

ECONOMIC BENEFIT AND CLINICAL ADVANTAGES WITH THE INCLUSION OF PATIENTS IN CLINICAL TRIALS RELATED TO PARAMYLOIDOSIS

D. PINTO¹, A. OLIVEIRA², A. FONTES², D. MONTEIRO², M. CRUZ², T. CUNHA², P. BARBEITA², A. MATOS², P. ROCHA³.

¹PHARMACY RESIDENT, UNIDADE LOCAL DE SAÚDE DE SANTO ANTÓNIO, PORTO, PORTUGAL.

²PHARMACIST, UNIDADE LOCAL DE SAÚDE DE SANTO ANTÓNIO, PORTO, PORTUGAL.

³CHIEF PHARMACIST, UNIDADE LOCAL DE SAÚDE DE SANTO ANTÓNIO, PORTO, PORTUGAL.

CLINICAL TRIALS, PARAMYLOIDOSIS, ECONOMY

BACKGROUND AND IMPORTANCE

Access to innovative medicines requires extensive and careful pharmacoeconomic evaluation.

The inclusion of patients in Clinical Trials (CT) allows early access to new experimental medicines and considerable economic saving for the healthcare system.

AIM AND OBJECTIVES

Evaluate the economic benefit of including patients with hereditary transthyretin amyloidosis (hATTR) in clinical trials between 2018 and 2023.

MATERIAL AND METHODS

Retrospective analysis of paramyloidosis-related clinical trials taking place at the center since 2018. The data collected were the number of paramyloidosis-related CT, the number of patients included, the time of participation in the CT and the average price of conventional treatment.

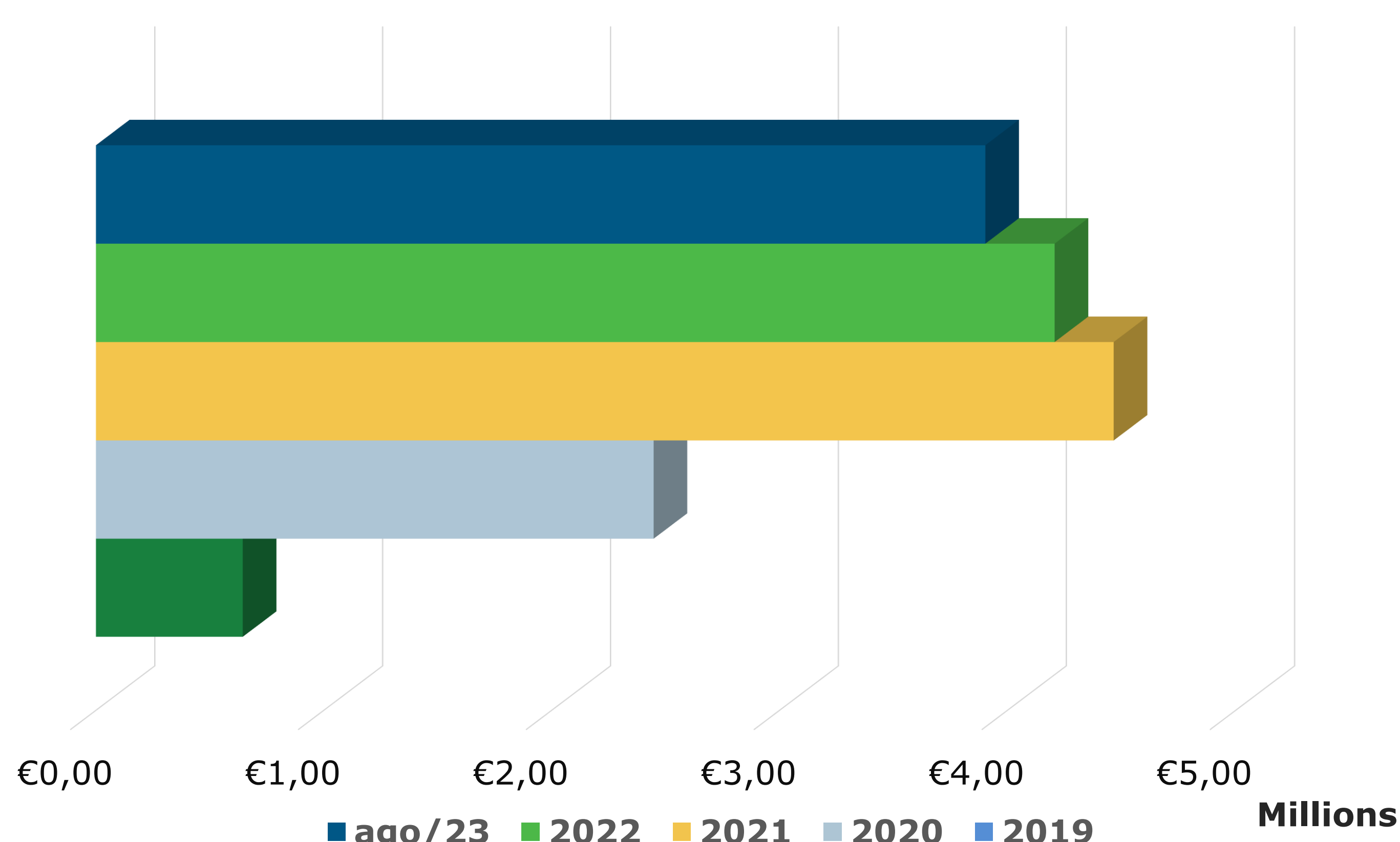
RESULTS

At our Clinical Trials Unit there are currently 6 Paramyloidosis-related CT underway, involving a total of 65 patients.

In economic terms, patient participation on ongoing CT related to Paramyloidosis has led to a cumulative saving of **15,667,487.98€**, compared to the costs of conventional therapy (tafamidis [1], inotersen [2] and patisiran [3]).

The distribution of annual savings was:

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With the approval of new therapies for the treatment of paramyloidosis, an increase in costs is expected to ensure access to therapeutic innovation.

Therefore, with the emergence of new therapies, the cost borne by the healthcare system undergoes an exponential increase. Participation in clinical trials fills these costs, while also ensure the access to experimental drugs.

CONCLUSION AND RELEVANCE

Participation in CT allows early access to new experimental therapies and contributes to the development of new drugs and/or new therapeutic indications. In Paramyloidosis, new agents like TTR stabilizers, subcutaneous antisense oligonucleotides and iRNA therapies are potential new alternatives [4].

By participating in CT, centers obtain an extra source of funding. The participation of patients in CT also allows for a reduction in costs, through the preservation of financial resources and medication.

The savings generated by the participation in CT help to provide better care and an efficiency healthcare system.

REFERENCES AND/OR ACKNOWLEDGEMENTS

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[4] Gertz, M. A., Mauermann, M. L., Grogan, M., & Coelho, T. (2019). Advances in the treatment of hereditary transthyretin amyloidosis: A review. *Brain and Behavior*, 9(9). <https://doi.org/10.1002/brb3.1371>

