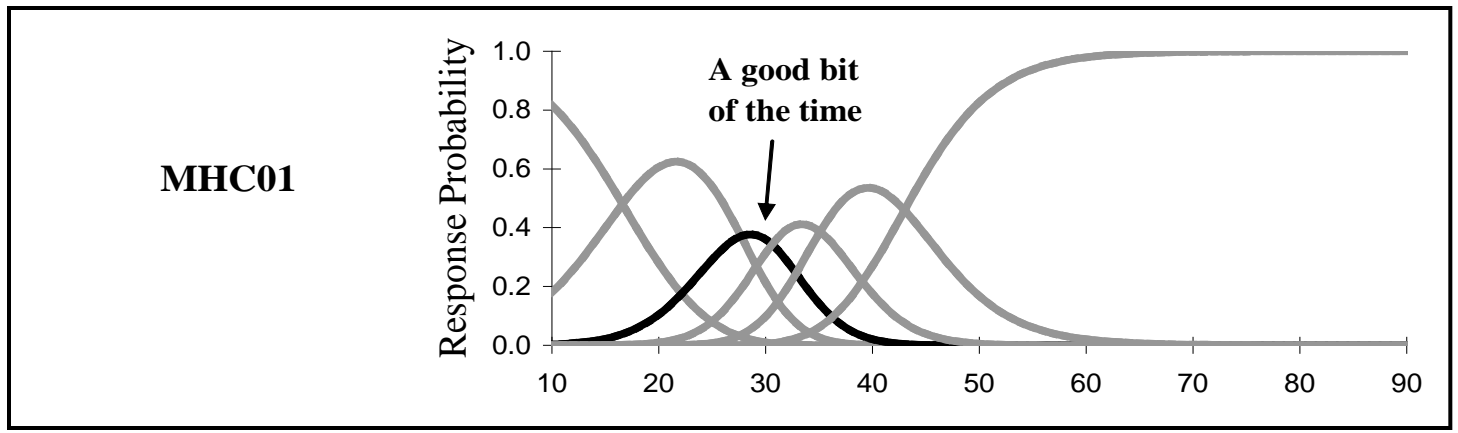
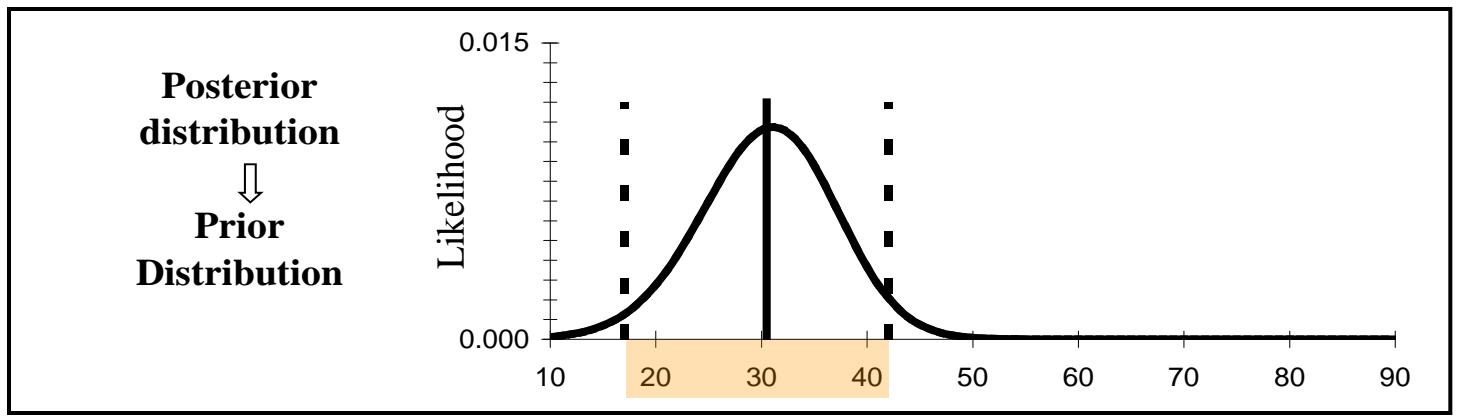
$$SEM = \frac{1}{\sqrt{I}}$$

How to Score a Patient ?

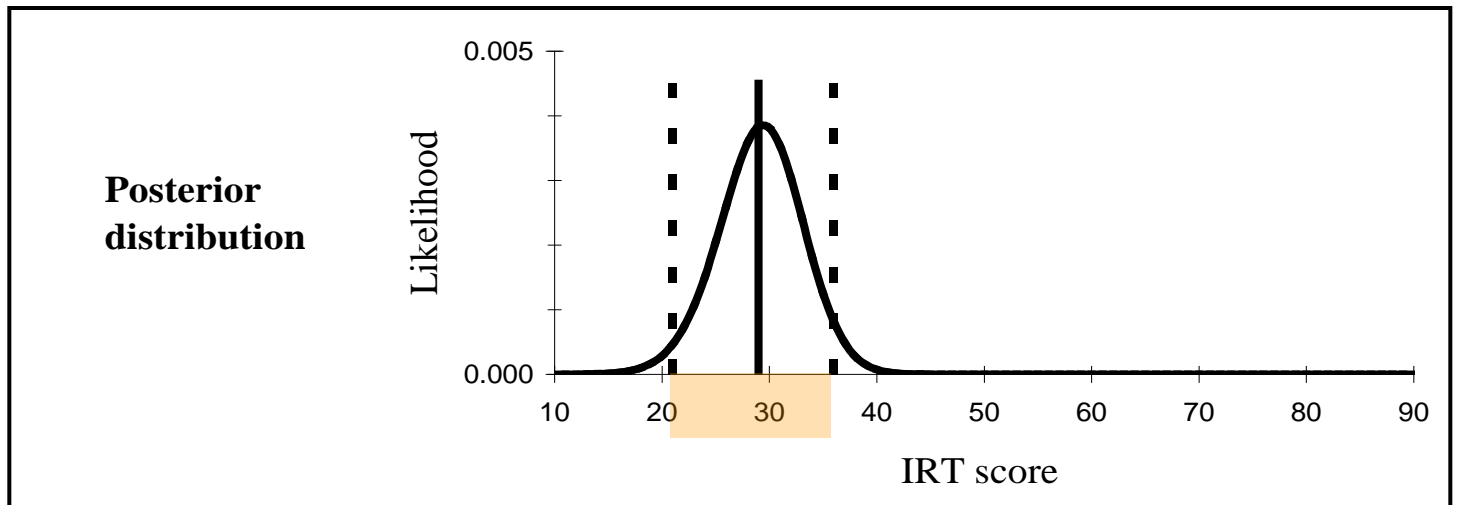
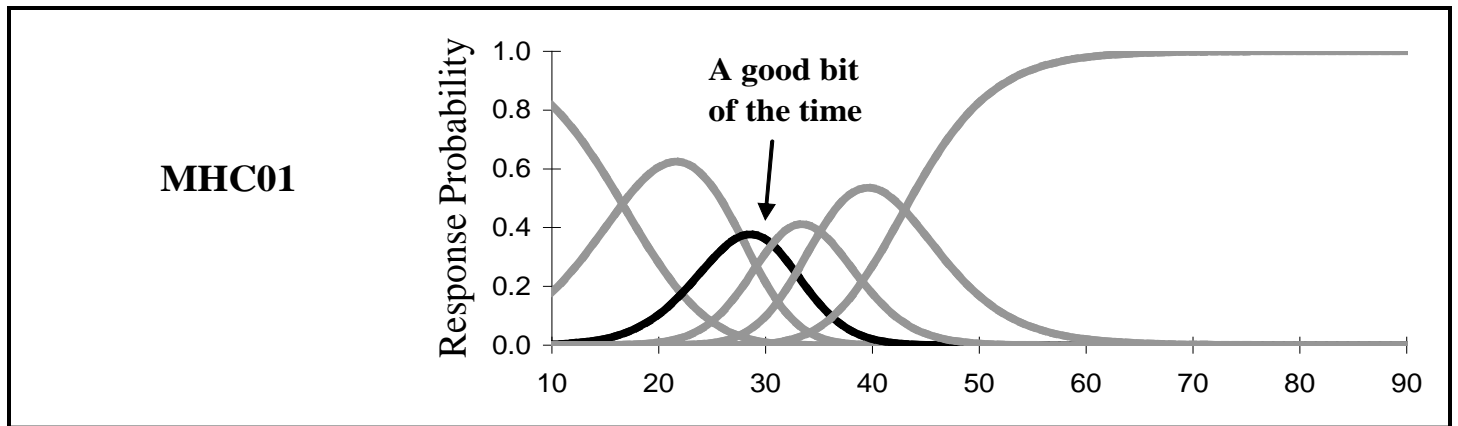
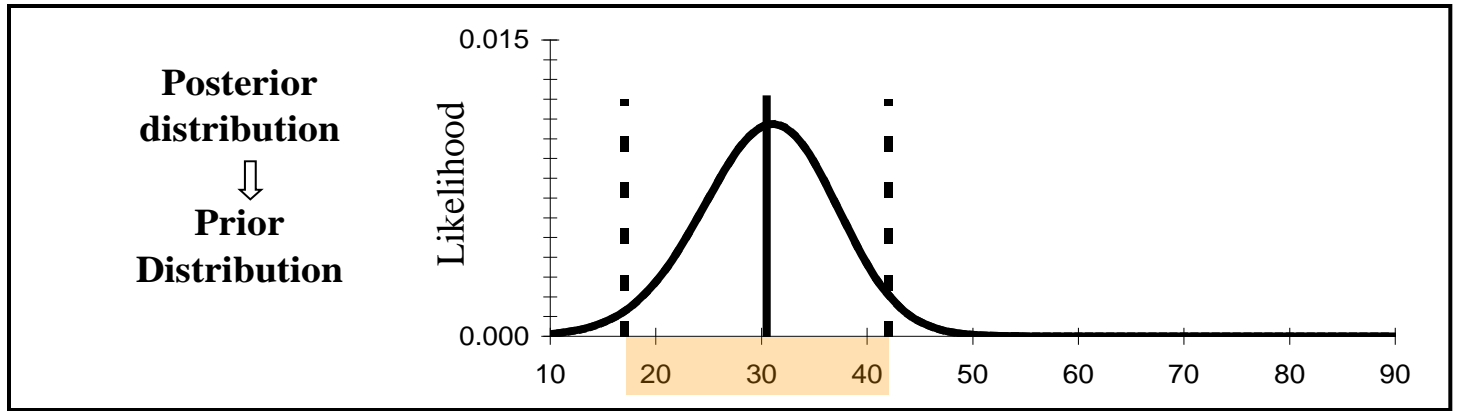
IRT
Scoring
in a
CAT
(EAP
Approach)



IRT
Scoring
in a
CAT
(EAP
Approach)



IRT
Scoring
in a
CAT
(EAP
Approach)



Assessment Center

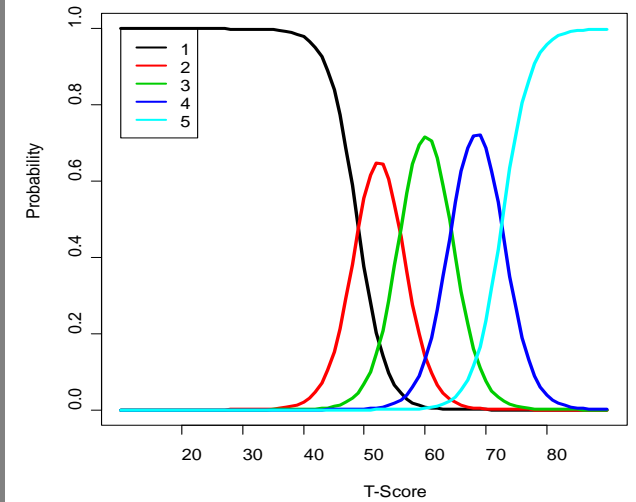
Beginning of CAT

T-Score = **50**

SE = **10**

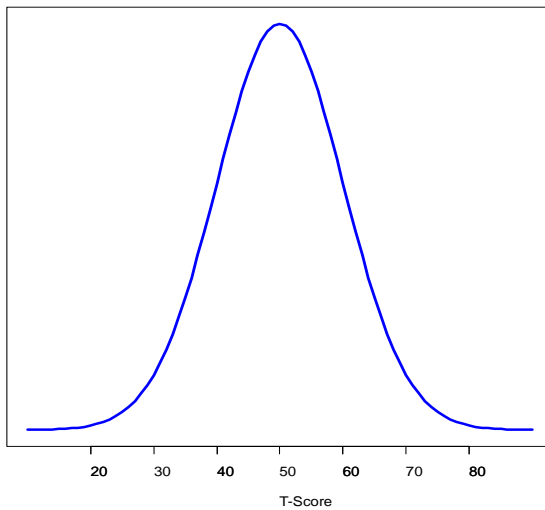
Best Item-I felt depressed

item characteristic curves

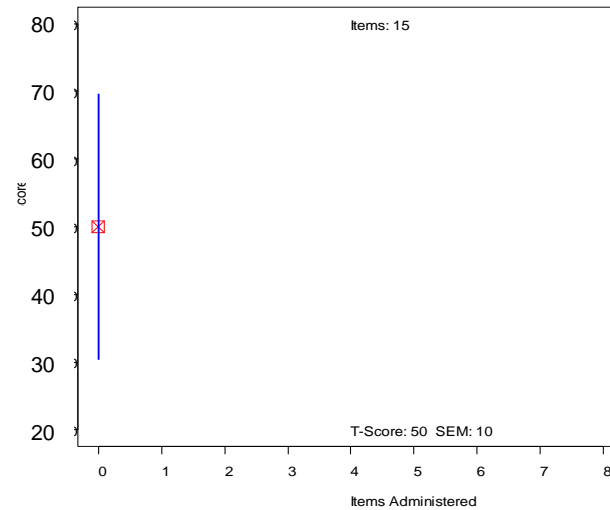


a priori distribution

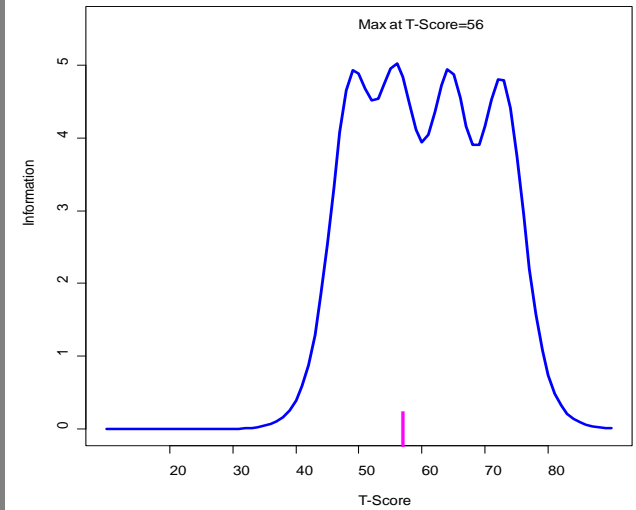
Posterior Distribution



score \pm 95% confidence interval



item information function



I felt depressed

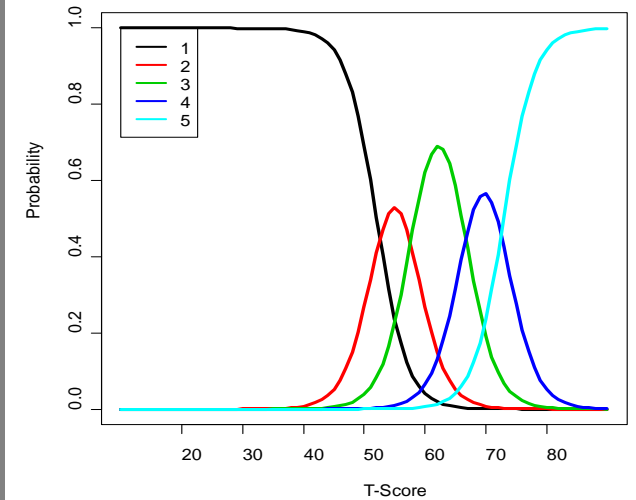
1. Never
2. Rarely
3. Sometimes
4. Often
5. Always

T-Score = **52**

SE = **4**

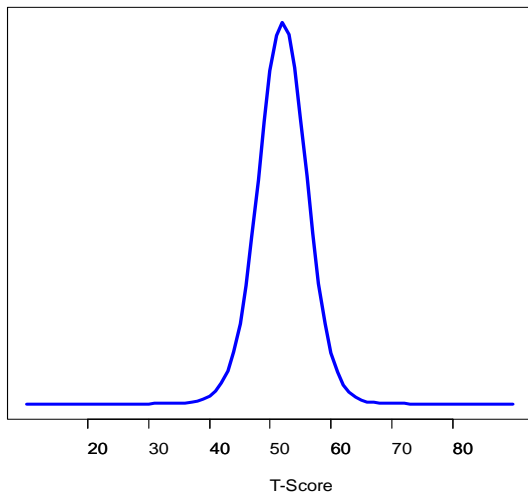
Next Best Item-I felt like a failure

item characteristic curves

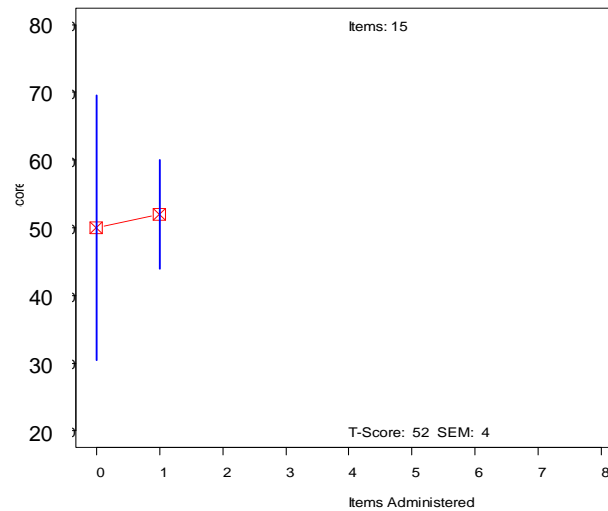


posterior distribution

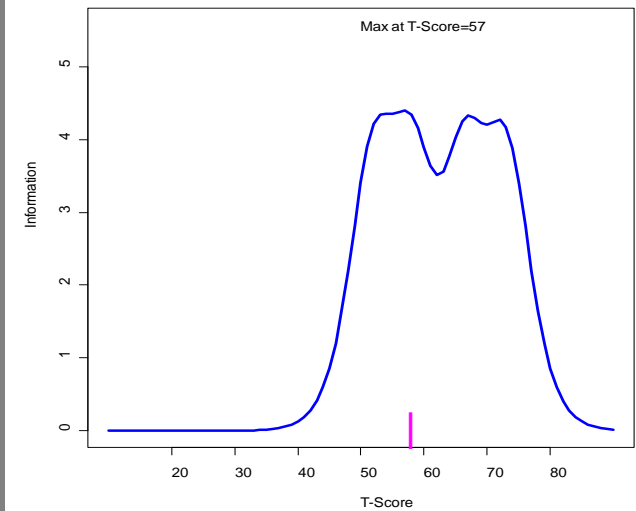
Posterior Distribution



score \pm 95% confidence interval



item information function



I felt like a failure

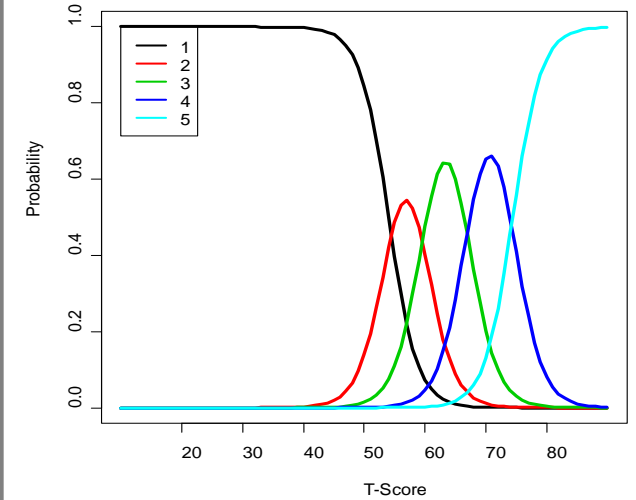
1. Never
2. Rarely
3. Sometimes
4. Often
5. Always

T-Score = **53**

SE = **3**

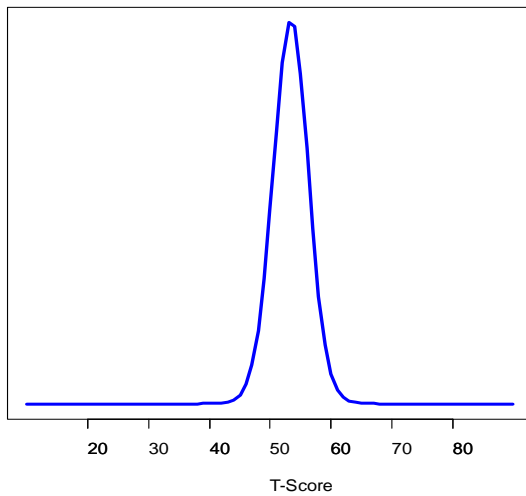
Next Best Item-I felt worthless

item characteristic curves

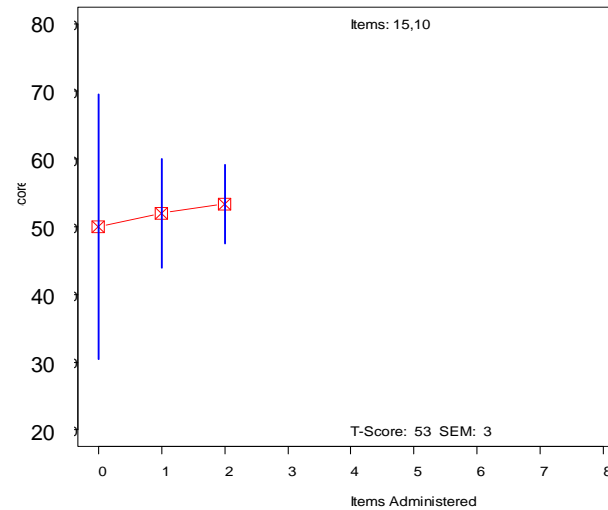


posterior distribution

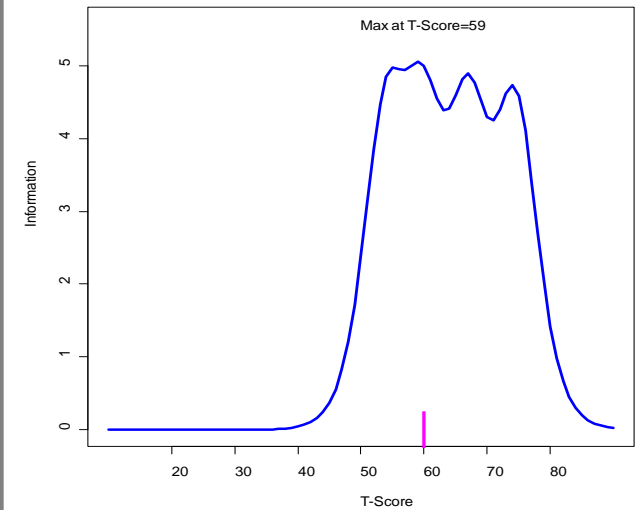
Posterior Distribution



score ± 95% confidence interval



item information function



I felt worthless

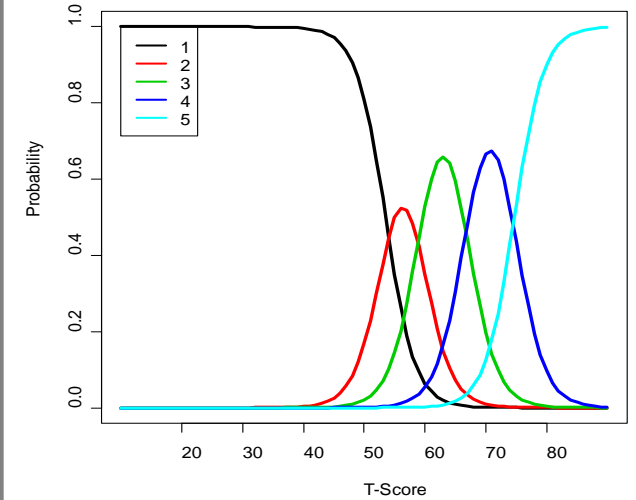
1. Never
2. Rarely
3. Sometimes
4. Often
5. Always

T-Score = **55**

SE = **2**

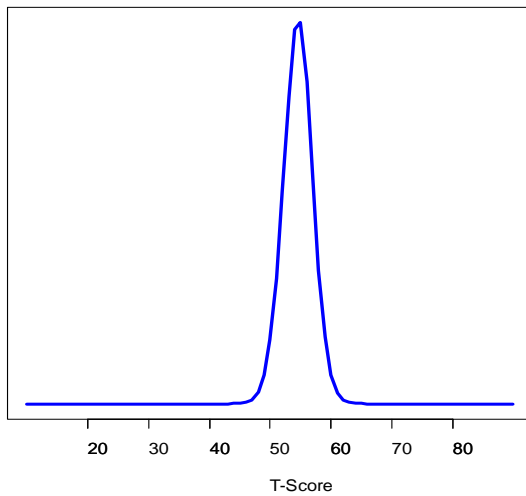
Next Best Item-I felt helpless

item characteristic curves

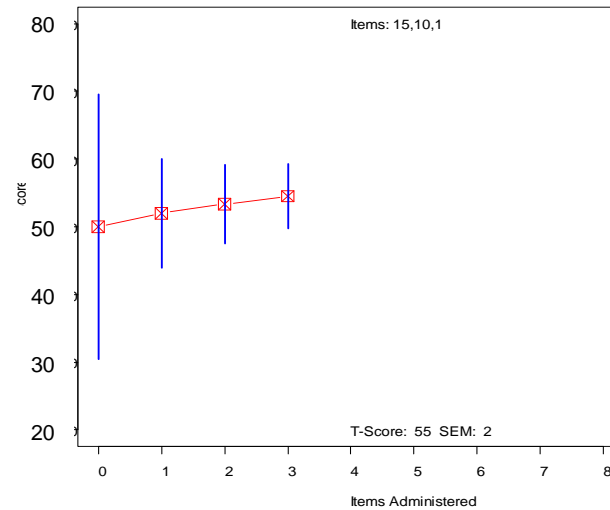


posterior distribution

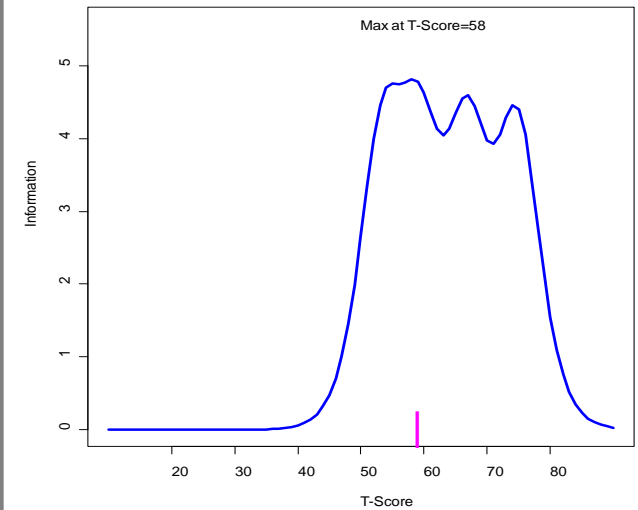
Posterior Distribution



score \pm 95% confidence interval



item information function

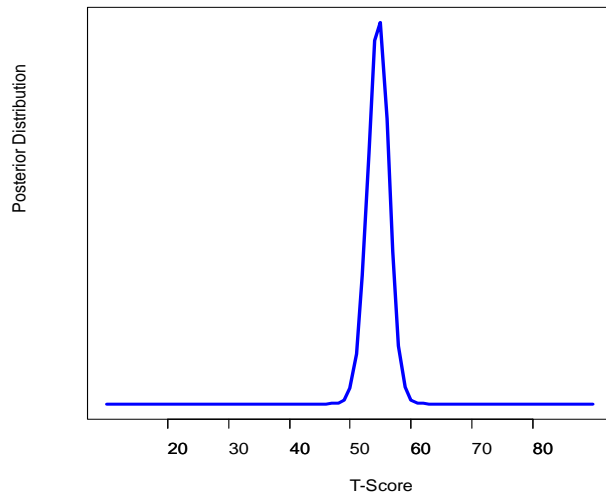


I felt that nothing could cheer me up

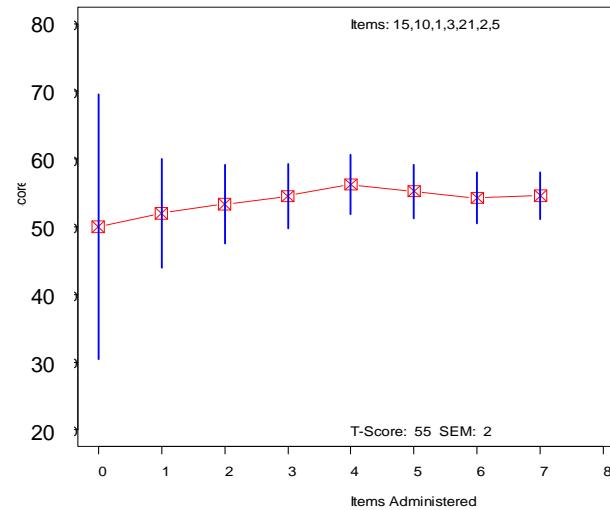
1. Never
2. Rarely
3. Sometimes
4. Often
5. Always

T-Score = **55** SE = **2**

posterior distribution

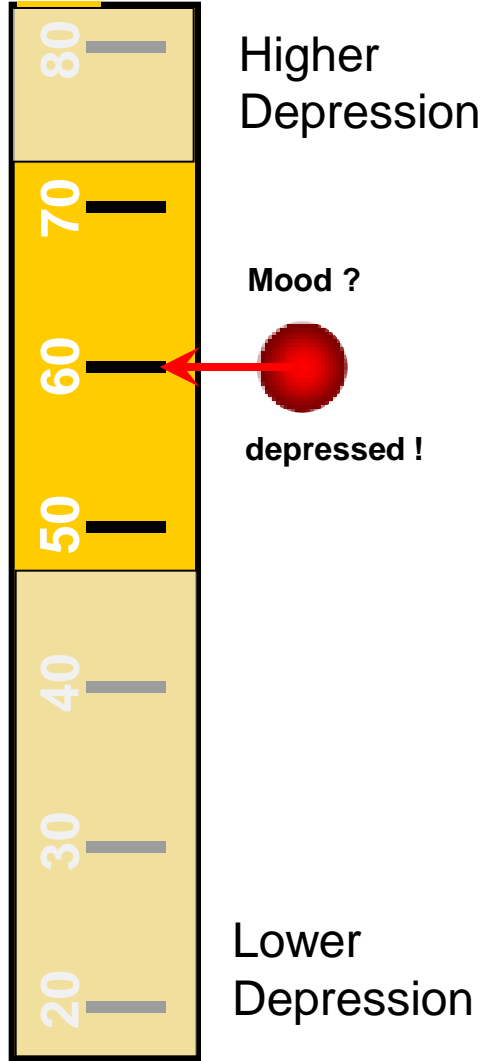


score \pm 95% confidence interval



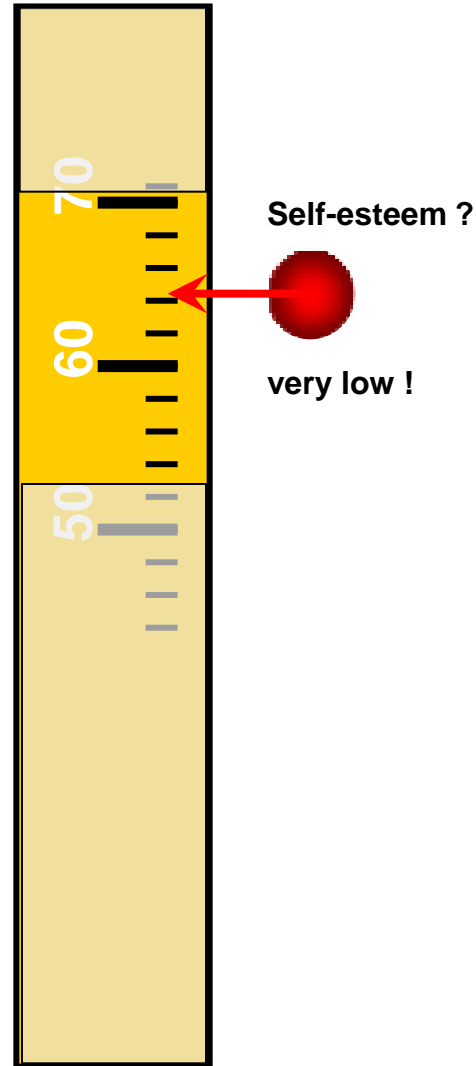
Computer Adaptive Tests

1. Question



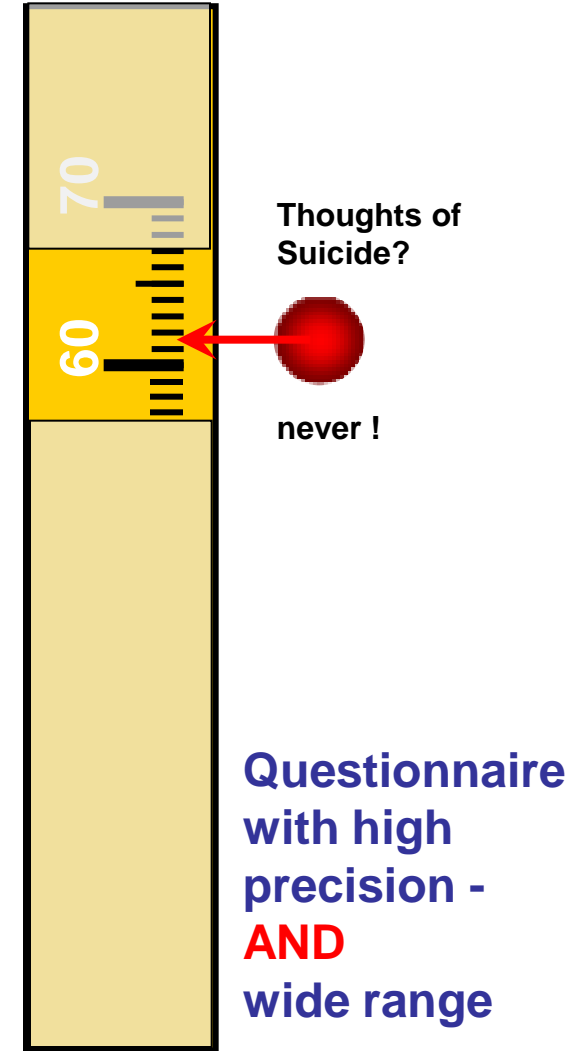
SE 1,3

2. Question



SE -30%

3. Question

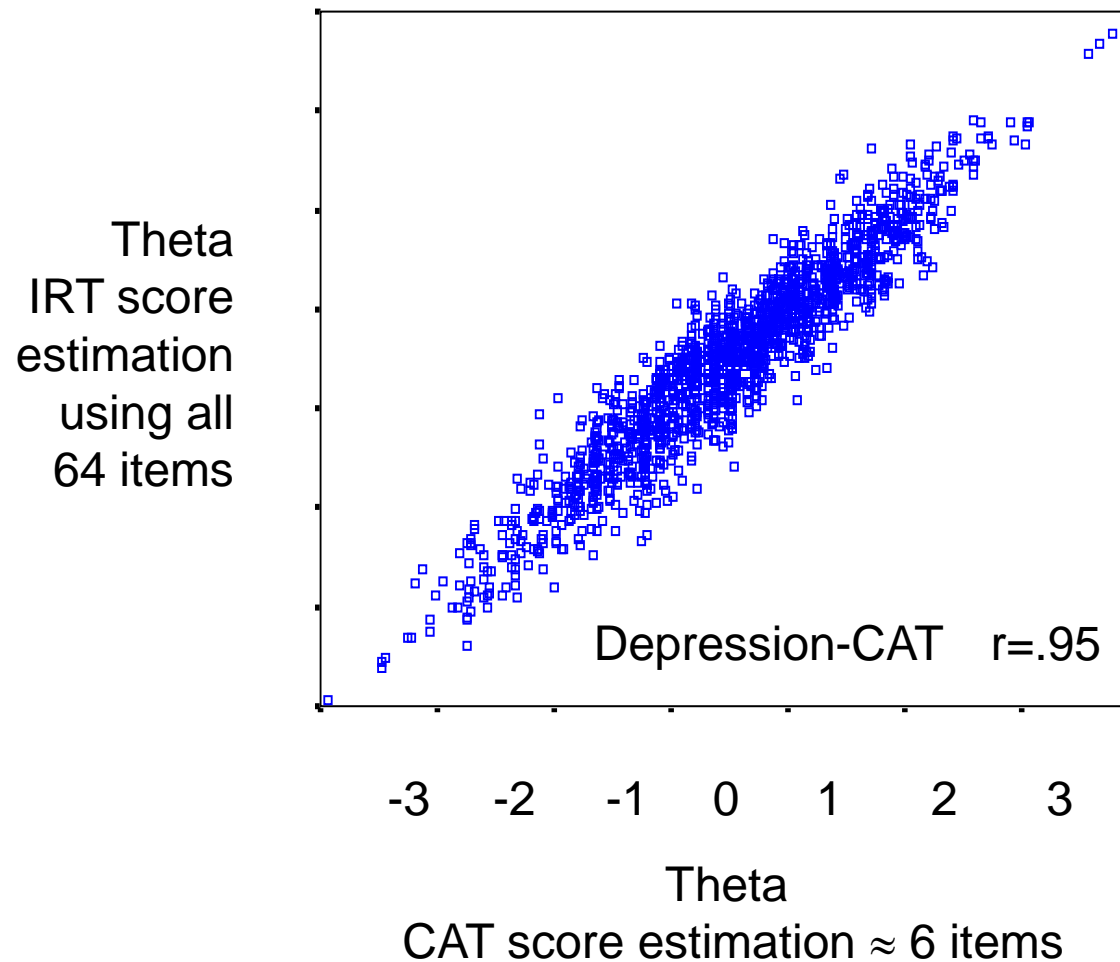


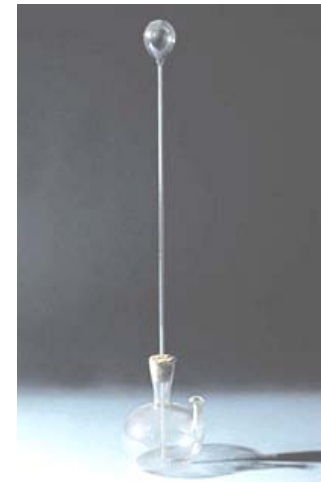
SE -60%

Dynamic vs Static Assessment

n=3270

Stopping rule $SE < .32$ (reliability $> .90$)





**Thermoscope
Galileo 1597**