

The Feasibility of Goal Attainment Scaling in SCN2A-associated Neurodevelopmental Disorders

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Background

Individuals living with rare neurodevelopmental disorders, like SCN2A-associated developmental and epileptic encephalopathies (SCN2A-DEE), experience severe functional impairments.

Individualized patient-centric outcomes are well suited for people living with DEEs given the heterogeneity in their disease expression across the spectrum of severity.

Goal Attainment Scaling (GAS)¹ is a patient-centric outcome that captures meaningful change by measuring progress towards individualized goals set by participants and/or their caregivers.

The objective of *The Inchstone Project* is to accelerate clinical trial readiness in the severe DEEs by adapting and developing fit-for-purpose outcome measures for this population. The aim of this pilot was to determine the feasibility of using GAS to capture clinically meaningful change in the SNC2A-DEE population.

Methods

As part of *The Inchstone Project*, caregivers of people living with SCN2A-DEE took part in the GAS pilot conducted at an SCN2A Summer Seminar in Columbus, Ohio in July 2022.

At the goal-setting interview, which took place in person during the conference, trained GAS raters guided caregivers of people living with SCN2A-DEE to identify meaningful goals. Goals were quantified on a 5-point attainment scale for evaluation of goal progress at follow-up. For each identified goal area, the caregiver gave a detailed description of the patient's current (baseline) status (-1) and goal (0). The remaining levels of the scale were set as somewhat better than the goal (+1), much better than the goal (+2), and much worse than the goal (-2).

An inventory of 26 potential goal areas, developed pre-study using a draft conceptual model of severe DEEs, was available to raters and caregivers to aid in goal setting. Caregivers also had the option to set "other" or customized goals not found in the inventory.

Follow-up interviews were conducted remotely approximately 1-3 months later, and goals were rated by both the GAS Interviewer and caregiver. Raters also completed a survey to assess the experience of performing GAS.

Results



10 participants and their caregivers participated in goal-setting interviews with four GAS interviewers (Table 1).

Table 1: Participant characteristics.

Characteristic		Participants (N=10)
Age	Mean (SD)	8.2 (5.6)
	Range	3.4 – 20.4
Sex	Female, n (%)	2 (20%)
	Male, n (%)	8 (80%)
Caregiver relationship	Parent/Guardian, n (%)	10 (100%)



Average time to conduct goal-setting interviews was 59.4 minutes (Fig 1) and average time to conduct follow-up visits was 19.8 minutes (Fig 2).

Figure 1: Distribution of the approximate time taken to conduct goal-setting interviews, by interviewer

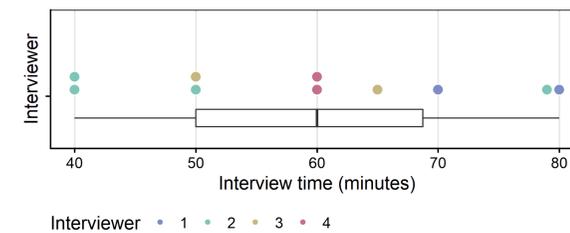
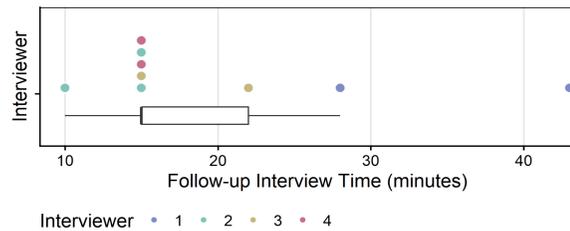


Figure 2: Distribution of the approximate time taken to conduct follow up interviews, by interviewer

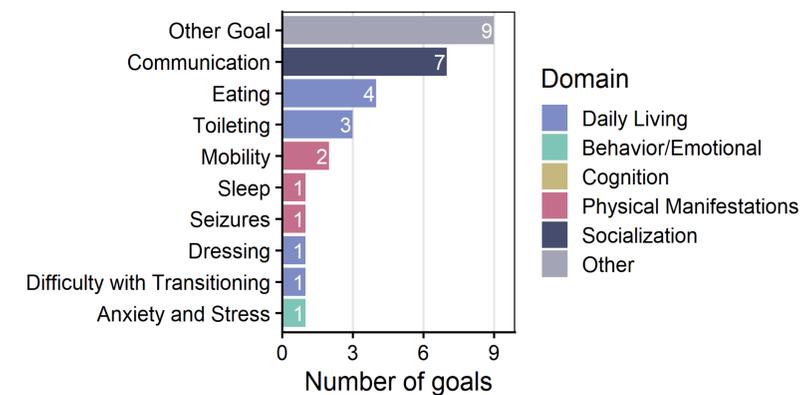


Results



21 of 30 goals were selected from the goal inventory. Most frequent goals related to communication, eating, and toileting (Fig 3).

Figure 3: Goal frequency represented by goal domains.



*Some "other" or customized goals mapped back to communication (n=4) and mobility (n=1) in the goal inventory



9 of 10 participants completed follow-up interviews.



GAS interviewer and caregiver ratings matched 100% of the time. Participants showed statistically significant progress toward goals at follow-up (Table 2).

Table 2: GAS Interviewer and Caregiver GAS scores

Multiple-Goal GAS	Scores	P value
Interviewer GAS Score	Mean (SD)	39.35 (3.95)
	Range	36.31 – 45.44
Caregiver GAS Score	Mean (SD)	39.35 (3.95)
	Range	36.31 – 45.44

Discussion & Conclusion

Over 30% of the goals set during GAS were related to communication identifying it as an important concern to caregivers of those living with SCN2A-DEE.

Most of the 9 "other" or customized goals set could be qualitatively mapped back to the inventory pointing to good content validity and adding to the feasibility of conducting GAS in this population. Four personalized goals could not be mapped back to the inventory. More research is needed to refine the inventory for this population.

Further evidence of the feasibility of GAS was captured through qualitative examination of survey responses by GAS interviewers. All GAS raters either strongly agreed or agreed that the time to conduct GAS was appropriate, that GAS interviews offered insights into the challenges and priorities of people living with SCN2A-DEE and their caregivers, and that they found the overall GAS process to be positive.

There is a critical need for sensitive and valid measures to embrace the range and complexity of disease in people living with rare neurodevelopment disorders.

These data suggest that Goal Attainment Scaling may offer a feasible, family-centered approach to capturing the patient experience and meaningful change in people most severely impacted by DEEs.

Acknowledgements & References

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