

Factors Associated with Recurrent Clinical Events Among Pediatric Generalized Myasthenia Gravis Patients in the United States

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Background

- Myasthenia gravis (MG) is a rare, chronic autoantibody disease, characterized by fluctuating muscle weakness and fatigability¹
 - Children presenting with ocular MG may go on to develop generalized MG (gMG), usually within 6 months of symptom onset^{2,3}
- Among children in the United States, prevalence of MG is estimated at 25.3 (95% confidence interval [CI], 19.9–32.2) per million population for commercially insured patients and 37.6 (95% CI, 31.9–44.4) per million population for Medicaid recipients⁴
- Acute clinical events (MG exacerbation and myasthenic crisis) are costly,^{5,6} and patients with higher disease activity have lower health-related quality of life^{7,8}
- There are limited data on the occurrence of acute clinical events in pediatric patients with gMG

Objectives

- To estimate rates of MG exacerbation and myasthenic crisis and identify associated risk factors for myasthenic crisis and repeat exacerbations among commercially insured and Medicaid-recipient children and young adults with gMG in the United States

Methods

- This was a longitudinal, retrospective cohort study using data from the Merative™ MarketScan® Commercial Claims and Encounters Database and the Multi-State Medicaid Database
- Eligibility criteria for the gMG cohort were as follows:
 - ≥1 qualified MG event between January 1, 2010, and June 30, 2025 (≥1 claim with an MG diagnosis code [International Classification of Diseases, Ninth Revision, Clinical Modification codes 358.00/358.01 or Tenth Revision, Clinical Modification codes G70.00/G70.01] in the primary billing position for an inpatient visit or ≥2 claims on separate days with an MG diagnosis code in any billing position for an outpatient/emergency department/other visit except for inpatient within a 60-day period)
 - Age 2–25 years on index date (date of qualified MG event)
 - ≥6 months’ continuous health plan enrollment before index date
- Exclusion criteria were presence of congenital and developmental myasthenia or ocular MG
- Patients were followed until the earliest of disenrollment, death, or end of data
- The number of patients with gMG experiencing MG exacerbation or myasthenic crisis during follow-up (after index date) and the rate per 100 person-years were calculated
- The association between selected variables on the occurrence of MG exacerbation or myasthenic crisis during follow-up was assessed using a case–control analysis with regression models

Results

Cohorts of pediatric and young adult patients with gMG were identified						
Characteristic	Commercially Insured Patients (N=1,361)			Medicaid Recipients (N=681)		
	Pre-Pubertal ^a (n=226)	Post-Pubertal ^a (n=282)	Young Adults ^a (n=853)	Pre-Pubertal ^a (n=218)	Post-Pubertal ^a (n=179)	Young Adults ^a (n=284)
Sex, female, n (%)	139 (61.5)	196 (69.5)	642 (75.3)	127 (58.3)	126 (70.4)	219 (77.1)
Age, mean (SD), years	6.8 (2.9)	14.9 (1.7)	21.9 (2.3)	6.2 (2.9)	14.7 (1.7)	22.0 (2.4)
Length of follow-up, median, months	25.5	23.0	18.0	51.5	32.0	29.0
Insurance plan non-capitated (fee-for-service), n (%)	195 (86.3)	249 (88.3)	751 (88.0)	84 (38.5)	73 (40.8)	139 (48.9)
Prior MG exacerbation, n (%)	1 (0.4)	2 (0.7)	6 (0.7)	1 (0.5)	2 (1.1)	2 (0.7)
Prior myasthenic crisis, n (%)	1 (0.4)	1 (0.4)	3 (0.4)	1 (0.5)	2 (1.1)	2 (0.7)
Comorbidities ^b , n (%)						
Pharyngitis	33 (14.6)	37 (13.1)	62 (7.3)	38 (17.4)	23 (12.8)	17 (6.0)
Otitis media	25 (11.1)	8 (2.8)	11 (1.3)	21 (9.6)	2 (1.1)	2 (0.7)
Asthma	24 (10.6)	21 (7.4)	52 (6.1)	29 (13.3)	19 (10.6)	28 (9.9)
Allergic inflammation of the nasal airways	23 (10.2)	22 (7.8)	50 (5.9)	22 (10.1)	16 (8.9)	9 (3.2)
Autoimmune condition	5 (2.2)	29 (10.3)	113 (13.2)	10 (4.6)	18 (10.1)	30 (10.6)
Anxiety	4 (1.8)	26 (9.2)	113 (13.2)	9 (4.1)	21 (11.7)	28 (9.9)
Fluid and electrolyte disorders	3 (1.3)	4 (1.4)	23 (2.7)	7 (3.2)	11 (6.1)	29 (10.2)
Depression	1 (0.4)	8 (2.8)	85 (10.0)	6 (2.8)	19 (10.6)	32 (11.3)
Treatments ^c , n (%)						
Acetylcholinesterase inhibitors	69 (30.5)	74 (26.2)	222 (26.0)	69 (31.7)	58 (32.4)	77 (27.1)
Corticosteroids	34 (15.0)	59 (20.9)	199 (23.3)	52 (23.9)	35 (19.6)	53 (18.7)
Fc receptor antagonist	0	0	0	0	0	0
Nonsteroidal immunosuppressant	3 (1.3)	17 (6.0)	67 (7.9)	3 (1.4)	10 (5.6)	17 (6.0)
Anti-CD20 monoclonal antibody	0	0	3 (0.4)	0	0	0
Complement C5 inhibitors	8 (3.5)	3 (1.1)	8 (0.9)	10 (4.6)	6 (3.4)	12 (4.2)
Intravenous immunoglobulin	1 (0.4)	6 (2.1)	20 (2.3)	1 (0.5)	5 (2.8)	8 (2.8)
Subcutaneous immunoglobulin	0	0	2 (0.2)	0	0	0
MG-related thymectomy	0	0	0	0	0	0
MG-related plasmapheresis	0	2 (0.7)	9 (1.1)	0	1 (0.6)	4 (1.4)

^aPre-pubertal patients were aged 2–11 years, post-pubertal patients 12–17 years, and young adult patients 18–25 years. ^bComorbidities present at any time up to the index date (only those present in ≥10% of patients in any group are shown). ^cTreatments received at any time up to the index date. **Fc**=fragment crystallizable, **gMG**=generalized myasthenia gravis, **MG**=myasthenia gravis, **SD**=standard deviation.



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Key Takeaways

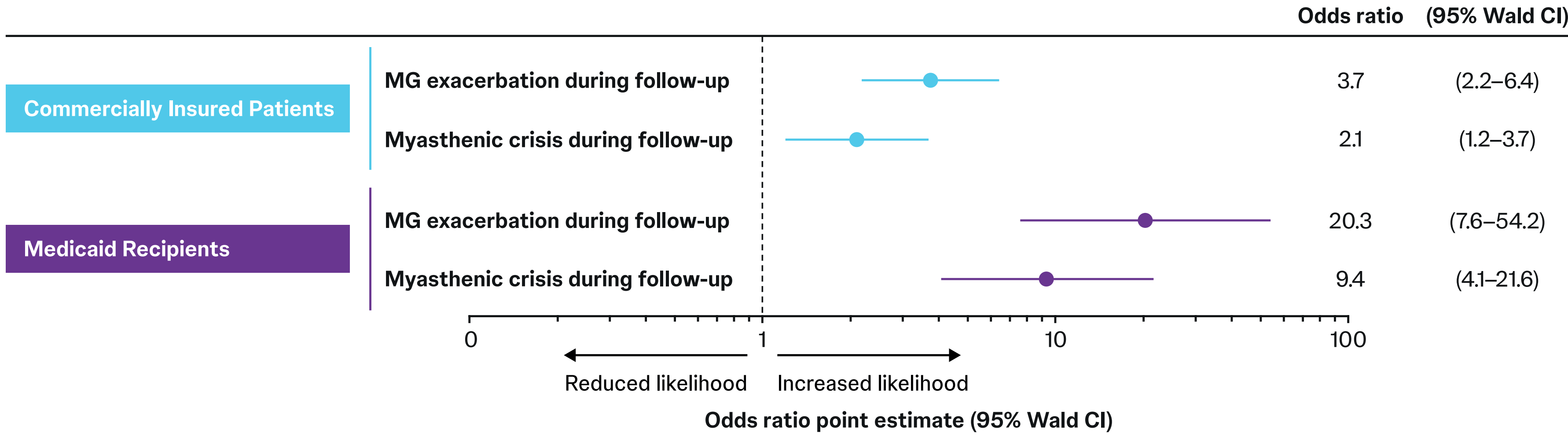
- Many pediatric and young adult patients with gMG experienced acute clinical events (MG exacerbation and myasthenic crisis)
- The occurrence of acute clinical events was strongly associated with prior events
- This study highlights the need for improved management of gMG symptoms for pediatric and young adult patients to reduce the frequency of acute clinical events

Pediatric and young adult patients with gMG had high rates of MG exacerbation and myasthenic crisis during follow-up

Event	Commercially Insured Patients (N=1,361)			Medicaid Recipients (N=681)		
	Pre-Pubertal ^a (n=226)	Post-Pubertal ^a (n=282)	Young Adults ^a (n=853)	Pre-Pubertal ^a (n=218)	Post-Pubertal ^a (n=179)	Young Adults ^a (n=284)
MG exacerbation ^b	Number of patients with ≥1 event, n (%)	57 (25.2)	98 (34.8)	204 (23.9)	65 (29.8)	73 (40.8)
	Number of events in patients with ≥1 event ^c , mean (SD)	2.2 (2.9)	2.1 (2.0)	1.7 (2.0)	2.2 (2.1)	2.3 (1.8)
	Rate (95% CI) per 100 person-years	18.2 (11.4–29.1)	22.8 (16.9–30.1)	19.3 (15.9–23.5)	13.3 (9.4–18.8)	26.9 (19.9–36.4)
Myasthenic crisis ^d	Number of patients with ≥1 event, n (%)	35 (15.5)	47 (16.7)	116 (13.6)	29 (13.3)	42 (23.5)
	Number of events in patients with ≥1 event ^c , mean (SD)	1.8 (1.9)	1.4 (0.8)	1.4 (0.7)	2.1 (1.9)	1.8 (1.4)
	Rate (95% CI) per 100 person-years	9.3 (5.1–17.1)	7.3 (4.7–11.4)	8.7 (6.8–11.2)	5.8 (3.3–10.1)	12.0 (8.2–17.5)

^aPre-pubertal patients were aged 2–11 years, post-pubertal patients 12–17 years, and young adult patients 18–25 years. ^bMG exacerbation = inpatient episode where MG was principal diagnosis or patient had respiratory failure as principal diagnosis and MG diagnosis in same episode. ^cPatients could have multiple events. ^dMyasthenic crisis = MG exacerbation where intensive care unit service also identified (except for surgical purposes), patient received mechanical ventilation, or patient had respiratory failure as principal diagnosis and MG diagnosis in the same episode. **CI**=confidence interval, **gMG**=generalized myasthenia gravis, **MG**=myasthenia gravis, **SD**=standard deviation.

Among pediatric and young adult patients with gMG, previous MG exacerbation was associated with recurrence of MG exacerbation and myasthenic crisis during follow-up in a case–control, regression analysis



Controlled variables were age category, sex, insurance plan, presence of comorbidities (hypertension, hypothyroidism, obesity, weight loss, fluid and electrolyte disorders, depression, anxiety, autoimmune condition, bronchitis, pharyngitis, allergic inflammation of the nasal airways, otitis media, asthma), and treatment received (acetylcholinesterase inhibitor, corticosteroid, nonsteroidal immunosuppressant, anti-CD20 monoclonal antibody, complement C5 inhibitors, intravenous immunoglobulin). **CI**=confidence interval, **gMG**=generalized myasthenia gravis, **MG**=myasthenia gravis.