Economic Burden of Gaucher Disease in Colombia

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INTRODUCTION

Gaucher disease (GD) is an orphan disease with heavy burden for patients, causing cytopenia, splenomegaly, hepatomegaly, bone lesions and neuronopathic involvement.

OBJECTIVE

This study aims to estimate the annual economic burden of GD in Colombia from the healthcare system perspective.

METHODS

- Expected cases were calculated based on literature prevalence and compared with the number of diagnosed cases recorded in national registries[1,2,3,4].
- Diagnosed cases were distributed according to disease classification (I, II and III) and treatment status (new diagnosis, first year and two or more years of treatment).
- Cost of care per patient per year including treatment, routinary follow-up and disease-related events, was estimated from HMO's administrative records (RWD) consisting of 3 consecutive years of care for 7 million members, representative for the disease and national population.
- The disease-related events occurrence was obtained from scientific literature and validated with clinical experts[5,6].
- Costs are expressed in 2023 USD$.

POSTER HIGHLIGHT: Despite the low prevalence of the Gaucher disease, its economic impact is significant. The cost of disease-related events is reduced by timely diagnosis and treatment, improving patients' quality of life.

Figure 1: Distribution of GD cases

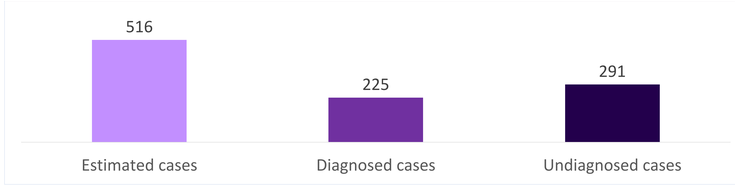

Figure 2. Total costs of GD

Table 1. Total event costs by treatment time

Event	New diagnosis	1st year of treatment	2nd+ years of treatment	Overall reduction
Follow-up	$903.36	$903.36	$903.36	0.00%
Splenectomy	$517.78	$0	$0	100.00%
Necrosis	$399.35	$123.60	$40.32	89.90%
Splenomegaly	$310.41	$232.52	$109.33	64.78%
Hepatomegaly	$255.22	$83.13	$19.35	92.42%
Anemia	$165.14	$41.04	$3.95	97.61%
Others	$633.48	$353.21	$262.73	58.53%
Total	$3,184.73	$1,736.86	$1,339.04	57.95%

RESULTS

- Based on prevalence, 516 patients with GD are estimated of whom 225 are diagnosed. On the other hand, 291 patients remain undiagnosed and hence, have increased probability of developing disease event/complications (Fig. 1).
- For the cost estimation, 81 patients/year were identified in the administrative records database analyzed.
- The annual economic burden for diagnosed patients is $31,968,502, representing 0.21% of the total national Basic Benefits Plan (Fig. 2).
- Adults classified as type I account for 98.23% of these costs (Fig. 2).
- Treatment reduces the cost of event care per patient from $3,185 for the untreated/newly diagnosed patient to $1,737 (45% reduction) in the first year of treatment and $1,340 (58.3% reduction) in the following years (Fig. 3).

CONCLUSIONS

Timely diagnosis and treatment may represent a reduction in the use of resources and costs associated with healthcare. However, a possible under-diagnosis of 56.4% is observed, which implies the economic burden might be higher due to an increased use of health care resources from individuals without treatment.

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