

# Cost of Treatment for Mucopolysaccharidosis IV-A in a Poor Population in Colombia

EPH260

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## Introduction

Mucopolysaccharidosis IV-A, also known as Morquio A syndrome, is an autosomal recessive lysosomal hereditary disease caused by the deficiency of the lysosomal hydrolase, N-acetylglucosamine-6-sulfatase (GALNS).

## Objective

The aim of this study was to estimate the cost of treatment for Mucopolysaccharidosis IV-A per patient in a poor population in Colombia.

## Methods

A cross-sectional descriptive study was developed on patients with Morquio A syndrome. Data were extracted from the individual outpatient dispense records of a health insurance company in Colombia. Monthly consumption of drugs during the years 2022-2024 was considered. The mean cost per patient and its confidence interval were estimated. RMR: \$4.090.

## Results

In the period 2022-2024, 6 patients with Morquio A syndrome were identified as medication consumers. Elosulfase Alfa IV was the main and most expensive treatment consumed by these patients. Of the patients diagnosed with the disease, 50% were women with a mean age of  $26 \pm 13$  years, while men had a mean age of  $25 \pm 8$  years. The average cost per patient was \$23,498.75 USD (95% CI: \$22,267-\$24,728) with an average use of 34 vials of 1 mg/ml/5ml.

The economic burden of treating Morquio A syndrome with Elosulfase Alfa IV was approximately \$1,637,411 USD for 2022 and is projected to increase by 2.8% for 2024 (\$1,682,840 USD).

## Conclusions

This study provides strategic information for the planning of health care strategies. Extrapolating the costs to the prevalence of the disease in Colombia could estimate the economic burden and its impact on the Colombian health system.

The use of enzyme replacement therapy (ERT) contributes to cost reduction by minimizing the risk of bone, respiratory, infectious, ear/hearing, cardiovascular, dental, and ophthalmological complications that deteriorate the quality of life of patients.

**Table 1. Estimation of the economic burden for Mucopolysaccharidosis IV-A by usage. 2022-2024.**

Med	Use	Average Cost per Patient	CI 95%
Elosulfase Alfa	Prophylactic use	\$ 23,499	(\$22,267-\$24,728)

Gender	Prop %	Age
Female	50%	$26 \pm 13$
Male	50%	$25 \pm 8$

## Contact information



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