

Goal Attainment Scaling



● What is the issue?

Imagine three boys with a rare muscle disease, who are in different stages of their disease. All three boys have different treatment goals. Regular measurement instruments are often not specific enough to capture all these goals. How can we measure whether a treatment is successful?

● What is GAS?

Goal Attainment Scaling is a **measurement instrument** that measures the attainment of different goals of patients in a standardized way, using scores

- 0 when the goal has been attained after treatment
- 1 when a little less is attained
- 2 when even less is attained
- 1 when a little more is attained
- 2 when even more is attained

The goals are measured in the same way for every patient, but **the content of the goals can be different between patients.**

● What is the procedure?

- 1 First, a doctor or therapist and a patient together decide what the goals of the patient are, and how they can be defined in five levels. Also, the goals can be ordered in terms of importance.
- 2 The patient receives the intervention, which may be a new drug or some other treatment, or a placebo. Preferably when a placebo is used, patients and doctors do not know who gets the 'true' intervention and who gets the placebo. This is called blinding.
- 3 The patient and doctor assess how well the goals have been attained. We expect that patients who received the 'true' intervention have attained more goals and have a higher score than patients who received the placebo.



● Asterix methods

In the Asterix project, we have worked on the validation of GAS. Validation means how valuable an instrument is, and we want to know how valuable GAS is in rare disease drug research. We have investigated whether any validation studies have been done so far, and we have also written a plan on what should be done to further validate GAS in the setting of rare disease trials. Finally in a large dataset we have investigated how GAS is used best, for example how many goals should be chosen, or which statistical tests should be used.

For more information on trial designs and placebo see the EUPATI toolbox:
www.eupati.eu/discover-the-eupati-toolbox

● Possible benefits for patients

- GAS as instrument is very sensitive to change. We think that GAS will have better chance of detecting whether a treatment is effective when the regular outcome measures are not specific.
- **GAS can be used in groups of patients who all have different complaints**, which is often the case in rare diseases.
- GAS may be a good option for diseases for which no disease specific measurement instrument has been developed yet, a problem that occurs more often in rare diseases.

● Possible downsides

- GAS is **more time consuming** to use than other instruments, and can be difficult to interpret.
- GAS as measurement instrument has **not yet been validated** properly, which means that there is no consensus among researchers and regulators about its value. In the Asterix project, we are currently working on the validation of GAS.*
- We hope to convince decision makers that it is suitable as a measurement instrument for rare disease trials.

More information

A **measurement instrument** is an instrument that can be used to measure physical or psychological traits, such as machines to measure blood pressure, or questionnaires to measure personality traits.

In clinical trials, a **placebo** is a medicine that contains no active ingredients. Placebos have no known medical effects. The 'placebo effect' is a benefit or side effect perceived by patients taking a placebo, despite the fact that no medicine is involved. A placebo-controlled trial is one in which a new medicine is tested against a placebo. In placebo-controlled trials, people are assigned to a group (treatment arm) that receives the medicine, or a group that receives the placebo. This is one way to improve the chances that any benefit experienced by the treatment group receiving the medicine is due to the active ingredient in that medicine rather than some other factor.

*Gaasterland CM, Jansen-van der Weide MC, Weinreich SS, van der Lee JH. A systematic review to investigate the measurement properties of goal attainment scaling, towards use in drug trials. *BMC medical research methodology*. 2016;16(1):99.

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