EE361

Economic Burden of Generalized Myasthenia Gravis (gMG) in Latin America (LA): a Micro-costing Analysis of Direct Medical Costs (DMCs)

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Key Takeaways

- **gMG Patients in later treatments present greater resource use and direct costs, placing a significant burden on the healthcare system.**
- **The higher rates of disease exacerbations and crises among patients in later treatments are an important driver in the cost-per-patient, showing the impact of uncontrolled disease.**
- **Patients in later treatments appear to not achieve disease control, highlighting the need for improved disease management strategies**

Background

- Generalized myasthenia gravis (gMG), a rare autoimmune disease marked by fluctuating muscle weakness, exerts a negative impact on patient's ability to perform daily activities and quality of life
- The frequent disease fluctuations, the variable and unpredictable disease course, and the heterogeneity in response to therapy are important challenges in gMG management, imposing a high burden on patients and caregivers and increasing healthcare resource use (HCRU)[1-3]
- In Latin America, decision-making in clinical practice and healthcare policy are impacted by the limited treatment options available, and by the lack of evidence on patient profile, treatment patterns, and on HCRU and costs associated with gMG[4]
- A three-step approach comprising a target literature review, a modified Delphi panel with experts, and a micro-costing analysis was used to investigate the individual and societal impact of gMG in Latin America. Diagnosis and treatment challenges were previously reported.[5] Herein, we report results related to HRCU and costs

Objectives

To assess the economic burden of gMG in Latin America through the estimation of DMCs across the treatment sequence

Methods

LITERATURE REVIEW

- Identification of studies describing data related to gMG:
  - epidemiology
  - disease burden
  - unmet medical needs
  - local practice guidelines
- Studies published in English, Spanish or Portuguese
- Data collection and assumptions on the epidemiology, treatment profile, HCRU, and adverse events to be validated

MODIFIED DELPHI PANEL

- 28 key opinion leaders from Argentina, Brazil, Colombia, and Mexico
  - Eligibility criteria: neuromuscular specialists involved in gMG care (private or public settings); ≥ 5 years of experience treating gMG patients after medical residency; and ≥ 50% of professional time dedicated to direct patient care
- Panel steps:
  - Step 1: a survey was individually responded by the experts
  - Step 2: panel discussions were held separately in each country to reach consensus, validate and further explore the findings
- Aim: to capture perceptions on treatment challenges and HCRU

MICROCOSTING ANALYSIS

- A bottom-up costing approach was used, with identification of HCRU in clinical practice followed by the attempt to estimate the total cost related to the disease
- HCRU
  - Estimated using epidemiological data and expert input
  - Calculated based on the proportion of patients undergoing each procedure and the frequency of their use
  - Analysis included pharmacological treatments, IVIg, plasma exchange, and healthcare services such as outpatient care, emergency visits, hospitalizations, and ICU admissions
- Costs were calculated by multiplying official unit costs based on the frequency and duration of HCRU for each resource. Procedure prices were taken from standard price lists for each country. Expenses were converted to US dollars (USD, World Bank's rates) using a Purchasing Power Parity methodology

Results

Panel participants

- The panel was composed by seven neuromuscular specialists from each of the four countries of interest totalizing 28 members
- Physicians profile:
  - Experience time: ranged from 5 to 30 years (median: 12 years)
  - Annual number of gMG patients under care: ranged from 10 to 300 patients (median: 32 patients)

Key estimates used as assumptions for the cost analysis (per country)

	ARG	BRA	COL	MEX
Adult population[1]	36,048,082	173,131,214	41,084,789	97,601,219
gMG prevalence	13,338 (37 cases / 100,000)	34,626 (20 cases / 100,000)	5,875 (14.3 cases / 100,000)	10,736 (11 cases / 100,000)
Diagnosed patients[2]	9,470 (71%)	27,008 (78%)	3,408 (58%)	6,227 (58%)
Treated gMG population[2]	8,144 (86%)	24,038 (89%)	2,726 (80%)	5,106 (82%)
First treatment*	3,665 (45%)	7,211 (30%)	1,118 (41%)	1,838 (36%)
Second treatment*	3,421 (42%)	10,577 (44%)	1,063 (39%)	2,247 (44%)
Third treatment*	652 (8%)	4,808 (20%)	436 (16%)	664 (13%)
Fourth and subsequent treatments*	407 (5%)	1,442 (6%)	109 (4%)	357 (7%)
AChR+ patients[3]	6,761 (83% of cases)	19,471 (81% of cases)	2,317 (85% of cases)	4,340 (85% of cases)

*[1]Source: World Bank, September 2024. [2]Other subtypes (muscle-specific tyrosine kinase [4 to 10%], low-density lipoprotein receptor-related protein [1%], and seronegative [8 to 10%]) were also estimated by panelists, but there is generally insufficient access to testing in the region.[3] Estimated according to medical experts' perspective. [3] AChR+: Acetylcholine receptor antibody positive. * As percentage of treated patients*

Estimates of HCRU in each country over the past 12 months, pooling all treatment lines

Percentage of patients requiring each of the resources analysed and the mean frequency of utilization

Exacerbations

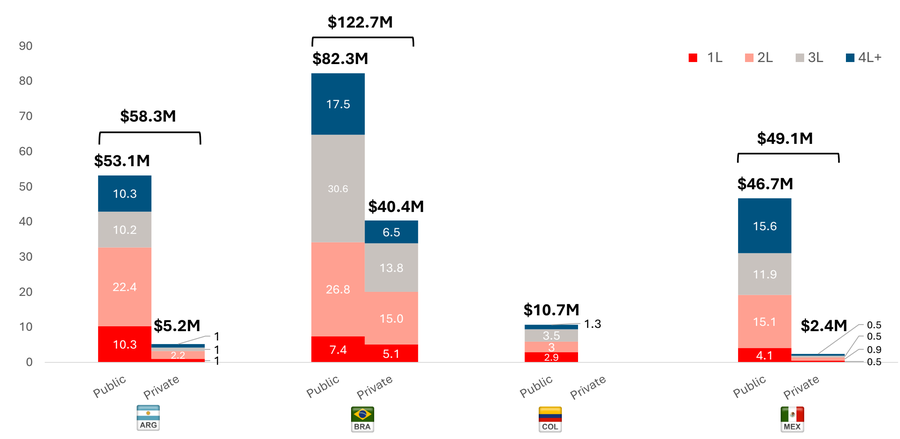
Item	ARG Patients	ARG Mean utilization	BRA Patients	BRA Mean utilization	COL Patients	COL Mean utilization	MEX Patients	MEX Mean utilization
Hospital stay	61%	6 days	41%	8 days	66%	10 days	33%	6 days
ICU stay	13%	4 days	26%	4 days	31%	5 days	14%	4 days
IVIg	35%	5*	44%	5*	17%	5*	30%	5*
PE	11%	5*	22%	5*	44%	6*	14%	5*
VS	10%	1**	17%	1**	12%	2**	11%	2**
OM	25%	1**	50%	1**	52%	1**	46%	1**
Ambulance	30%	1**	12%	1**	35%	1**	20%	1**

Crises

Item	ARG Patients	ARG Mean utilization	BRA Patients	BRA Mean utilization	COL Patients	COL Mean utilization	MEX Patients	MEX Mean utilization
Hospital stay	97%	9 days	100%	11 days	93%	13 days	74%	12 days
ICU stay	69%	9 days	100%	7 days	100%	8 days	53%	6 days
IVIg	52%	5*	51%	5*	27%	5*	52%	5*
PE	75%	5*	24%	5*	74%	6*	12%	5*
VS	50%	4**	57%	4**	100%	4**	51%	5**
OM	97%	2**	72%	1**	96%	2**	64%	2**
Ambulance	39%	1**	37%	1**	73%	2**	57%	1**

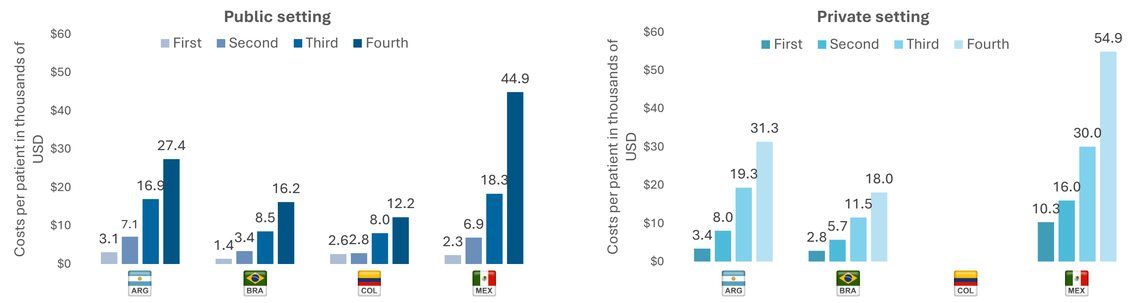
**Courses per treatment cycle; **Units per episode. ICU, Intensive care unit; IVIg, intravenous immunoglobulin as rescue treatment; OM, oxygen monitoring; PE, plasma exchange as rescue treatment; VS, ventilatory support (non-invasive or invasive mechanical ventilation used during hospitalization).*

Estimates of total costs per country per treatment (Million USD)



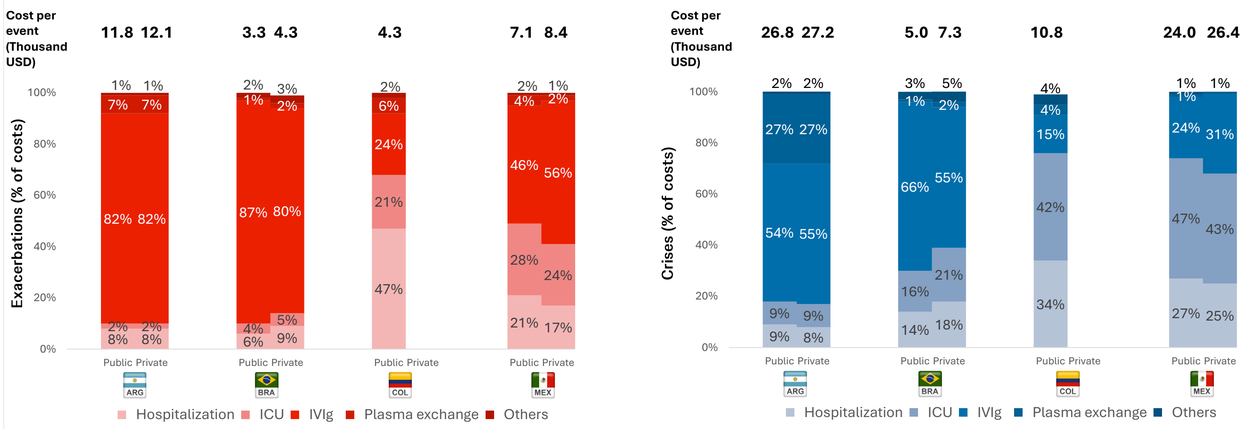
- Total costs (public + private) per country ranged from $10.7 M (Colombia) to $122.7 M (Brazil) per year, and in Argentina, Brazil, and Mexico, is largely represented by costs in the public sector.
- In general, the overall costs were higher in second ($85.4 M) and third ($71.5 M) treatments compared to first ($31.3 M) and fourth or subsequent treatments ($52.7 M).

Distribution of costs per patient across the treatment sequence (Thousands of USD)



- Noteworthy, as patients moved across the treatment sequence, costs per patient increased from 4 to 20 times, depending on the setting and country, mainly due to more exacerbations and crises.

Cost per exacerbation or crisis event



- Costs per exacerbation event and per crises event varied among different countries, with Mexico and Argentina counting for around the double of the cost compared to Brazil and Colombia
- IVIg was the largest cost component for management of exacerbations and crises, followed by PLEX

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