Budget Impact of Adding Acoramidis to a United States Health Plan Formulary for the Treatment of Transthyretin Amyloid Cardiomyopathy

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INTRODUCTION

- ATTR-CM is a rare, chronic, progressive, and ultimately fatal disease caused by destabilization of the TTR protein[1]
- DMTs for ATTR-CM act by stabilizing the TTR protein tetramer to prevent its dissociation (tafamidis, approved by the US Food and Drug Administration in 2019,[2] and acoramidis, approved in 2024[3]; TTR stabilizers) or by reducing TTR expression in the liver (vutrisiran[4]; TTR silencer)
- Treatment with acoramidis leads to rapid, sustained, durable, and near-complete (≥90%) TTR stabilization[5,6]
  - In the phase 3 ATTRibute-CM trial and open-label extension, continuous acoramidis had a significant, clinically meaningful reduction in the risk of all-cause mortality or first CVH compared to placebo/delayed acoramidis over 54 months, with benefits observed as early as 3 months[7,8]
- Given the potential for costly disease-related events such as CVH and the growing number of novel therapies, US healthcare payers need to understand the budgetary impact of treatment utilization to inform access decisions

OBJECTIVE

- To estimate the financial impact of adding acoramidis to a US health plan formulary for the treatment of ATTR-CM using a BIM

METHODS

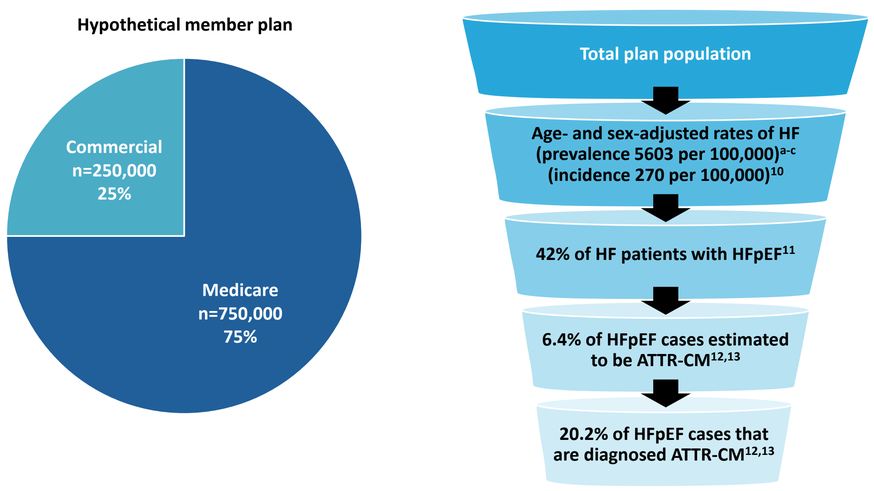
Study Design and Time Horizon

- A BIM was developed in Microsoft Excel® following guidance from the International Society for Pharmacoeconomics and Outcomes Research[9]
- A "Reference Case" (acoramidis off-formulary for the treatment of ATTR-CM) was compared with a "New Case" (acoramidis on-formulary, with increased uptake over a 3-year time horizon), with a start year of 2026

Model Description

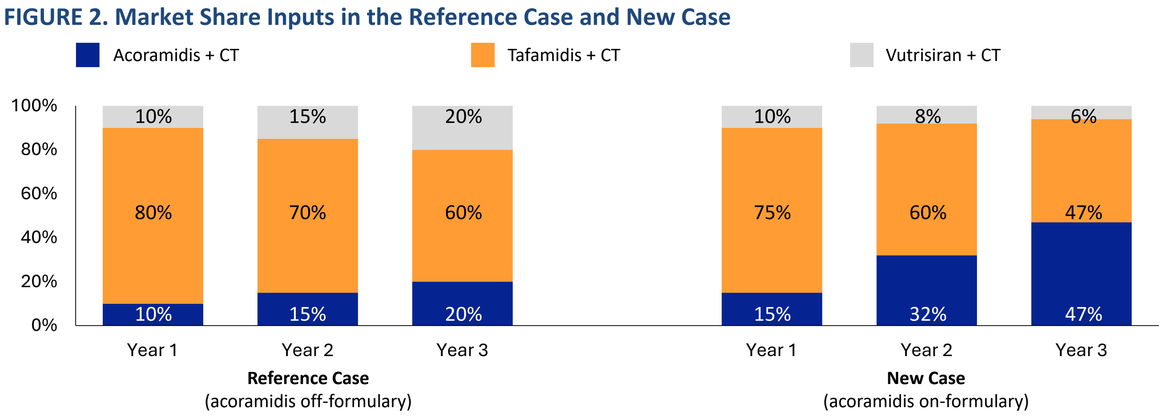
- The model assumed a US third-party healthcare payer perspective with a hypothetical 1-million-member plan, comprised of 75% Medicare beneficiaries (assumed to be ≥65 years of age) and 25% commercially insured members (**Figure 1**)

FIGURE 1. Patient Population and Epidemiology Funnel[10-13]



  - The target population included adults diagnosed with ATTR-CM who received monotherapy (ie, DMT) for ATTR-CM in conjunction with conventional therapy for the management of heart failure
- Due to the prevalence and incidence of ATTR-CM being poorly understood,[16] we utilized a funnel approach to calculate the DMT-eligible population (**Figure 1**)
  - The funnel applied increasingly restrictive criteria to filter down from patients with HF to patients diagnosed with ATTR-CM
  - In Year 1, the model includes prevalent patients with ATTR-CM treated with DMT. In subsequent years, the model adds newly diagnosed incident patients to the prior-year total
- Deaths are accounted for in each year of the model, and costs are no longer incurred after patient death
- Since tafamidis entered the market first and held the entire market share, it was assumed to be the dominant DMT at the start of the time horizon (**Figure 2**)

FIGURE 2. Market Share Inputs in the Reference Case and New Case



- Treatment effectiveness was modeled using rates of CVH and mortality (**Table 1**)
- Costs related to treatment acquisition, CVH, terminal care, and management of AEs were considered (**Table 2**)

TABLE 1. Clinical Inputs

Model parameter[a]	Base case value
CVH	
Annualized rate for CT only[17]	0.45 events/year
DMT vs CT only, HR	
Acoramidis + CT[17]	0.50
Tafamidis + CT[18]	0.71
Vutrisiran + CT[19]	0.68
All-cause mortality	
Annualized rate for CT only[17]	0.112 events/year
DMT vs CT only, HR	
Acoramidis + CT[17]	0.77
Tafamidis + CT[18]	0.70
Vutrisiran + CT[19]	0.71

TABLE 2. Cost Inputs

Model parameter	Base case value
Annual treatment regimen costs, commercial[a]	
CT only	$5,211
Acoramidis + CT	$263,188
Tafamidis + CT	$291,865
Vutrisiran + CT	$482,615
Annual treatment regimen costs, Medicare[a]	
CT only	$1,027
Acoramidis + CT	$155,813
Tafamidis + CT	$173,019
Vutrisiran + CT	$469,181
CVH costs[20]	
Cost per hospitalization, commercial	$49,319
Cost per hospitalization, Medicare	$37,239
Terminal care costs[21]	
Cost of final month of care, commercial	$34,458
Cost of final month of care, Medicare	$34,458
Annual AE cost, by treatment	
Acoramidis + CT	$5,621
Tafamidis + CT	$6,127
Vutrisiran + CT	$3,254

Model Outputs

- Model outputs included the size of the eligible treatment population, annual CVH, total plan costs, and costs stratified by source (drug acquisition, AEs, CVH, and terminal care costs)
- The budget impact of increased acoramidis uptake was calculated as the difference in budget between the New Case and Reference Case (overall model results)
- Results were reported annually and cumulatively over the 3-year time horizon as total budget impact, percentage change in total, PMPM, and PTMPM costs

Scenario Analyses

- A series of scenario analyses were conducted to understand how the overall model results were impacted under alternative inputs (**Table 3**)

TABLE 3. Scenario Analysis Description

Scenario	Base case value	Scenario value
Perspective	Lives in plan are split; 75% Medicare and 25% commercial	100% Medicare
		100% commercial
Clinical inputs	Use unadjusted HRs anchored to placebo rate from ATTRibute-CM trial	Use matching-adjusted indirect treatment comparison (MAIC) hazard and risk ratios for tafamidis[23]
	Acoramidis displaces tafamidis	Acoramidis displaces vutrisiran + tafamidis combination therapy

CONCLUSIONS

- **This BIM projected that formulary adoption of acoramidis within a hypothetical 1-million-member US health plan was associated with projected savings of $21.95 million over the 3-year time horizon, including in scenarios with "pure" payer perspectives (ie, fully Medicare or fully commercial), MAIC-derived inputs, and acoramidis displacement of combination DMT treatment**
- **Savings were driven by reduced drug costs, with fewer CVHs providing additional cost offsets**
- **Clinical benefits associated with acoramidis may translate into meaningful economic value for US health plan payers**

RESULTS

Population Estimates

- The model estimated there would be 306 prevalent cases of ATTR-CM requiring DMT in Year 1, 316 total cases in Year 2, and 326 total cases in Year 3

Overall Budget Impacts

- Adding acoramidis to the formulary resulted in projected savings of $21.95 million over the 3-year time horizon (**Figure 3**)
  - Expressed in standardized terms, the use of acoramidis is expected to result in savings of $0.61 PMPM and $1,917 PTMPM

FIGURE 3. Budget Impact of Acoramidis in the Reference Case and New Case

Total Budget Impact of Acoramidis Over 3 Years

Total cost outcomes (Years 1-3)	Reference Case (acoramidis off-formulary)	New Case (acoramidis on-formulary)	Overall model results (incremental cost)
Total	$248,560,736	$226,607,813	−$21,952,922
Drug acquisition costs	$227,966,349	$206,404,445	−$21,561,904
CVH costs	$12,618,893	$12,079,258	−$539,635
AE costs	$5,325,135	$5,436,805	$111,670
Terminal care costs	$2,650,358	$2,687,305	$36,946

Adding acoramidis to formulary in a hypothetical 1-million-member plan resulted in:

$21.95 million savings over 3 years
$0.61 PMPM
$1,917 PTMPM

13 CVHs avoided
Savings of $539,635 in CVH costs

- Savings were largely attributable to reductions in drug acquisition costs (−$21.6 million) and to fewer CVHs (−$539,635)
  - Over 3 years, 13 CVHs were avoided

Scenario Analyses

- Across all scenarios evaluated, the direction of the budget impact remained directionally unchanged relative to the overall model results, with increased acoramidis uptake consistently associated with reductions in total healthcare expenditures (**Table 4**)
  - Notably, results were consistent when MAIC-derived inputs[23] were used in place of unadjusted estimates

TABLE 4. Scenario Analysis Results

Scenario value	Total budget impact over 3 years	% Change vs overall model results	PMPM impact	% Change vs overall model results
Overall base case results	−$21,952,922	N/A	−$0.61	N/A
100% Medicare	−$26,539,432	−20.9%	−$0.74	−20.9%
100% commercial	−$10,344,020	52.9%	−$0.29	52.9%
Use MAIC HRs for tafamidis[23]	−$21,772,136	0.8%	−$0.60	0.8%
Acoramidis displaces vutrisiran + tafamidis combination therapy	−$54,088,006	−146.4%	−$1.50	−146.4%

LIMITATIONS

- Limitations of the analysis pertain primarily to data availability and the assumptions required for model inputs
- To date, no head-to-head trial directly comparing outcomes of tafamidis and acoramidis has been conducted
- Total cost estimates hinged on market share and acoramidis uptake, which can be difficult to predict
  - Drug costs were based on WAC and assumed full adherence with no discontinuation; actual payer net prices may differ
  - However, as long as the uptake of acoramidis in the New Case exceeds that of the Reference Case, the use of acoramidis would be expected to drive cost savings

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NOTES: FIGURE 1. [a]Assumes that all diagnosed patients receive disease-modifying therapy. [b]The HF prevalence figure accounts for the population's age and sex distribution, and age- and sex-specific HF prevalence estimates. [c]Calculated based on age- and sex-specific prevalence estimates, and the anticipated age and sex distribution of the plan per NHANES data,[14] as reported in Tsao et al.[15]
TABLE 2. [a]Base case assumptions using unadjusted hazard ratios from the phase 3 ATTRibute-CM,[17] ATTR-ACT,[18] and HELIOS-B[19] studies.
TABLE 3. [a]Treatment acquisition costs applied in the model were considered annually. Patients were assumed to be fully adherent with no treatment discontinuation. The unit costs of drug acquisition were based on wholesale acquisition cost estimates obtained from Micromedex REDBOOK.[22] Treatment doses were based on US prescribing information.[6]
ABBREVIATIONS: *AE* adverse event; *ATTR-CM* transthyretin amyloid cardiomyopathy; *BIM* budget impact model; *CVH* cardiovascular hospitalization; *CT* conventional therapy; *DMT*, disease-modifying therapy; *HF* heart failure; *HFpEF* heart failure with preserved ejection fraction; *HR* hazard ratio; *MAIC* matching-adjusted indirect comparison; *NHANES* National Health and Nutrition Examination Survey; *N/A* not applicable; *PMPM* per member per month; *PTMPM* per treated member per month; *TTR* transthyretin; *US* United States; *WAC* wholesale acquisition cost.
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