

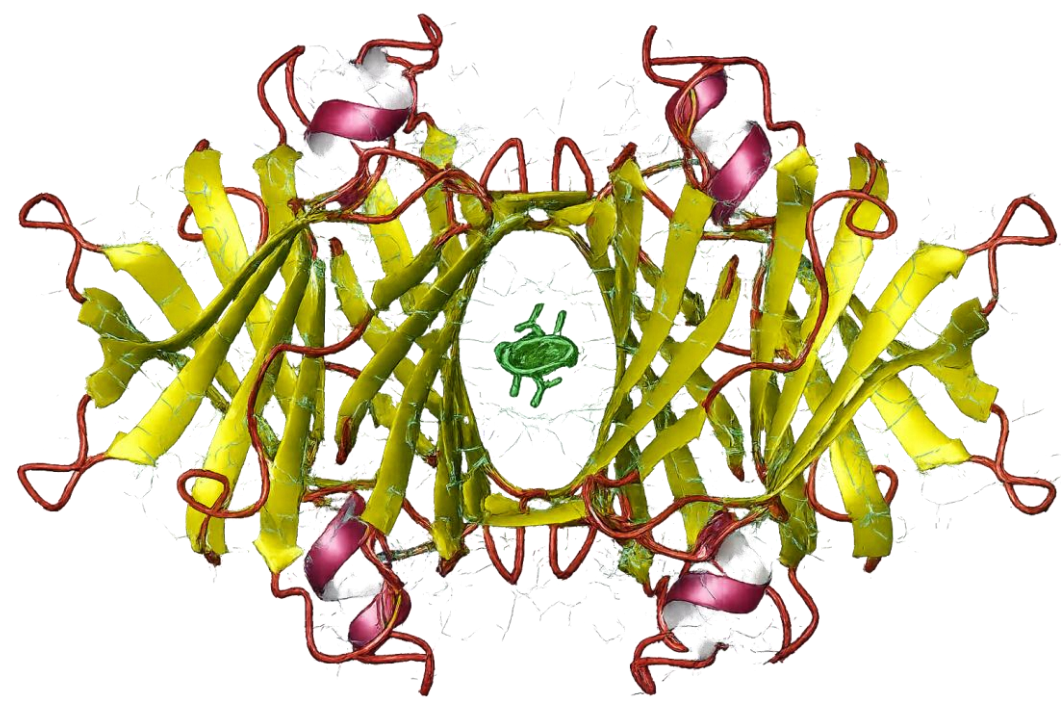
Avoided drug cost derived from participation in clinical trials on transthyretin amyloidosis

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Background



Avoided cost (AC) is an objectively quantifiable economic impact for clinical trials



Transthyretin amyloidosis is associated with high pharmaceutical expenditure due to lifelong treatment with tafamidis

Aim and objectives



To quantify the **avoided drug cost** associated with the participation of patients in clinical trials on transthyretin amyloidosis,

Assess the **real economic impact** of transthyretin amyloidosis clinical trials

Study design:

Retrospective observational study:
2019-2024



Trial characteristics:

Sponsor-provided drugs free of charge

Material and Methods

Inclusion criteria:

Active trials
Marketed medicines
Sponsor-provided drugs

Data source:

Institutional clinical trial management software
Pharmacy database

Cost calculations:

AC was calculated assuming patients would have received the **same treatment outside the trial**



Two clinical trials were conducted:

Only one clinical trial met inclusion criteria:

Phase III
International
Multicentre

Results

€ 9,710,268 total AC

Total avoided drug cost (2019–2024)

€1.6 million annual saving

72 patients

€134,865 AC per patient



Conclusion

The participation of hospitals and patients in clinical trials contributes significantly to **reducing direct drug costs** associated with transthyretin amyloidosis.

Facilitates early access to innovative therapies for patients

