Accelerating ALS Drug Approval using Digital Health Technologies: Findings from the ADDS 2025 ALS Workshop [Poster ID: 44]

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INTRODUCTION

AMYOTROPHIC LATERAL SCLEROSIS (ALS)

- A devastating, incurable neurodegenerative disease with an urgent need for **effective treatments and therapies.**
- Despite extensive research, recent ALS drug development has faced significant setbacks, with multiple late-stage clinical trials failing to demonstrate meaningful efficacy.
- A contributor is the continued reliance on insensitive and variable outcome measures that are not patient-validated.

WHAT WE NEED

- There **is an urgent need** for more sensitive, patient-centric and objective measures of disease progression and treatment effect.
- Sensitive measures in early-stage trials can provide **early evidence of treatment effect**, encouraging investment for further development.

WHAT WE DID

- The ActiGraph Digital Data Summit (ADDS) 2025 ALS workshop brought together experts from academia, industry, regulatory agencies, and patient advocacy groups.
- **Objective** -to explore how digital health technologies (DHTs), specifically actigraphy-based measures can accelerate ALS drug development and approval.

METHODS

Fig. 1. ActiGraph Digital Data Summit (ADDS) 2025 at ActiGraph HQs in Pensacola, FL

PRESENTATIONS, ROUNDTABLE DISCUSSIONS, WHITEBOARD ACTIVITIES exploring **patient perspectives**, and the current state of **ALS drug development**, identifying **evidence gaps**, **patient-centered** outcomes, **digital measures**, **industry adoption challenges** and potential **regulatory pathways** for drug approvals.

THE AGENDA was structured into three thematic sessions

1. Current Measurement Tools and Clinical Trial Use Cases
2. Novel Digital Tools and Industry Adoption
3. Evidence Generation and Regulatory Readiness

ADDS 2025
ACTIGRAPH DIGITAL DATA SUMMIT 2025
FEBRUARY 10-12 | PENSACOLA BEACH, FL

DISCUSSION

PATIENT PERSPECTIVES

The survey demonstrated:

- HIGH DEGREE OF WILLINGNESS to incorporate actigraphy into daily life
- ALL open to wearing one or more device.
  - 76% willing to wear devices all day.
  - 32% willing to wear three or more devices
- Patients are more likely to show interest in actigraphy if it would REDUCE IN-PERSON VISITS
- Access to REAL-TIME DATA would help make decisions around care (83%) and may definitely/possibly improve quality of life (77%).
- 65% thought that measures of actigraphy will help identify periods of fatigue or pain.

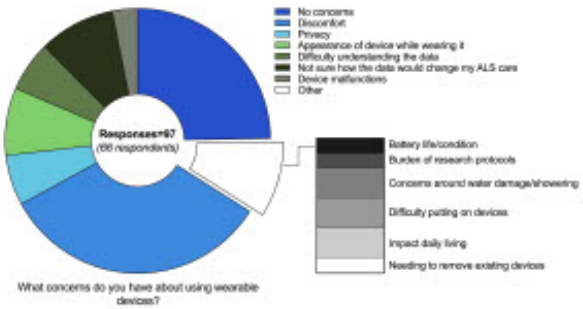

Fig. 2. Patient responses guiding feasibility and acceptance of DHTs in ALS

48% of patients with ALS identified comfort as a concern. 36% reported no wearability concerns, affirming the feasibility and acceptability of actigraphy from a patient perspective.

EVIDENCE GENERATION AND REGULATORY READINESS

Table 1. Evidence generation requirements for sponsor buy-ins and regulatory acceptance

Evidence Needed	Sponsors' buy-in	Regulatory acceptance as surrogate biomarkers or intermediate clinical endpoint (Accelerated approval)	Regulatory acceptance as a primary or secondary endpoint (Full approval)	Priority
Improved sensitivity over the ALSFRS-R	X		X	High
Strong measurement properties (low variability, high reliability)	X	X	X	High
Correlations with clinical scales (construct validity)	X	X	X	High
Ability to detect treatment effect	X	X	X	High
Precedence of success in other studies	X			Moderate
Prioritizing a measure or two over many	X			Moderate
Encouragement from regulatory bodies	X			Moderate
Explicitly defined measure			X	Moderate
Prognostic value		X		Low

CURRENT MEASUREMENT TOOLS AND THE WISHLIST

Fig. 3. Highlights of current measurement tools, their limitations and what the future measures should look like

PROPOSED STAKEHOLDER CONTRIBUTIONS ACROSS DIGITAL BIOMARKER DEVELOPMENT LANDSCAPE

Fig. 4. Proposed stakeholder contributions across the digital biomarker development landscape

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