

Economic Burden of Fabry Disease in Colombia

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INTRODUCTION

Fabry disease (FD) is a severe multisystemic orphan disease. The clinical spectrum is wide, ranging from a severe phenotype, the classical male, to a mild phenotype in females.

OBJECTIVE

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

METHODS

- Diagnosed cases were identified from national registries.
- Unidentified cases were estimated using diagnosed cases and a 53% literature under-reporting rate.
- Diagnosed cases were distributed according to disease classification (classical and non-classical) and treatment status (newly diagnosed, first year and two or more years of treatment)^{1,2,3,4}.
- 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.

CONCLUSIONS

FD is a highly disabling disease with a major impact on quality of life and healthcare resources. Under-diagnosis may be as high as 70%, increasing healthcare resources for untreated patients. Early diagnosis can improve patients' quality of life and reduce the cost of complications.



POSTER HIGHLIGHT: Given the severe underdiagnosis of Fabry disease, it is critical to develop strategies that improve diagnosis rates, allowing patients to receive timely treatment, and avoiding major events/complications.

Figure 1: Distribution of FD cases

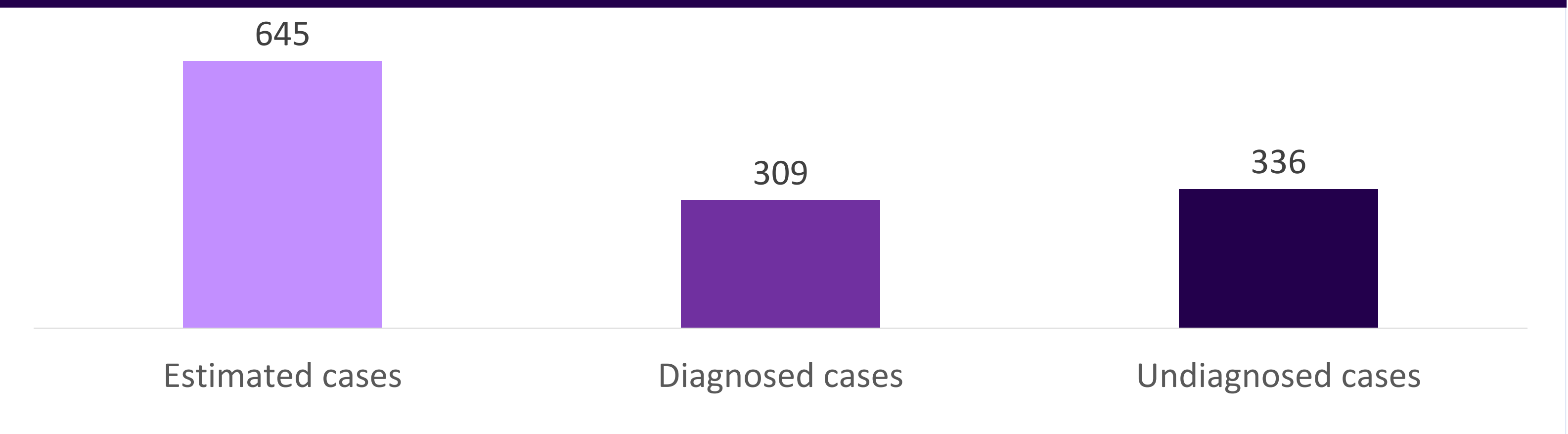


Figure 2. Total costs of FD

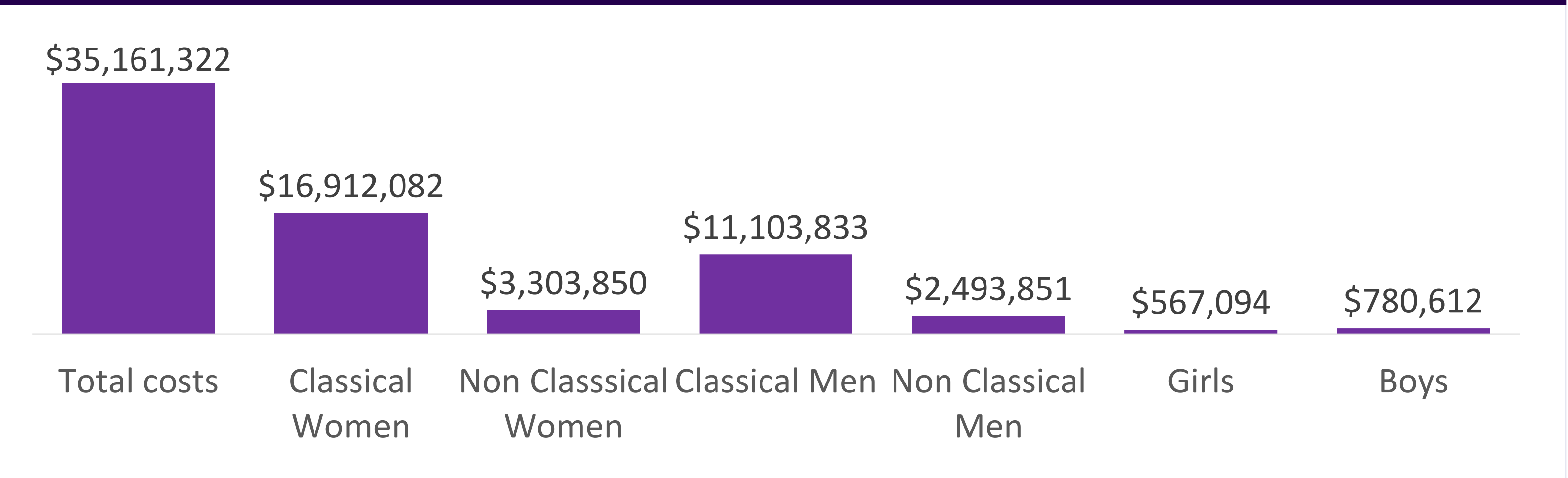


Table 1. Total event costs by treatment time

Event	New diagnosis	1st year of treatment	2nd+ years of treatment	Overall reduction
Cardiac	\$1,371	\$356	\$219	84.00%
Follow-up	\$727	\$727	\$727	0.00%
Kidney	\$369	\$231	\$242	34.40%
Neuropathic pain	\$401	\$158	\$112	72.10%
Others	\$0	\$0	\$0	100.00%
Total	\$2,868	\$1,473	\$1,300	54.66%

RESULTS

- Based on the under-reporting rate, 645 patients with FD are estimated of whom 309 are diagnosed. On the other hand, 336 patients remain undiagnosed and hence, have increased probability of developing disease event/complications (Fig. 1).
- For the cost estimation, 58 patients/year were identified in the administrative records database analyzed.
- The annual economic burden for diagnosed patients is \$35,161,322 representing 0.23% of the total national Basic Benefits Plan (Fig. 2).
- The classical type accounts for 79.68% of costs, of which women account for 60.4%.
- Early diagnosis reduces the cost of event care per patient from \$2,868 for the untreated/newly diagnosed patient to \$1,473 (48.6% reduction) in the first year of treatment and \$1,300 (55.0% reduction) in the following years (Table 1).

REFERENCES

- Germain, D. P., Oliveira, J. P., Bichet, D. G., Yoo, H. W., Hopkin, R. J., Lemay, R., & Warnock, D. G. (2020). Use of a rare disease registry for establishing phenotypic classification of previously unassigned GLA variants: a consensus classification system by a multispecialty Fabry disease genotype–phenotype workgroup. *Journal of Medical Genetics*, 57(8), 542-551 .
- Arends, M., Wanner, C., Hughes, D., Mehta, A., Oder, D., Watkinson, O. T., & Hollak, C. E. (2017). Characterization of classical and nonclassical Fabry disease: a multicenter study. *Journal of the American Society of Nephrology*, 28(5), 1631-1641.
- Martins, A. M., Kyosen, S. O., Garrote, J., Marques, F. M., Guilhem, J. G., Macedo, E., & Ura, S. (2013). Demographic characterization of Brazilian patients enrolled in the Fabry Registry. *Genet Mol Res*, 12(1), 136-142.
- Hopkin, R. J., Bissler, J., Banikazemi, M., Clarke, L., Eng, C. M., Germain, D. P., & Wilcox, W. R. (2008). Characterization of Fabry disease in 352 pediatric patients in the Fabry Registry. *Pediatric research*, 64(5), 550-555.
- Mehta, A., Beck, M., Elliott, P., Giugliani, R., Linhart, A., Sunder-Plassmann, G., & Clarke, J. T. R. (2009). Enzyme replacement therapy with agalsidase alfa in patients with Fabry's disease: an analysis of registry data. *The Lancet*, 374(9706), 1986-1996.
- Rombach, S. M., Smid, B. E., Bouwman, M. G., Linthorst, G. E., Dijkgraaf, M. G., & Hollak, C. E. (2013). Long term enzyme replacement therapy for Fabry disease: effectiveness on kidney, heart, and brain. *Orphanet journal of rare diseases*, 8, 1-9.
- Katsigianni EI, Petrou P. A systematic review of the economic evaluations of Enzyme Replacement Therapy in Lysosomal Storage Diseases. *Cost Effectiveness and Resource Allocation*. 2022;20(1):1–12.

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