316

Baseline Characteristics and Demographics of Patients Enrolled in an Early-Access Program for Efgartigimod in Adult Patients With Generalized Myasthenia Gravis

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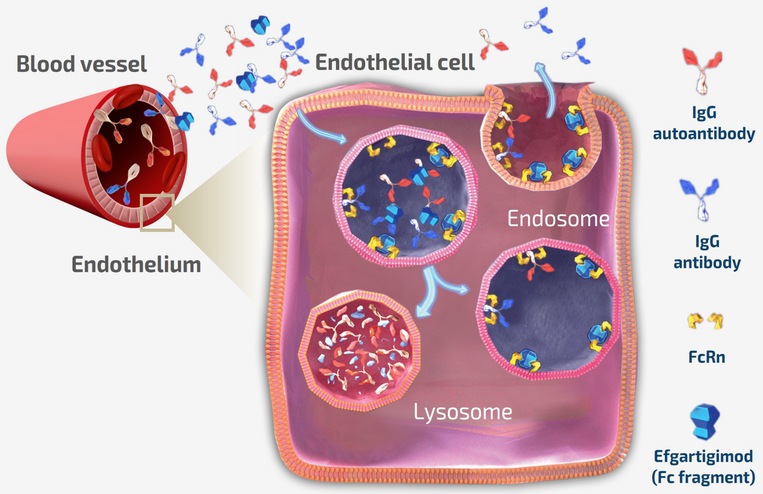
Background



FcRn, neonatal Fc receptor; IgG, immunoglobulin G.
Image adapted from Kang TH, Jung ST. Boosting therapeutic potency of antibodies by taming Fc domain functions. *Exp Mol Med*. 2019;51:1–9, and distributed under the terms of the Creative Commons CC-BY license (https://creativecommons.org/licenses/by/4.0/).

- Immunoglobulin Gs (IgGs) are regulated by the neonatal Fc receptor (FcRn), which rescues them from lysosomal degradation and returns them to circulation[1]
- Efgartigimod is a human IgG1 Fc fragment, a natural ligand of FcRn, engineered for increased affinity to FcRn[2]
- Efgartigimod was designed to outcompete endogenous IgG, preventing recycling and promoting lysosomal degradation of IgG without impacting its production[2–5]
  - Targeted reduction of all IgG subtypes
  - No impact on IgM or IgA
  - No reduction in albumin levels
  - No increase in cholesterol
- The ADAPT trial demonstrated that efgartigimod was efficacious and well tolerated, after which argenx made efgartigimod available through an early-access program, which offers pre-approval access to efgartigimod for patients with myasthenia gravis (MG) in need of further symptom control
- Efgartigimod is now approved for the treatment of generalized MG (gMG) in the United States (US) and Japan

Summary

- The argenx early-access program for efgartigimod provides further patient data on this rare disease, covering a more complete spectrum of the diverse patient population
- For patients lacking an effective management strategy, the program offers an important treatment option
- Enrolled patients had used the range of treatment options available to them, yet still had unresolved symptoms, including 58% who had experienced crisis or hospitalization in the 12 months prior to screening
- Owing to the FDA approval in 2021, patient enrollment has ceased in the US but continues in Europe. Additional insight regarding the status of the enrolled patients will be provided in future presentations and publications

Methods and Results

Study Inclusion Criteria and Treatment Dosing

- Patients are eligible for the early-access program if they meet the following criteria:
  - Adult patients (≥18 years of age)
  - Confirmed diagnosis of gMG, regardless of antibody status
  - Total Myasthenia Gravis Activities of Daily Living score ≥5 at screening (with >50% of the total score due to nonocular symptoms)
  - Documented IgG level of >6 g/L 1 month prior to screening
    - Patients with IgG levels between 4–6 g/L, after confirming no evidence of immune deficiency, will be discussed with the sponsor, and the decision to include these patients will be made individually
- Enrolled patients receive an initial cycle of 4 intravenous infusions of efgartigimod (10 mg/kg) weekly, followed by an individualized treatment regimen
- Percentages are derived from the number of patients with available/recorded data

Patient Enrollment

- Patients were enrolled in Europe and the US. As of August 12, 2022, 90 patients were enrolled across these regions. Enrollment ended in the US owing to the approval of efgartigimod by the US Food and Drug Administration (FDA), but is continuing in Europe

Time from diagnosis: 5 years (Median, n=61)

History of thymectomy: 27% (n=63)

Patients previously experiencing MG exacerbation or crisis: 58% (within 12 months of screening, n=64)

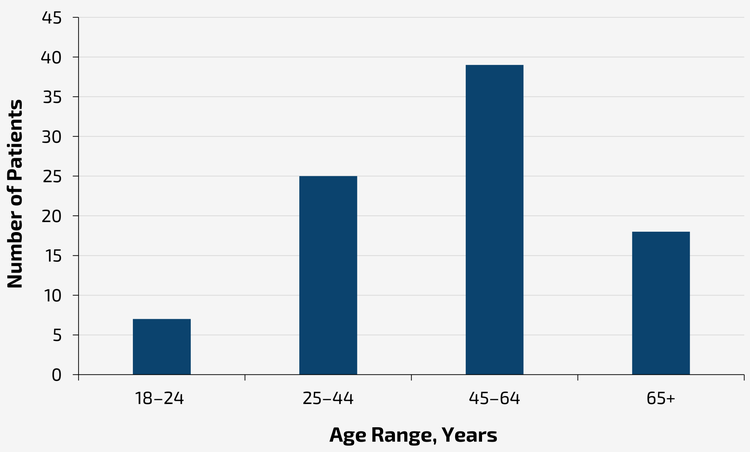
Patients with ≥2 comorbidities: 63% (n=63)

MG, myasthenia gravis.

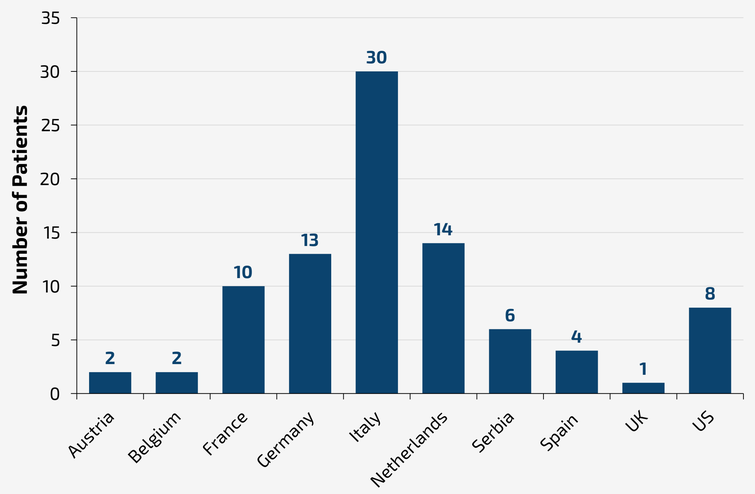
Demographics (n=88)

♀ **68% (n=60)** female ♂ **32% (n=28)** male

Patient Age Subgroups (N=89)



Patients per Country (N=90)



Baseline Characteristics

Most Common (≥15% of Patients) Prior Treatments, n (%)	Patients (n=62)
Intravenous Ig	27 (44)
Azathioprine	26 (42)
Plasma exchange/immunoadsorption	22 (35)
Steroids	17 (27)
Rituximab	15 (24)
Cyclosporine	11 (18)
Mycophenolate mofetil	11 (18)
Eculizumab	9 (15)

Ig, immunoglobulin.

MGFA Classification, n (%)*	Patients (n=85)
II	12 (14)
II A	3 (4)
II B	7 (8)
III	25 (29)
III A	9 (11)
III B	14 (16)
IV	48 (56)
IV A	45 (53)
IV B	1 (1)

*MGFA subclass unknown for some patients.
MGFA, Myasthenia Gravis Foundation of America.

Ab Status, n (%)	Patients (n=85)
AChR	55 (65)
MuSK	9 (11)
LRP4	1 (1)
Negative	20 (24)

Ab, antibody; AChR, acetylcholine receptor; LRP4, low-density lipoprotein receptor-related protein 4; MuSK, muscle-specific tyrosine kinase.

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