

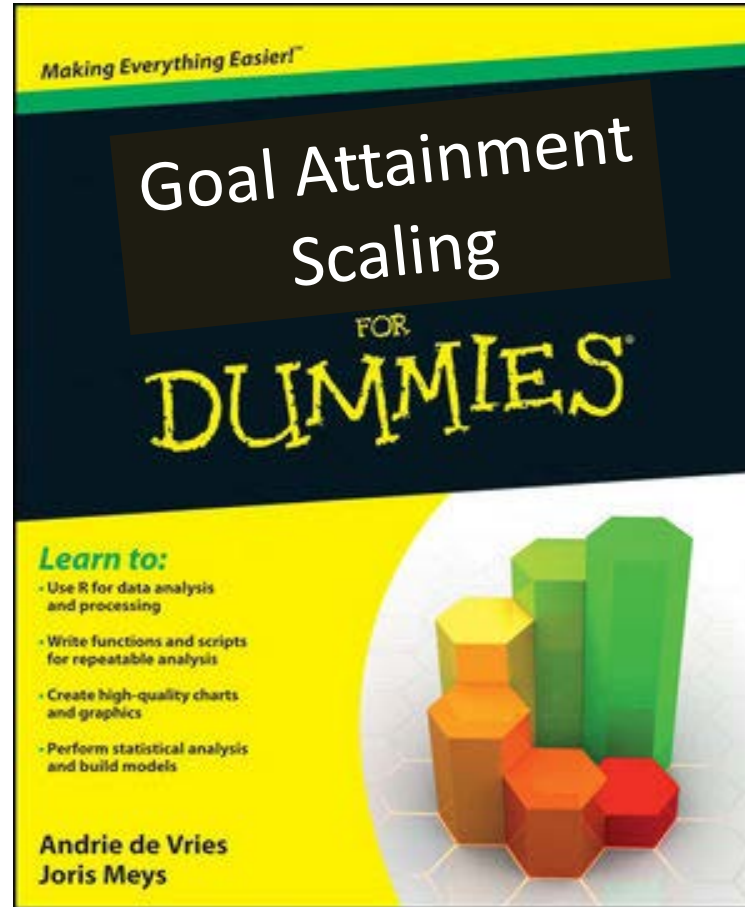
Goal Attainment Scaling



A systematic review of GAS
as an outcome measure in
drug trials

*ISCB Conference Utrecht
Charlotte Gaasterland
August 2015*

GAS...?



Imagine 3 boys with Duchenne disease:



Adam

'I want to be able to walk'



Brad

'I want to be able to eat
independently'



Chris

'I want to breathe
independently'

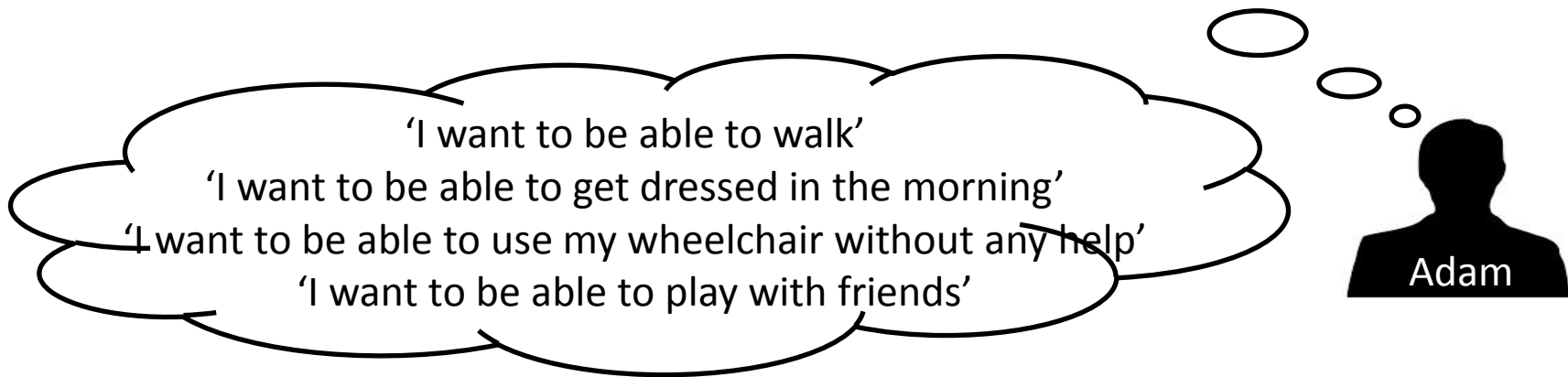
Six minute walk test

- 2 Adam is unable to walk
- 1 Adam can take 3 steps
- 0 Adam is able to walk for 5 minutes
- 1 Adam can walk for 15 minutes
- 2 Adam can walk longer distances




- 2 Chris is unable to breathe independently
- 1 Chris can breathe for 10 minutes
- 0 Chris can breathe for one hour
- 1 Chris can breathe for two hours
- 2 Chris can breathe for at least three hours





$$T = 50 + \frac{10 \sum w_i x_i}{\sqrt{(1-\rho) \sum w_i^2 + \rho (\sum w_i)^2}}$$


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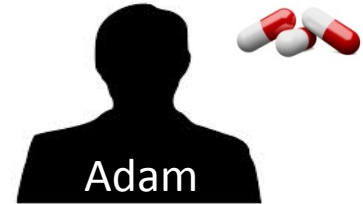
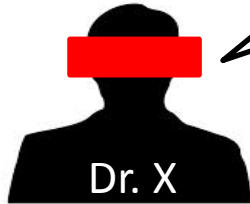
T = *GAS score*

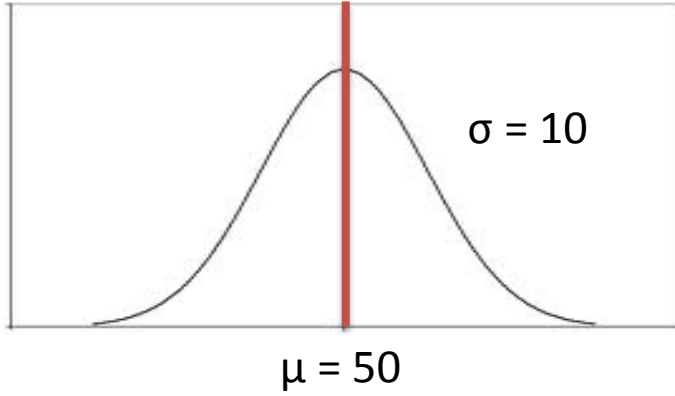
x_i = *Original score*

w_i = *Weight given to the original score*

ρ = *Intercorrelation among goal scores (estimated at 0.3)*

1. What are your goals, defined in 5 levels of attainment?
2. Which goals are most important to you?
3. *Intervention*
4. Have you attained your goals?







**Advances in Small Trials dEsign for Regulatory
Innovation and eXcellence**

Sounds like a
good idea!



Systematic Review

Is GAS useful in drug trials?



Is GAS used in drug trials?

Has GAS been validated in drug trials?

Has GAS been validated in other studies?

Methods



- ❑ Search in Medline, Embase, PsychInfo (with help of a librarian)
- ❑ Search terms: Trial, RCT, variations of Goal Attainment Scaling
- ❑ Selection by 2 reviewers independently
- ❑ Data extraction and critical appraisal by 2 reviewers independently

Results



Primary search:
5459 titles &
abstracts

3818 titles &
abstracts assessed
for eligibility

307 full text articles
assessed for
eligibility

58 articles included

1641 duplicates
removed

3511 articles
excluded based on
title & abstract

249 articles
excluded based on
full text

What did all those papers tell us?

- ❏ Validation is mainly done in geriatrics/rehabilitation
- ❏ Usually in non-drug trials





Drug trials

- ❏ Botulinum Toxin (Botox) used in patients with Cerebral Palsy
- ❏ Donepezil hydrochloride in Alzheimer Disease patients



Data extraction

Face validity

Content validity

Construct validity

Intra-rater reliability

Inter-rater reliability

Responsiveness



Data extraction

Face validity

Content validity

Construct validity

~~Intra-rater reliability~~

Inter-rater reliability

Responsiveness





Face validity }
Content validity }



Construct validity



Inter-rater reliability



Responsiveness



Face validity



In drug studies: 1

In GAS validity studies: 0

- ❏ *Therapists and intakers evaluated the relevance of the goal area for a patient on a 5-point scale.*
- ❏ *'Goal areas were suitably chosen'*

Content validity



In drug studies: 0

In validity studies: 3

- ❏ *Different methods were used*
- ❏ *'Goals were grouped in major categories, and reviewed by clinicians'*

Construct validity



In drug studies: 6

In validity studies: 11

- *Mainly correlations with other relevant measurement instruments*
- *Correlations between change scores: is that construct validity?*

Inter-rater reliability



In drug studies: 2

In validity studies: 10

- 🔴 Overall: Good inter-rater reliability ($ICC > 0.9$, $\kappa > 0.5$)
- 🔴 Only in one study the ICC calculation was reported

Responsiveness



In drug studies: 3

In validity studies: 11

 *Responsiveness is usually good to very good: $ES > 1$*

To summarize...



GAS is an inventive measurement instrument for heterogenous patient groups



Validation of GAS in drug trials needs more research..



..but how?

Questions to consider...

Should we validate GAS in drug trials?

How should GAS be validated in drug trials?

When is GAS 'valid'?





Thank you for your
attention!