

# Goal Attainment Scaling as an Outcome Measure in Orphan Drug Studies



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# *Goal Attainment Scaling*

Imagine 3 boys with Duchenne disease:



Adam

'I want to be able to walk'



Brad

'I want to be able to eat  
independently'



Chris

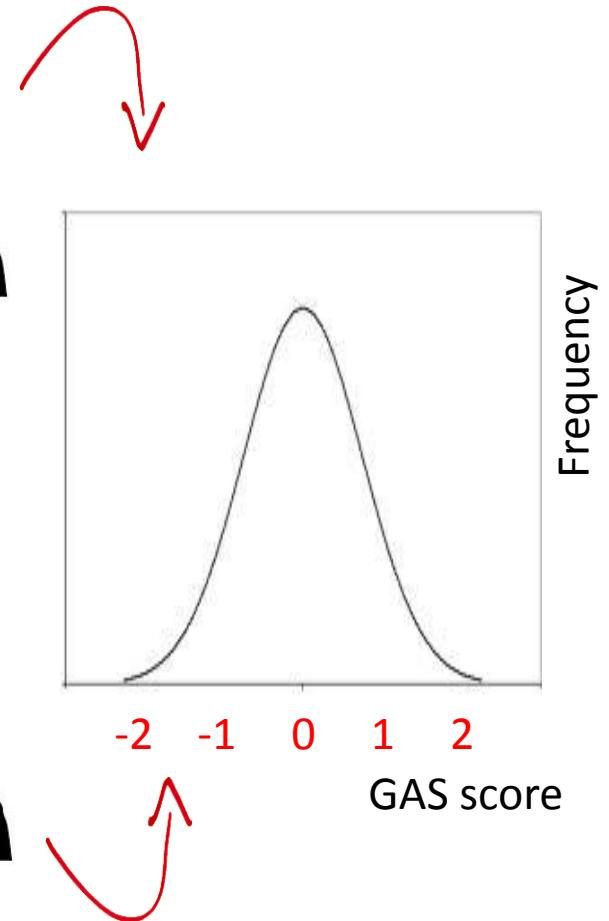
'I want to breathe  
independently'

How do we measure potential drug effect?

# Goal Attainment Scaling

- 2 Adam is unable to walk
- 1 Adam can make 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 five hours



# *Goal Attainment Scaling: Systematic Review*

*Our search yielded **3818** papers*

*We included **58** articles*

*Validity, responsiveness and reliability of  
GAS in drug studies have hardly been  
researched...*

*and need to be investigated again!*



**Thank you for your attention!**