CAPESESP

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BUDGET IMPACT ANALYSIS OF MIGALASTAT INCORPORATION VERSUS ENZYME REPLACEMENT THERAPIES FOR FABRY DISEASE: VALIDATION OF A COST ASSESSMENT TOOL IN BRAZILIAN PRIVATE HEALTHCARE SYSTEM

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BACKGROUND

Fabry disease is a rare X-linked lysosomal storage disorder caused by mutations in the GLA gene, leading to deficient α-galactosidase A activity and accumulation of globotriaosylceramide in multiple organs. The global prevalence is estimated at 1:40,000 to 1:170,000 live births, with an underdiagnosis rate that complicates accurate epidemiological assessment. In Brazil, data from the Ministry of Health and patient registries suggest a prevalence of approximately 0.003% in the population. The clinical picture covers a wide spectrum ranging from mild cases in some heterozygous females, to severe cases in classically affected hemizygous males with no residual alpha-galactosidase A activity. The classical form typically has onset in childhood and may have all the characteristic neurological, cutaneous, renal, cardiovascular, cochleo-vestibular and cerebrovascular signs of the disease. Female patients may have very mild to severe symptoms. Pain is a common early symptom (chronic pain characterized by burning and tingling paresthesia and occasional episodic crises) but may wane in adulthood. Enzyme replacement therapies (ERTs) have been the mainstay treatment for decades, requiring lifelong biweekly infusions, which impose substantial financial costs and affect patient adherence and quality of life. Migalastat, an oral pharmacological chaperone, has emerged as an alternative for patients with amenable GLA mutations, potentially improving adherence and reducing healthcare expenditures.

Fabry disease

- Chronic Pain
- Progressive Genetic Disorder
- Onset in Childhood
- Rare Disease

OBJECTIVES

This study aims to assess the budgetary impact of incorporating migalastat for Fabry disease compared to enzyme replacement therapies (ERTs) over a 5-year horizon in the Brazilian private healthcare system, and to validate a calculator tool designed for cost assessment.

METHODS

A budget impact analysis (BIA) was conducted from the payer´s perspective, incorporating epidemiological data, treatment costs, and Fabry diagnosis stratified by age and GLA mutation eligibility for migalastat. The model used static prevalence and cost for ERT and migalastat. A validated calculator enabled customization of inputs such as prevalence, costs, and adherence was developed and validated through pilot testing. Key assumptions included Fabry prevalence (0.003%) and migalastat eligibility (35%). Migalastat prices considered standard dose and regimen with price proposed by the manufacturer (10% discount), and for available ERTs, the average annual cost based on dose, frequency of use and cost of administration. Annual costs were set at US$158,298 for ERT and US$143,469 for migalastat. Sensitivity analysis was performed.

A validated calculator enabled customization of inputs such as

Prevalence ⟷ Costs ⟷ Adherence

46 million Brazilian private healthcare system

3 per 100.000 Prevalence

35% Eligibility

Annual costs were set at

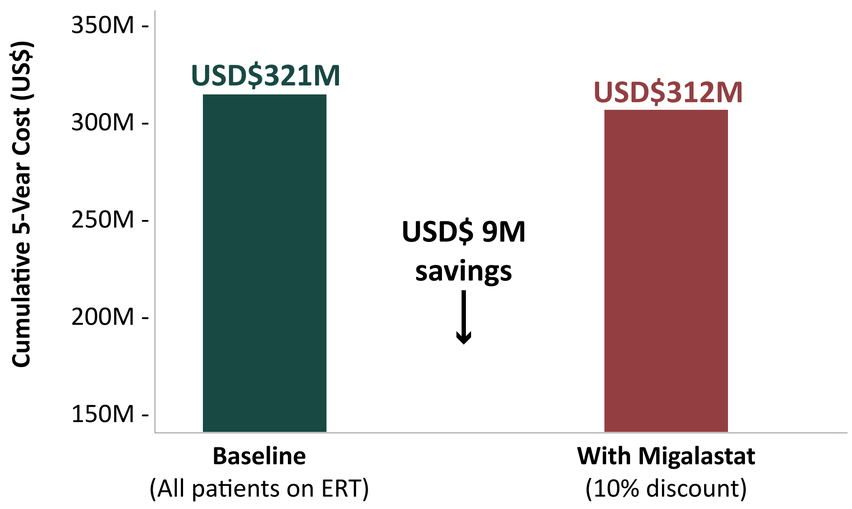
USD$158,298 for Enzyme Replacement Therapies VS **USD$143,469 for Migalastat**

RESULTS

The budget impact model estimated that, within the Brazilian private healthcare system comprising approximately 46 million beneficiaries, around half of Fabry patients would be eligible to transition from enzyme replacement therapy (ERT) to migalastat. Over the 5-year analytic horizon, the patient population was projected to increase marginally due to demographic growth, without significant shifts in epidemiological assumptions. Under the baseline scenario, in which all patients remained on ERT, the cumulative cost of treatment reached USD$321 million. Introducing migalastat at a negotiated price of 10% below the official list resulted in total cumulative expenditures of USD$312 million. This generated a cumulative negative budget impact of USD$9 million, reflecting direct cost savings associated with oral therapy. These findings indicate that migalastat, beyond offering a less invasive oral treatment alternative with potential adherence benefits, may reduce the long-term financial burden on payers. Sensitivity analyses confirmed the robustness of these results, demonstrating that the introduction of migalastat has the potential to be cost saving.

Budget Impact Model Results

For the Brazilian private healthcare system



CONCLUSIONS

Considering that Fabry disease affects 2,5 patients in 100,000 people, it should be remembered that this number could be underestimated. Although enzyme replacement therapies (ERTs) are well-established and widely available, they require lifelong biweekly infusions, which can compromise patient adherence, impact quality of life, and generate high cumulative cost. In addition to the direct financial benefits, the adoption of migalastat, an oral therapeutic alternative, has broader implications for health policy. By reducing infusion-related logistical challenges and enabling an oral regimen, migalastat has the potential to improve patient with Fabry disease, autonomy and reduce indirect costs related to infusion infrastructure, absenteeism, and caregiver burden. These findings reinforce the importance of integrating pharmacoeconomic evaluations into decision-making processes, ensuring access to innovative therapies that align clinical benefit with economic sustainability. This study highlights the importance of BIA models and tools, such as the tested calculator, which can provide transparency and support evidence-based decision-making in health policy for rare disease.

The incorporation of migalastat supports decisions that align clinical benefit with economic sustainability

This study highlights the importance of BIA models and tools

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