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Real-World Clinical, Safety and Economic Outcomes of Acoramidis Under an Early Access Program: A Pharmacist-Led Evaluation from a Portuguese NHS Reference Centre

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BACKGROUND & OBJECTIVES

The acoramidis Early Access Program (EAP) was approved in Portugal for the treatment of wild-type or variant transthyretin amyloid cardiomyopathy (ATTR-CM) in adult patients with disease progression despite tafamidis or with intolerance or contraindication to this therapy. This study aimed to:

1. Characterise the eligible population and access pathway at the Portuguese centre with the highest EAP enrolment
2. Evaluate early real-world clinical effectiveness and safety
3. Quantify drug acquisition cost avoidance for the National Health Service (NHS)
4. Assess adherence and persistence under pharmacist-led follow-up

METHODS

Retrospective, single-centre, observational study including all patients initiating acoramidis under the national EAP between June 2025 and January 2026.

Data source
Electronic health records and outpatient pharmacy dispensing system

Variables (at baseline and most recent follow-up)
- NYHA class
- NT-proBNP
- Estimated Glomerular Filtration Rate (eGFR)

Pharmacist-led care
Structured pharmacovigilance drug dispensing visits

Adherence, persistence and cost avoidance
- Proportion of Days Covered (PDC)
- Continuous treatment until progression, death or transition to reimbursed access
- Drug cost approved after reimbursement and duration of EAP

RESULTS

PATIENT CHARACTERISTICS

7 patients initiated acoramidis
5 male
Median age **83 years**
All with **wild-type ATTR-CM**

EAP ELIGIBILITY CRITERIA



6 patients
Tafamidis contraindication due to severe eGFR reduction

1 patient
Disease progression despite tafamidis

FOLLOW-UP

Median follow-up **6 months**

NYHA CLASS

Improved in **6 patients**
Remained stable in **1 patient**

NT-proBNP

Changes were heterogeneous, likely reflecting renal decline and short follow-up.

EFFICACY AND SAFETY

No cardiovascular hospitalisations or deaths occurred.

One non-serious adverse event resolved without discontinuation.

ADHERENCE AND PERSISTENCE



Median PDC **100%**
(range 91 - 100%)



Persistence **100%**

NHS DRUG COST AVOIDANCE

€188,446
Total cost avoidance

Mean €26,921
per patient

CONCLUSIONS

1. In this EAP cohort, representing the largest national experience, acoramidis showed favourable functional outcomes and manageable safety profile in a predominantly frail, impaired population otherwise ineligible for tafamidis.
2. The EAP generated substantial cost avoidance while enabling timely access to treatment prior to reimbursement, highlighting its dual role in facilitating access and generating early real-world evidence.
3. Pharmacist-led follow-up proved feasible for active pharmacovigilance, adherence monitoring and continuity of care.

Keywords: Early Access Program; Real-World Evidence; Pharmacist-Led Care; Pharmacovigilance