

Tracking Daily Impact of Generalized Myasthenia Gravis Using Smartphone-Based Digital Biomarkers: Lessons Learned from a 1-Year Study with ME&MGopen™

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INTRODUCTION

- Anti-acetylcholine receptor antibody-positive (AChR-Ab+) **general myasthenia gravis (gMG)** is a rare autoimmune disease causing **muscle fatigability and exertional weakness** resulting from post-synaptic neuromuscular junction damage^{1,2}.
- Commonly affected muscles are essential for breathing, speaking and movement of the limbs or eyes. Patients experience a variety of day-to-day **fluctuating symptoms** thus it is **critical to collect real-world data** to better assess disease progression and optimize management^{2,3,4}.
- Smartphone app-based platforms appear as a promising solution providing novel digital tests and biomarkers (dBMKs) for **tracking of daily disease fluctuations and at-home collection of patient data in between distant clinical visits**^{5,6,7}.
- The decentralized clinical trial **ME&MGopen™ (NCT05566964)** collected digital biomarkers through its investigational software aiming to enhance patient engagement and characterize the clinical relevance of novel digital tools.

OBJECTIVE

To assess digital biomarkers designed to measure gMG disease severity using the ME&MGopen™ app, and describe longitudinal real-world adherence and app's usability in a cohort of patients with gMG.

METHODS

- This interim analysis includes the portion of participants with AChR-Ab+ gMG who completed the study by November 14, 2024.
- The primary endpoints were to record adherence and app usability. The secondary endpoints were to explore associations between clinical questionnaires (MG-ADL and MG-QoL-15r), MGFA disease class and ME&MGopen™ digital biomarkers.

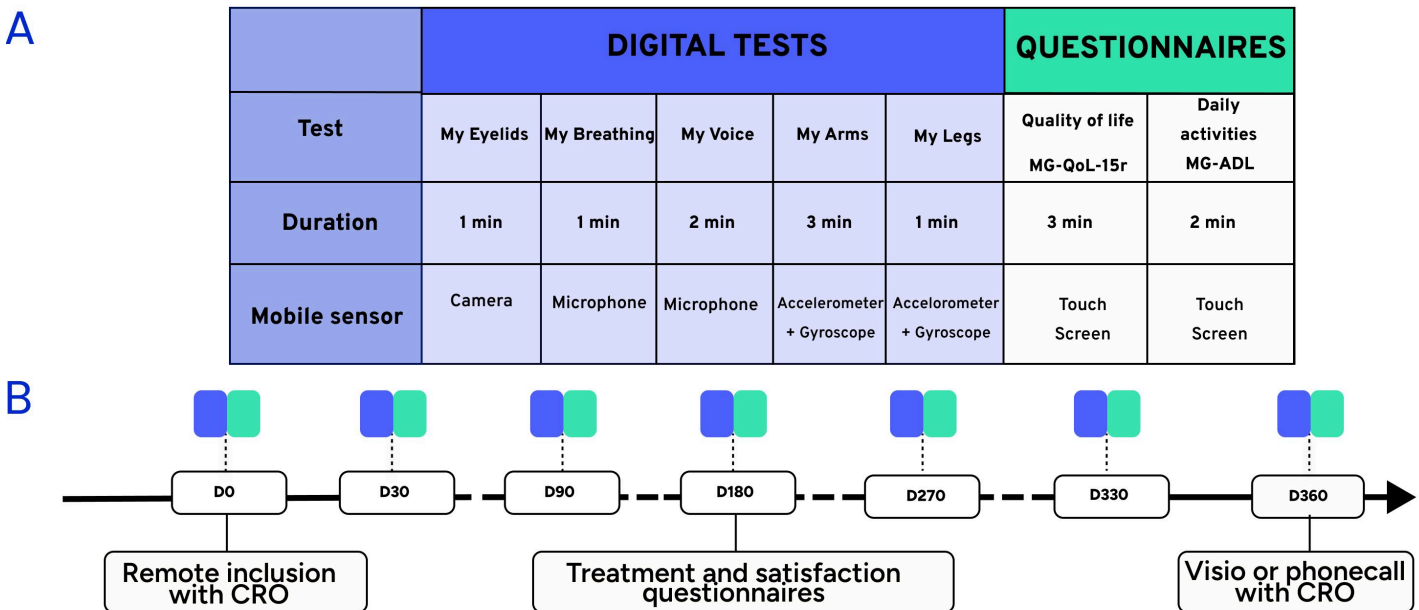


Figure 1. Study description of ME&MGopen™, a fully decentralized clinical trial. Participants, enrolled remotely, were asked to perform digital tests and answer clinically established questionnaires (A) during the 360 days of the study(B). The digital tests assessed ptosis, respiratory function, dysarthria and limb weakness.

RESULTS

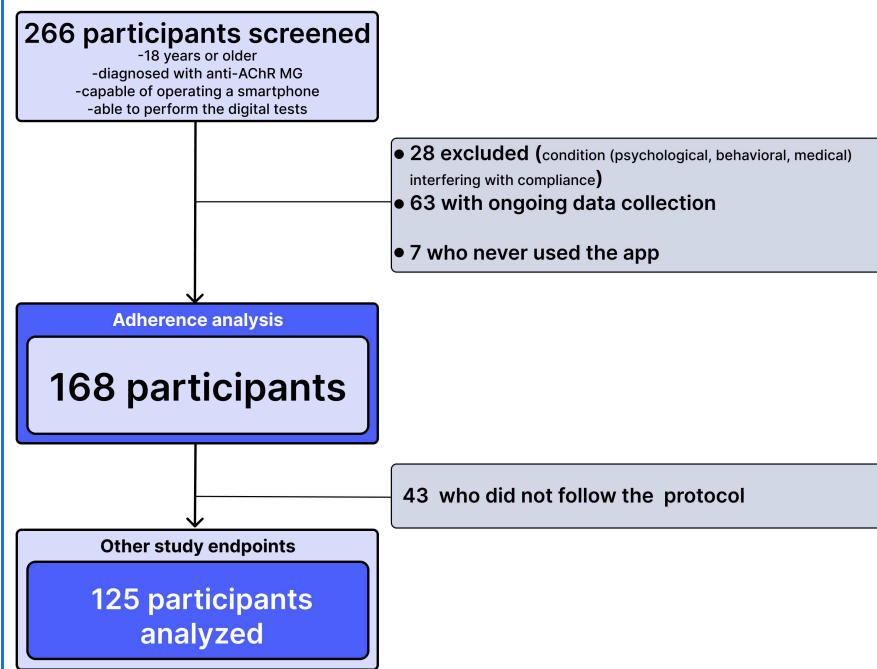


Figure 2. ME&MGopen™ interim analysis flowchart. Patients diagnosed with AChR-Ab+ gMG were enrolled and screened following this study inclusion and exclusion criteria. 43 enrolled patients having yet completed the study have not been included in this analysis.

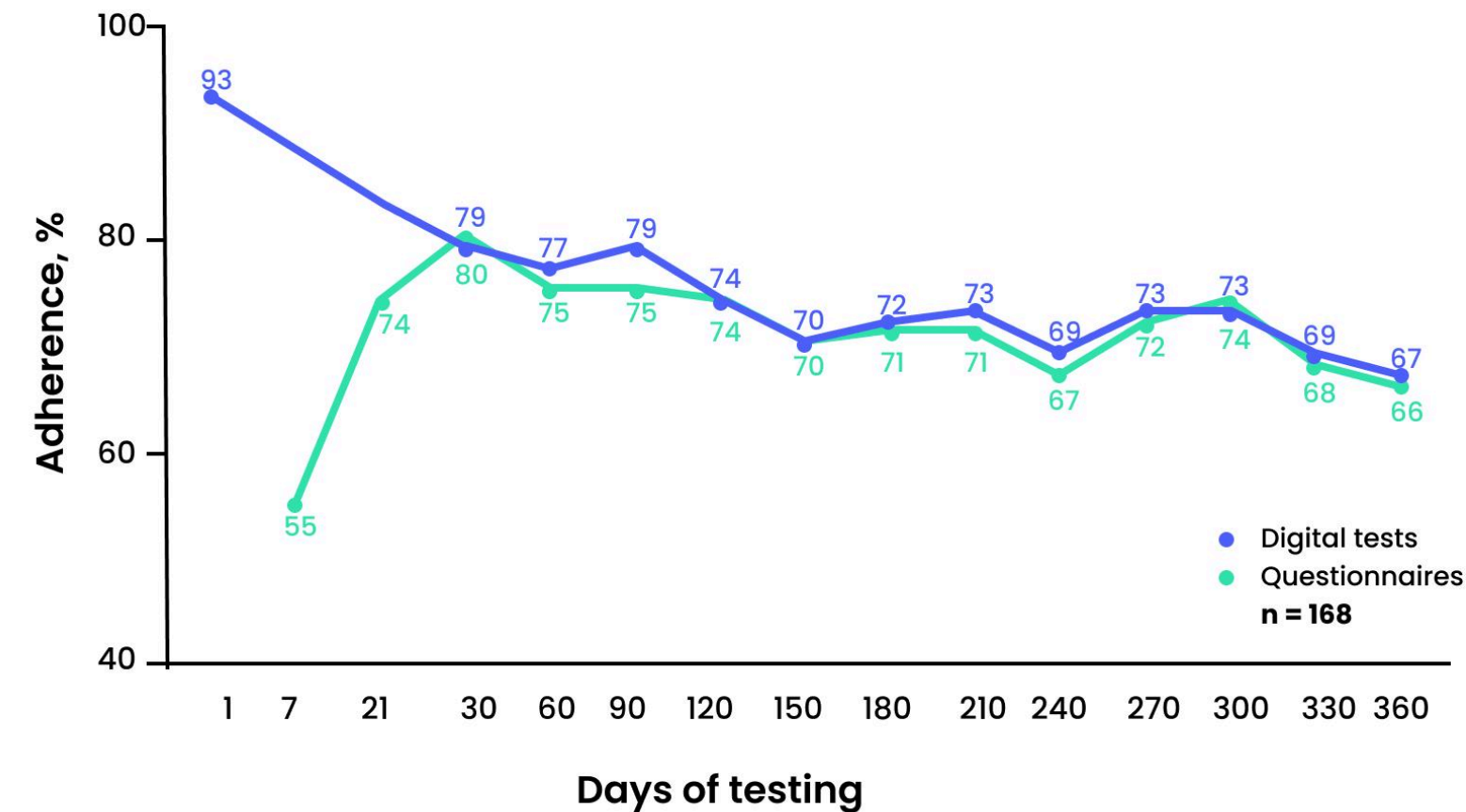


Figure 3. ME&MGopen™ participants remain adherent after 360 days. Participants respected the study protocol and completed both clinical questionnaires and digital tests.

Table 1. Baseline characteristics	
Number of patients, n (%)	125 (100)
Female	88 (70.4)
Male	37 (29.6)
Country of residence, n (%)	
Canada	8 (6.4)
United States	117 (93.6)
Age at disease onset, years (SD)	46.9 (21.0)
Disease duration, years (SD)	11.7 (13.0)
Need for caregivers, n (%)	
Yes	30 (24.0)
Thymectomy, n (%)	
Yes	65 (52.0)
MGFA disease class, n (%)	
Unreported	87 (69.6)
Class II	8 (6.4)
Class III	23 (18.4)
Class IV	7 (5.6)

Table 1. Baseline characteristics. This table summarizes the demographic and disease characteristic of patients who completed the study by the time of this interim analysis. All data is self-reported.

System usability scale

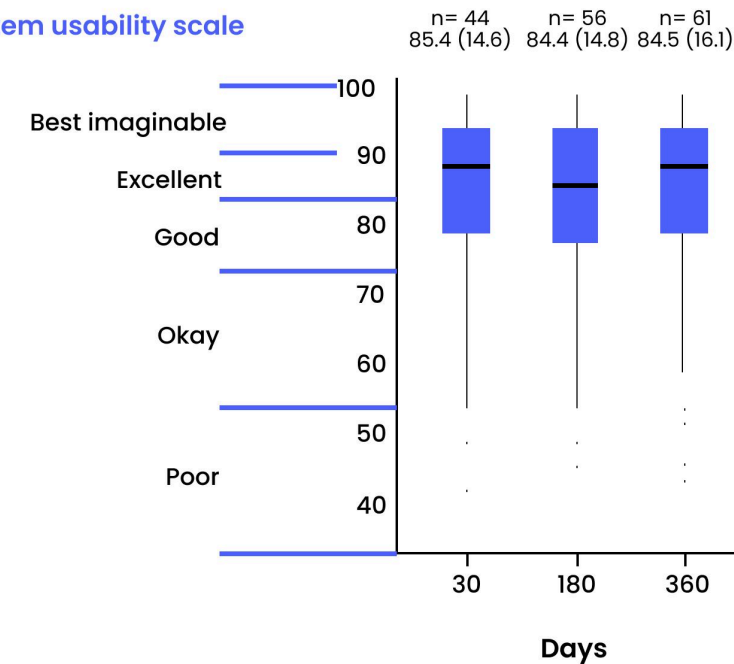


Figure 4. Participants reported ME&MGopen™ excellent software usability. The software allowed participants to efficiently and effectively complete their tests.

Table 2. Quality assessment of ME&MGOpen digital biomarkers				
	Parameter assessed	Number of evaluations, n (%)	Correlation with human annotations, R	Number of usable evaluations, n (%)
My Eyelids	MRD1	1515 (100)	0.8	1283 (84.7)
My Breathing	MPT	1527 (100)	0.99	1171 (76.7)
My Arms	Arm drop detection	1516 (100)	0.92	1117 (73.7)
My Legs	# sit-to-stand	1465 (100)	0.99	677 (46.2)
My Voice	dysarthria detection	1532 (100)	0.73	1403 (91.6)

Table 2. ME&MGopen™ digital biomarkers' quality assessments. Data collected through digital tests were evaluated to determine its quality. Values generated by the app were compared with human annotations and showed a strong correlation.

*MRD1- Margin to reflex distance 1
**MPT- Maximum phonation time

Table 3. Statistical significance between dBMK scores and subcategories of clinical MG questionnaires								
	MG-ADL subcategory					MG-QoL-15r subcategory		
	drooping eyelids	breathing score	brushing teeth/hair	arising from chair	talking	difficulties performing personal grooming	walking difficulties	speaking score
My Eyelids	p=0.019							
My Breathing		ns						
My Arms			p<0.001			p<0.001		
My Legs				p<0.001			p<0.001	
My Voice		p=0.001			p<0.001			p<0.001

Table 3. ME&MGopen™ digital biomarkers scores showed statistically significant association with scores from MG-ADL or MG-QoL-15r. Mixed models were used to assess the association between patients' digital tests scores and clinical questionnaire sub-item scores. Reported p-values show how this scores are related.

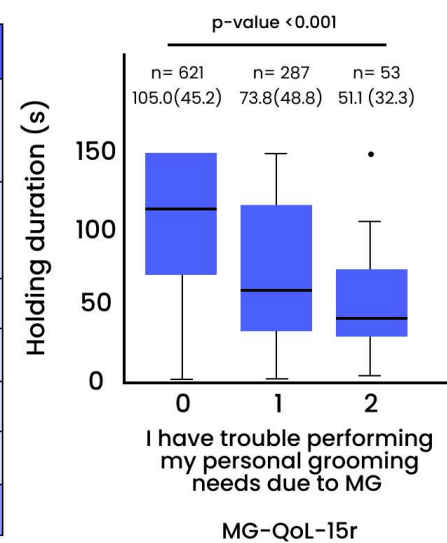


Figure 5. My Arms results showed significant association with the upper limb sub component of the MG-ADL score.

CONCLUSIONS

- Long term adherence and usability support the app potential use in clinical setting and its value in **tracking the daily impact of patients' gMG symptoms**.
- Preliminary results demonstrate associations of the app's digital biomarkers with current clinical assessments, highlighting **the importance of the ongoing validation study**.

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ACKNOWLEDGMENTS : Research sponsored by Alexion Pharmaceuticals and conducted by Ad Scientiam. Medical writing/editorial assistance were provided by Sylvia Nkombo Nkula (Ad Scientiam), Jennifer Tyson (Alexion) and Benjamin Yungher (Alexion), according to the Good Publication Practice Guidelines.
DISCLOSURES : Barnett-Tapia C.: Ad Scientiam, Alexion, Carseon Therapeutics, US Department of Defence, Muscular Dystrophy Canada, MGNet, Grifols and Octapharma research grants. Argens, Alexion, UCB, Novartis and Janssen consulting fees. Lehnerer S.: Ad Scientiam, Alexion, argens, Bogen, Hormosan, HUMA, Johnson & Johnson, Merck, UCB and Roche speaker and consulting fees, research grants, RareLink digital health GmbH, shareholder. Howard Jr JF: Ad Scientiam, Alexion, argens, Carseon Therapeutics, Centers for Disease Control and Prevention, MGFA, Muscular Dystrophy Association, NIH, NMD Pharma, and UCB Pharma research grants. AcademicCME, Alexion, Amgen, argens, Biohaven Ltd, Biologix Pharma, CheckRare CME, CoreEvitas, Curlebio, Hansa Biopharma, Medscape CME, Merck EMB Serono, Novartis Pharma, PeerView CME, Physicians' Education Resource (PER) CME, PlatformQ CME, Regeneron Pharmaceuticals, Sanofi US, TG Therapeutics, Toleranzia AB, and UCB Pharma consulting fees. Alexion, argens, Biohaven Ltd, Carseon Therapeutics, Toleranzia AB, and UCB Pharma non-financial support. Carment L., Gorin C., Ravindra D., Sellami N. and Zinai S.: Ad Scientiam — employees. Dutta B.: Alexion Pharmaceuticals Inc. — employee and may hold stock and/or stock options.

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