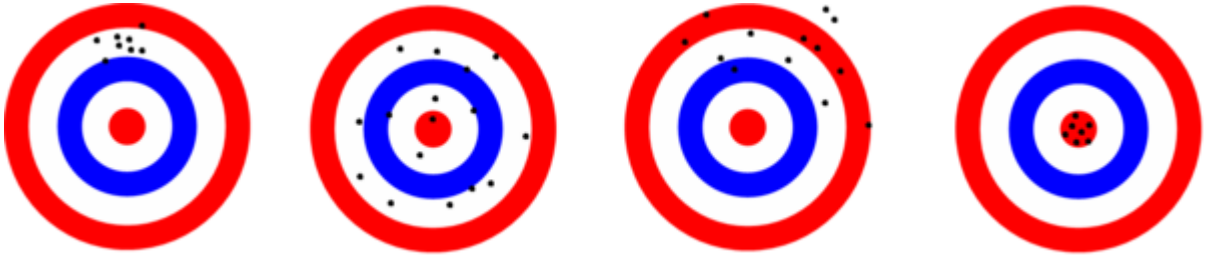


Goal Attainment Scaling: a potential way forward

SCT conference, Liverpool

Charlotte Gaasterland

May 2017

Imagine 3 boys with Duchenne disease:

‘I want to be able to walk’

‘I want to be able to eat independently’

‘I want to breathe independently’

Six minute walk test
Goal Attainment Scaling

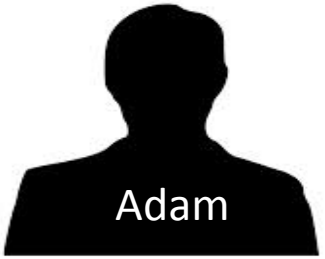
-2 Adam is unable to walk

-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



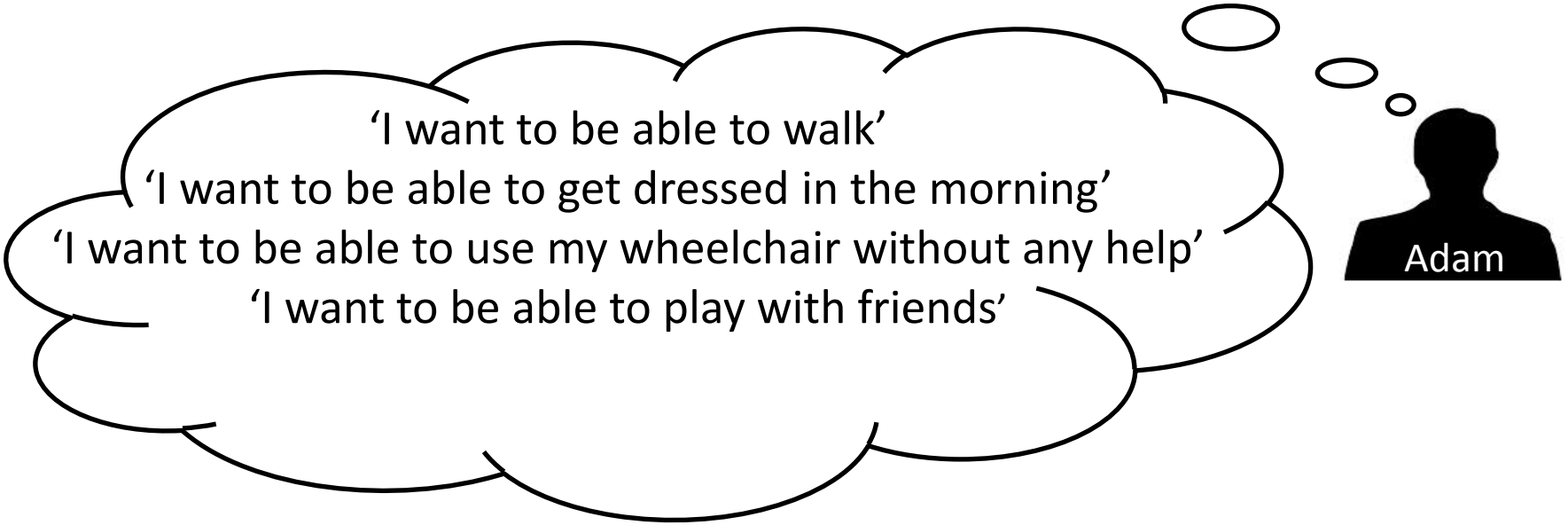
-2 Chris is unable to breathe independently

-1 Chris can breathe for 10 minutes

0 Chris can breathe for one hour

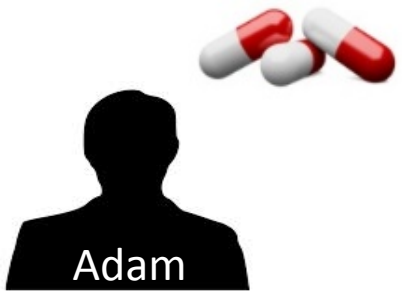
1 Chris can breathe for two hours

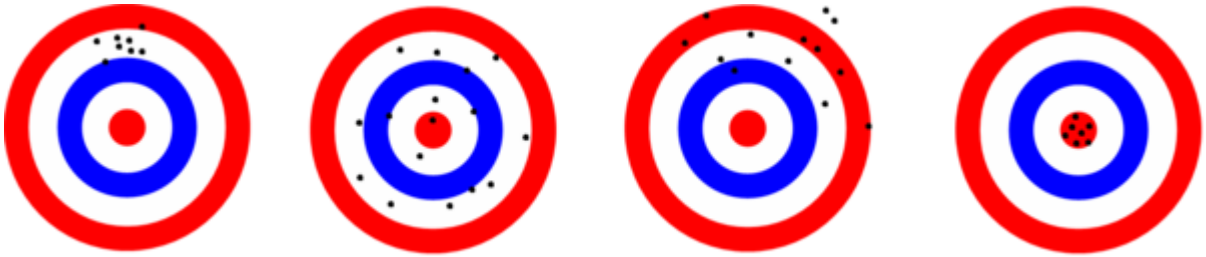
2 Chris can breathe for at least three hours



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

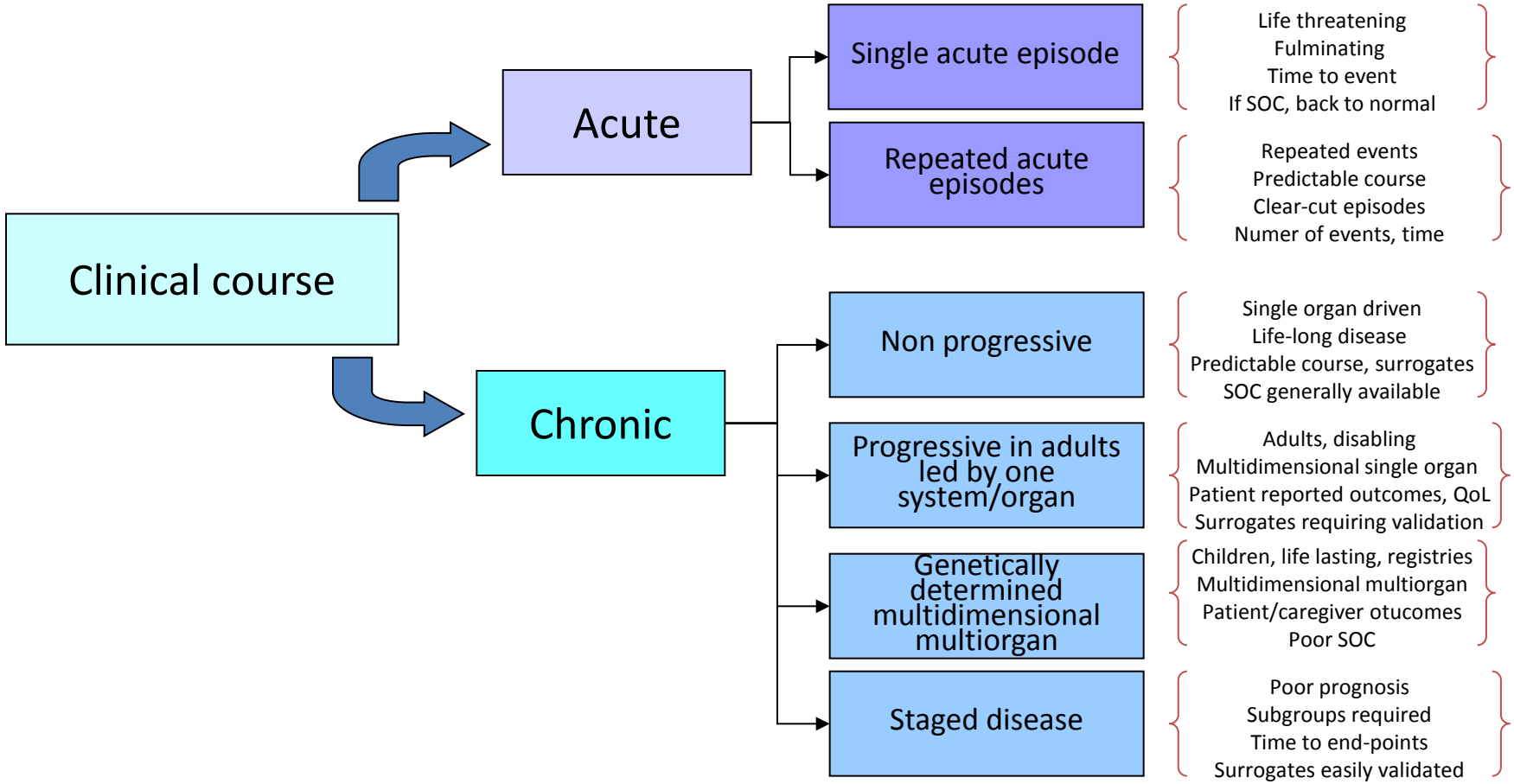
1. What are your goals, defined in 5 levels of attainment?
2. Which goals are most important to you?
3. *Intervention*
4. Have you attained your goals?





For which diseases could GAS be useful?

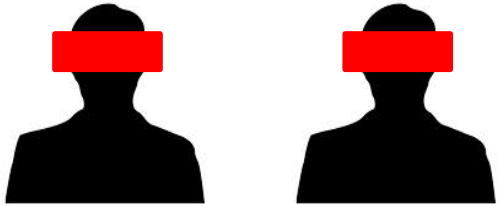
When can GAS be used?



When can GAS be used?

Useful:

- ✓ Chronic disease
- ✓ Effect of intervention expected on behavioral ability, that can be assessed independently
- ✓ Concurrent blinded controls

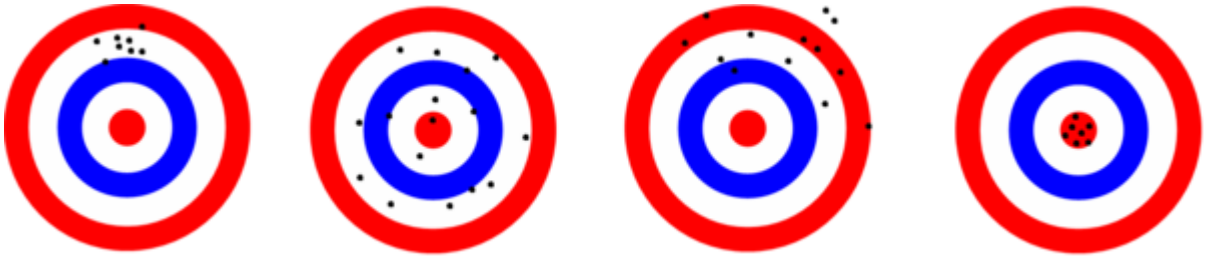


Not useful:

- Acute, episodic or unpredictable diseases
- Cross-over trials

Practical constraints

- Time
- Hawthorne effect
- Unknown or unpredictable disease course
- Lack of standardization
- Are the chosen goals realistic?



Has GAS been validated for rare disease drug studies?

Systematic review

- Is GAS used in drug studies?
- Has GAS been validated in drug studies?
- Has GAS been validated in other studies?

Gaasterland et al. BMC Medical Research Methodology (2016) 16:99
DOI 10.1186/s12874-016-0205-4

BMC Medical Research Methodology

RESEARCH ARTICLE Open Access

A systematic review to investigate the measurement properties of goal attainment scaling, towards use in drug trials

Charlotte M. W. Gaasterland[1*], Marijke C. Jansen-van der Weide[1], Stephanie S. Weinreich[1,2] and Johanna H. van der Lee[1]

Abstract

Background: One of the main challenges for drug evaluation in rare diseases is the often heterogeneous course of these diseases. Traditional outcome measures may not be applicable for all patients, when they are in different stages of their disease. For instance, in Duchenne Muscular Dystrophy, the Six Minute Walk Test is often used to evaluate potential new treatments, whereas this outcome is irrelevant for patients who are already in a wheelchair. A measurement instrument such as Goal Attainment Scaling (GAS) can evaluate the effect of an intervention on an individual basis, and may be able to include patients even when they are in different stages of their disease. It allows patients to set individual goals, together with their treating professional. However, the validity of GAS as a measurement instrument in drug studies has never been systematically reviewed. Therefore, we have performed a systematic review to answer two questions: 1. Has GAS been used as a measurement instrument in drug studies? 2: What is known of the validity, responsiveness and inter- and intra-rater reliability of GAS, particularly in drug trials?

Methods: We set up a sensitive search that yielded 3818 abstracts. After careful screening, data-extraction was executed for 58 selected articles.

Results: Of the 58 selected articles, 38 articles described drug studies where GAS was used as an outcome measure, and 20 articles described measurement properties of GAS in other settings. The results show that validity, responsiveness and reliability of GAS in drug studies have hardly been investigated. The quality of the reporting of validity in studies in which GAS was used to evaluate a non-drug intervention also leaves much room for improvement.

Conclusions: We conclude that there is insufficient information to assess the validity of GAS, due to the poor quality of the validity studies. Therefore, we think that GAS needs further validation in drug studies, especially since GAS can be a potential solution when a small heterogeneous patient group is all there is to test a promising new drug.

Trial registration: The protocol has been registered in the PROSPERO international prospective register for systematic reviews, with registration number CRD42014010619. http://www.crd.york.ac.uk/PROSPERO/display_record.asp?ID=CRD42014010619.

Keywords: Rare diseases, Goal attainment scaling, Drug trials, Validation, Systematic review

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Full list of author information is available at the end of the article

Gaasterland et al., 2016

Results

Primary search: 5459 titles & abstracts

→ 1641 duplicates removed

3818 titles & abstracts assessed for eligibility

→ 3511 articles excluded based on title & abstract

307 full text articles assessed for eligibility

→ 249 articles excluded based on full text

58 articles included

Results

- Is GAS used in drug studies?

Yes, Cerebral Palsy (Botox) and Alzheimer Disease (Donezepil)

- Has GAS been validated in drug studies?

Hardly

- Has GAS been validated in other studies?

Yes, but often with low quality

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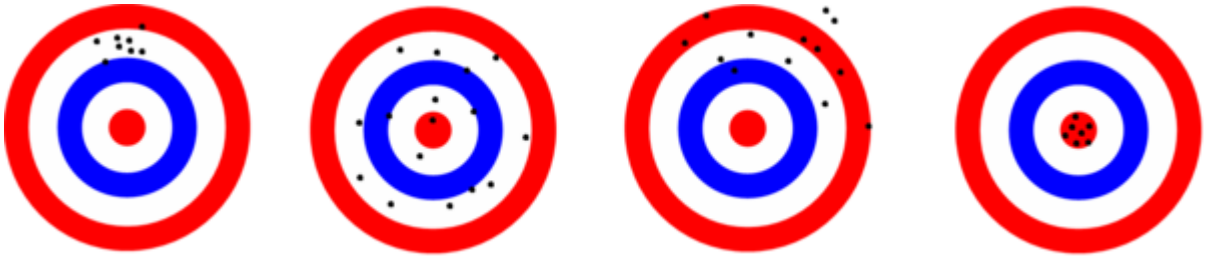


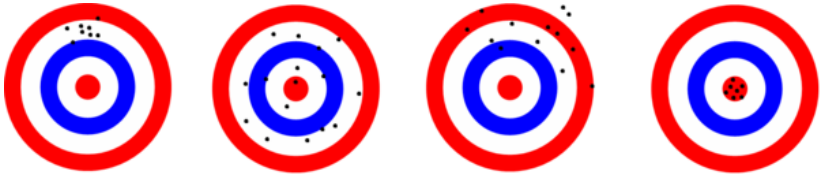
How can we further validate GAS?

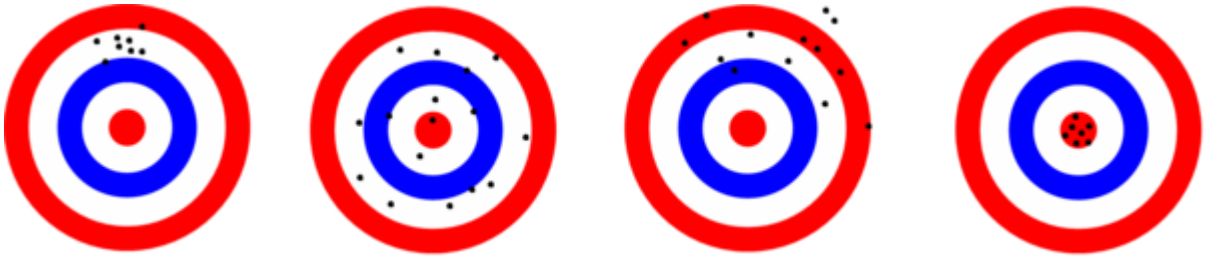
Next steps

Validation plan

Maybe during a trial?

Disease specific?





Thank you for your attention!