Economic Burden of Pompe Disease in Colombia

Romero M[1], Díaz A[1], Sanchez V[1], Salgado S[2], Chacón C[3], Londono S[3], Upegui A[3]

[1]ProyectaMe, Bogotá, Colombia, [2]Clínica Foscal, Santander, Colombia, [3]Sanofi, Bogotá, Colombia

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INTRODUCTION

Pompe disease (PD) is a disabling orphan disease that affects the contractility of skeletal, smooth, and cardiac muscle leading to hypotonia and muscle weakness.

OBJECTIVE

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

METHODS

- Diagnosed cases were identified from national registries.
- Unidentified cases were estimated using diagnosed cases and a 70% literature under-reporting rate[1,2].
- Diagnosed cases were distributed according to disease classification (infantile onset and late-onset) and treatment status (newly diagnosed, first year and two or more years of treatment)[3,4,5,6].
- 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 [3,4,5,6].
- Costs are expressed in 2023 USD.

POSTER HIGHLIGHT: PD is a disabling disease with a major effect on the patient's quality of life. On-time diagnosis may help to reduce disease complications/events, reducing the total PD economic burden.

Figure 1: Distribution of PD cases

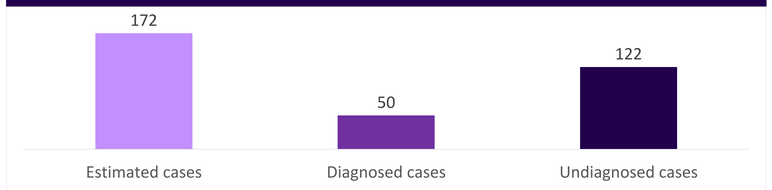

Figure 2. Total costs of PD

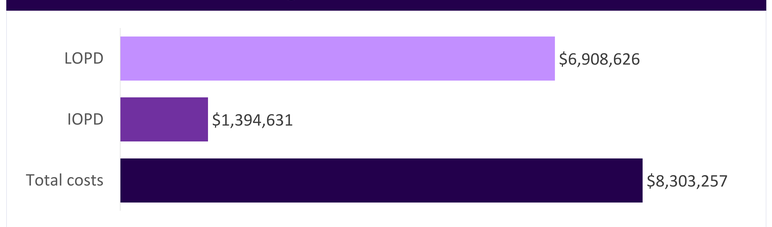

Table 1. Total event costs by treatment time

Event	New diagnosis	1st year of treatment	2nd+ years of treatment	Overall reduction
Muscular	$2,212	$1,673	$1,610	27.20%
Cardiomyopathy	$1,293	$803	$828	35.95%
Follow-up	$1,886	$886	$886	0.00%
Mobility	$779	$784	$720	7.56%
Ventilatory support	$728	$780	$716	1.66%
Nutrition	$604	$200	$260	56.95%
Respiratory	$451	$370	$474	-5.10%
Wheelchair	$170	$180	$166	2.65%
Total	$7,124	$5,677	$5,660	20.54%

RESULTS

- Based on the under-reporting rate, 172 patients with PD are estimated of whom 50 are diagnosed. On the other hand, 122 patients remain undiagnosed and hence, have increased probability of developing disease event/complications (Fig. 1).
- For the cost estimation, 25 patients/year were identified in the administrative records database analyzed.
- The annual economic burden for diagnosed patients is $8,303,257, representing 0.054% of the total national Basic Benefits Plan.
- Adults classified as late-onset account for 83.2% of these costs.
- Treatment reduces the cost of event care per patient from $7,124 for the untreated/newly diagnosed patient to $5,677 (20.3% reduction) in the first year of treatment and $5,660 (20.5% reduction) in the following years.

CONCLUSIONS

The disabling nature of PD is associated with high healthcare costs as the disease progresses, especially for late-onset patients. On time diagnosis and treatment allows for better health outcomes, reducing disease-related events and complications, which results in a lower average cost of event care per patient.

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Author contact information: Angie Upegui – angiegisseth.upeguipachon@sanofi.com
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