Capturing the patient-reported impact of myasthenia gravis in the real-world setting using a smartphone application

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Introduction

Myasthenia gravis (MG) is a chronic autoimmune disease, mediated by immunoglobulin G autoantibodies, which causes debilitating muscle weakness[1]. Patient-reported data are crucial to improve knowledge about the burden of MG and patients' unmet needs.

Objectives

To design a patient-centric real-world study to allow people with MG to provide data about disease burden using an application (app) on their smartphones.

Methods

We developed the study design and app in collaboration with patients, patient advocacy groups (PAGs), and disease experts. We also consulted health economists and statisticians.

Figure 1: Patient advocacy groups collaborating in the study design
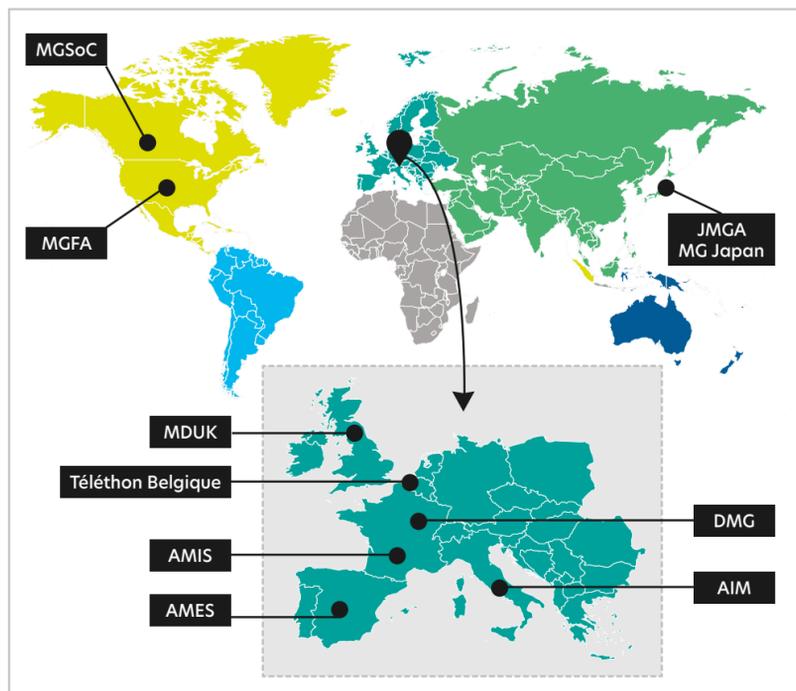
AIM = Associazione Italiana Miastenia e Malattie Immunodegenerative; AMES = Asociación Miastenia de España; AMIS = Association des Myasthéniques Isolés et Solidaires; DMG = Deutsche Myasthenie Gesellschaft e.V.; JMGA = Japan Myasthenia Gravis Association; MDUK = Muscular Dystrophy UK; MGFA = Myasthenia Gravis Foundation of America; MGSoC = Myasthenia Gravis Society of Canada

Results

We will conduct a prospective, observational, longitudinal digital study of ~2,000 people with MG.

Who can take part?

- Diagnosed with MG
- ≥18 years
- Living in one of the following countries:
  - US
  - Japan
  - Germany
  - UK
  - France
  - Italy
  - Spain
  - Canada
  - Belgium

How can people take part?

- Download the MyRealWorld® MG app
- Check eligibility and give consent
- Complete the following:
  - Personal profile on enrollment
  - Monthly event tracker over two years
  - Monthly selection of patient-reported outcome (PRO) instruments over two years

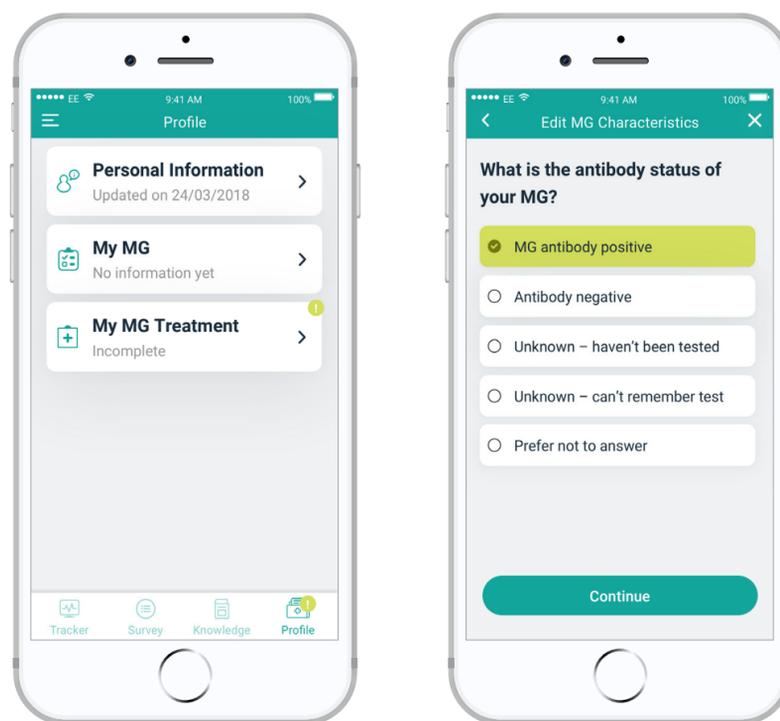Figure 2: Sample screens from the MyRealWorld MG app

Table 1: Data collected

Source	Data collected
Profile	• Demographics • MG characteristics • Diagnosis and past treatment • Past healthcare resource use

Tracker	• In past month:
	  • Routine and rescue treatments
	  • Treatment side effects
	  • Work/study productivity
	  • Healthcare resource use

Core PRO instruments (all participants)	• EuroQol Five-Dimension Health Survey, five-level version (EQ-5D-5L) • EQ-5D-5L bolt-ons for vision, breathing problems, sleeping, tiredness, self-confidence, social relationships • Myasthenia Gravis Activities of Daily Living (MG-ADL) • Myasthenia Gravis Quality of Life 15-item revised scale (MG-QoL15r) • Hospital Anxiety and Depression Scale (HADS) • Health Utilities Index III (HUI3)
Opt-in PRO instruments (participants who opt-in for completion)	• PROMIS Dyspnea Characteristics • FACIT-Fatigue • PROMIS Sleep Disturbance short form 6a

What support will be provided to engage participants?

- Notifications and in-app reminders
- Messages of thanks for data provided
- Updates on study findings via app and newsletters
- Clinician-validated in-app information about MG and its management

How will the data be analyzed?

Formal descriptive analyses are planned for 6, 12, 18, and 24 months after study launch.

Near real-time descriptive analyses of key variables will also be displayed on an ongoing basis via interactive 'dashboards' in an online research portal. External researchers will be able to apply for access to the portal.

Conclusions

Collecting data from people with MG via a smartphone app should enable a granular dataset to be generated, to help researchers and clinicians understand the real-world impact of the condition and the unmet needs of patients.