COST-EFFECTIVENESS ANALYSIS OF TRANSTHYRETIN-ASSOCIATED AMYLOID CARDIOMYOPATHY FROM THE BRAZILIAN PUBLIC HEALTHCARE SYSTEM PERSPECTIVE

INRODUCTION

Transthyretin-associated amyloid cardiomyopathy (ATTR-CM) is a rare condition, caused by cardiac infiltration of amyloid fibrils, leading to organ dysfunction and, consequently, progressive heart failure¹. The hereditary form of the disease usually manifests itself after 47 years of age, with a median survival of 2.5 years after diagnosis for patients without treatment. The wild form affects almost exclusively older people, with a mean age at diagnosis of 74 years and a median survival from diagnosis in untreated patients of approximately 3.5 years (depending on the stage of the disease)².

OBJECTIVE

Overall, ATTR-CM causes increased hospitalization, reduced quality of life and early death³. In Brazil, tafamidis meglumine is the only drug registered and approved for the treatment of ATTR-CM, being included in national and international clinical guidelines. Therefore, the aim of this analysis is to compare utility and incremental costs between tafamidis and symptomatic treatment.

METHODS

A cost-effectiveness analysis was developed from the perspective of the Brazilian public healthcare system, using a Markov model to follow up patients over 60 years old, with ATTR-CM in functional classes II or III in a lifetime time horizon, considering the transition through 3 different health states (alive without heart transplant, alive and heart transplanted, or death). The outcomes evaluated were life years gained (LY) and quality-adjusted life years gained (QALY). A model was considered that the drug price was reduced year by year, over a five-year horizon.

The transition diagram of the cohort simulation model is shown in the Figure 1 bellow.



Figure 1. Schematic representation of the model used in the costeffectiveness analysis of tafamidis meglumine in the treatment of wild and hereditary TTR-associated amyloid cardiomyopathy (ATTR-CM).

addition, it was conducted sensitivity analysis In (deterministic and probabilistic) in order to evaluate the impact of the variation of some parameters on the results. a variation of +/-20% was considered for all variables included in the deterministic analysis, except for utility data. In this specific case, the utility was varied according to data obtained from a Brazilian study^{4,5}. The probabilistic analysis calculated 1,000 iterations of Monte de Carlo, with a probability distribution of gamma for costs and beta for the other parameters.

RCEI/AVAQ of BRL 473.4 thousand (table 1).

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RESULTS

The results showed that tafamidis meglumine provides significant gains in effectiveness (LY = 3.28 and AVAQ = 2.55) when compared to the symptomatic treatment, with the need for incremental costs, resulting in an incremental costeffectiveness ratio (ICER) per LY of BRL 369.1 thousand and

Table 1. Result of the cost-effectiveness analysis of tafamidis meglumine in the treatment of ATTR-CM in the lifetime horizon, per patient from the brazilian public health care system

	Tafamidis	symptomatic	
	meglumine	treatment	III
Total	1,220,718.42	11,668.41	1,
Treatment (BRL)	1.,07,621.08	0,00	1,
Transplant (BRL)	2.23	1.03	
Hospitalization (BRL)	4,450.08	3,069.82	
Adverse events management (BRL)	12.29	13.96	
Follow-up (BRL)	2,805.53	1,442.52	
Life years gained	5.86	2.59	
ICER (/ LY) (BRL)			3
Quality-adjusted life	5.86	2.59	
years (QALY)			
ICER (/ QALY) (BRL)			4

LY: life years; QALY: quality-adjusted life years gained

According to the deterministic sensitivity analysis, the parameters with the greatest impact on the results of the economic evaluation were the utility values according to the patient's NYHA classification. However, none of these parameters was able to significantly change the results (Figure 2).





Figure 2. Deterministic sensitivity analysis tornado diagram for the QALY outcome.

cremental



69,124.83

3.28

473,457.61

The probabilistic sensitivity analysis results are presented in the incremental cost-effectiveness plan (Figure 3), where 100% of the iterations remained in guadrant I (higher cost and greater effectiveness).



Figure 3. Cost-effectiveness plan for the QALY outcome.

CONCLUSIONS

The clinical evidence and the result of the cost-effectiveness showed that tafamidis provides gains for patients with ATTR-CM. It's incorporation in the Brazilian public healthcare would be extreme important to patients, because it is the only specific treatment available for ATTR-CM.

References

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