

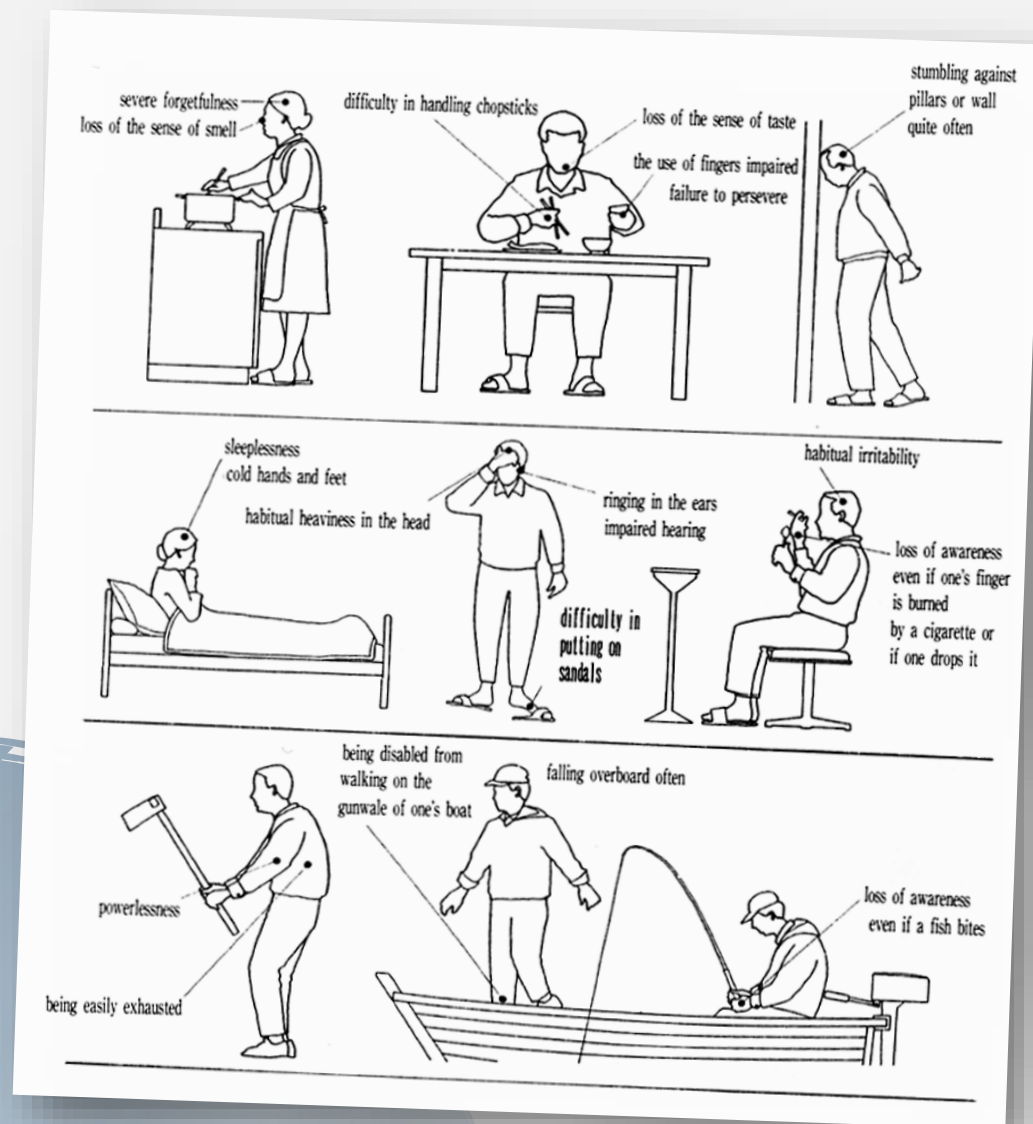


AN INNOVATIVE ENDPOINT FOR HETEROGENOUS DISEASES: GOAL ATTAINMENT SCALING

FRANÇOIS HOUÏEZ, EURORDIS

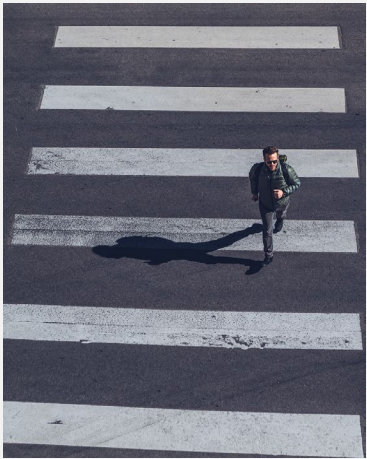
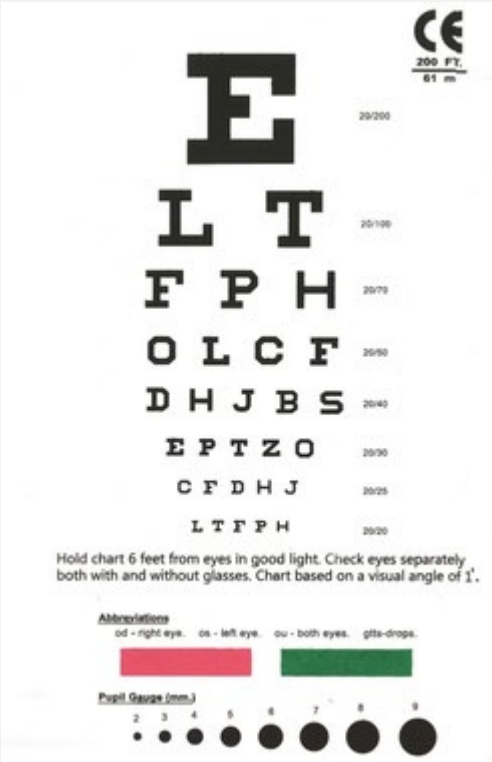
MEETING WITH ALL ELIGIBLE ORGANISATIONS,
EMA, 14-15 NOVEMBER 2023, AMSTERDAM

Same disease, different people, different manifestations

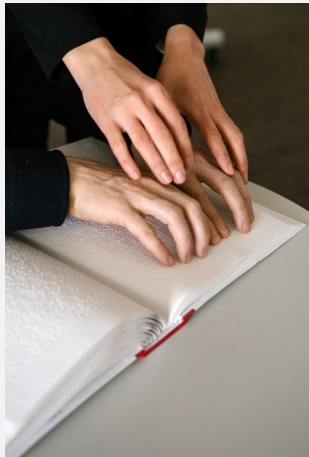


As a person, what are your main goals when changing treatment?

As per clinical trial endpoint?

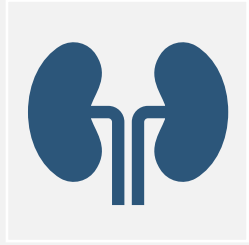


Or goals you assign to yourself?



Efficacy: asking patients to feedback on what matters to them

Individualised Efficacy Assessment: a patient-centred approach



Diseases where the product can improve many different functions/symptoms in different patients

Renal function, cognitive function, muscular function, motor skills...



Mixed-methods research can help identifying the most relevant outcomes



When there are many different outcomes:

Ask each patient to select the 3 that matter the most to him/her prior to entering the trial / starting treatment

Monitor how these 3 outcomes evolves

Analyse how many patients had 3, 2, 1 or 0 outcomes improved on treatment

Goal Attainment Scaling



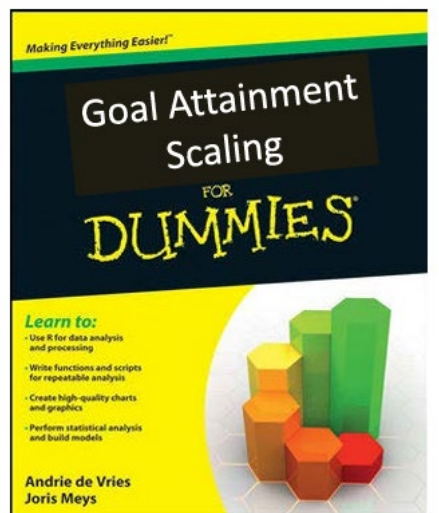
Welcome to the ASTERIX project

Advances in Small Trials dEsign for Regulatory Innovation and eXcellence

Latest news

EFSPi/BBS meeting on Small Populations - 27 June 2018, Basel

Research on Goal Attainment Scaling (GAS)



Imagine 3 boys with Duchenne disease:

Adam
'I want to be able to walk'

Brian
'I want to be able to eat independently'

Chris
'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 can 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

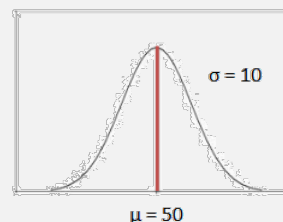


1. What are your goals, defined in 5 levels of attainment?
2. Which goals are most important to you?
3. *Intervention*
4. Have you attained your goals?



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

T = GAS score
 x_i = Original score
 w_i = Weight given to the original score
 ρ = Intercorrelation among goal scores (estimated at 0.3)



Urach S, Gaasterland C, Posch M, et al. Statistical analysis of Goal Attainment Scaling endpoints in randomised trials. *Statistical Methods in Medical Research*. 2019;28(6):1893-1910.

Goal Attainment Scaling

Systematic Review

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

*ISCB Conference Utrecht
Charlotte Gaasterland
August 2015*

Drug trials

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

Is GAS useful in drug trials?



- Is GAS used in drug trials? **YES**
- Has GAS been validated in drug trials? **HARDLY**
- Has GAS been validated in other studies?
YES, BUT GENERALLY WITH LOW QUALITY

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 Weide¹, Stephanie S. Weinreich^{1,2} and Johanna H. van der Lee¹

- https://www.asterix-fp7.eu/media/uploads/file/C38_04_GoalAttainmentScaling.pdf

Possible next steps? Could EMA pioneer its use?

A new priority for an IHI call to come?

1

- Apply GAS in phase III trials to gain more experience
- Investigate its properties in the trials

2

- Submit to EMA for endorsement / qualification as a Patient Reported Outcome Measure in selected trials

Maybe first

- To organise a scientific workshop
- At the EMA, and with HTA experts
 - With clinicians experienced in testing GAS (Kit Roes, Charlotte Gaasterland, Martin Posch...)
 - 58 articles published at the time of the Asterix Project (2016)
 - 27 more since
 - A young innovative approach!
- To discuss State-of-the-Art and research needed to adopt such an endpoint

