Access, Adherence, Outcomes: A Comprehensive Real-World Data Analysis of Tafamidis

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Background

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is a progressive disease caused by a deposition of misfolded transthyretin in cardiac tissue, leading to restrictive cardiomyopathy and heart failure
- Tafamidis is a first-in-class TTR stabilizer approved for treating hereditary or wild-type ATTR-CM
- The pivotal ATTR-ACT trial demonstrated that tafamidis reduced all-cause mortality and the number of annual cardiovascular-related hospitalizations compared to placebo[1]
- Despite clinical benefit, non-adherence and barriers to medication access in these patients can increase cardiac events and increase healthcare utilization including hospitalizations.

Objective

To evaluate medication access, adherence, and clinical outcomes among patients receiving tafamidis through an integrated health-system specialty pharmacy (HSSP) model

Methods

Retrospective chart review of patients who initiated tafamidis therapy in the 2025 calendar year

Specialty pharmacy liaisons conducted benefits investigations and referred patients to the health system prior authorization (PA) team for PA assistance

Patients reporting financial hardship were referred to the medication assistance team for additional support

The primary outcome was the time from provider signature to PA approval.

Secondary outcomes included medication adherence, same day PA approvals, discontinuation rates, and the number of hospitalizations observed within the cohort.

Results

Figure 1: Patient Demographics

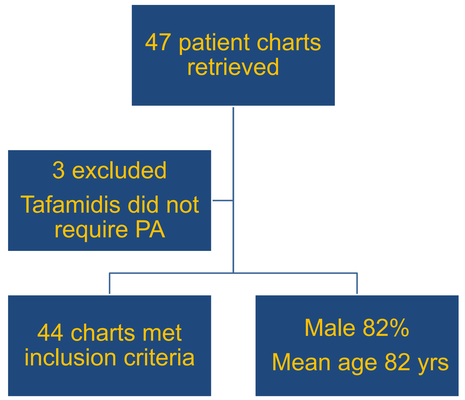


Figure 2: Cumulative PA Approval Times

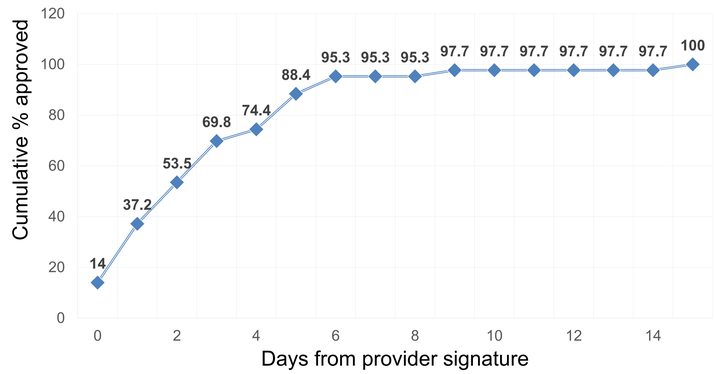


Figure 3: Median Days Between Tafamidis Order Date and First Dispense Date[2,3]

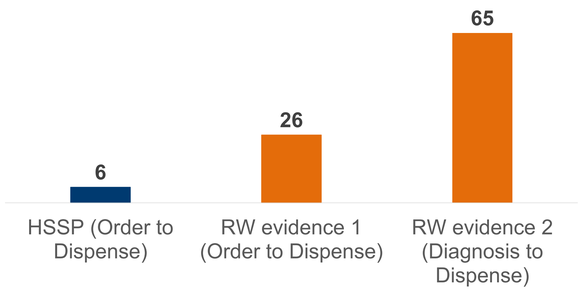
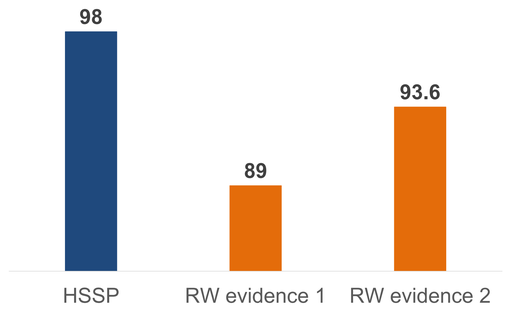
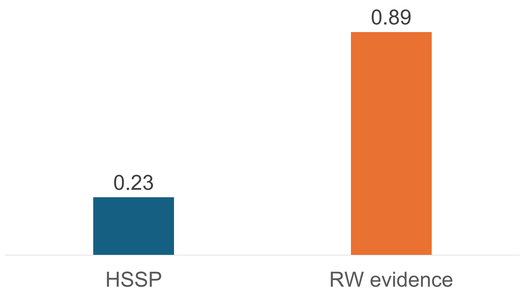


Figure 4: Mean PDC (%)[4,5]



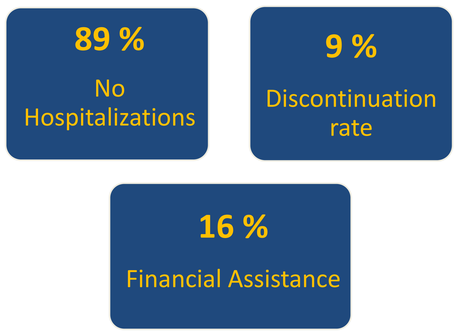
PDC = Proportion of Days' Covered

Figure 5: Mean Annual Frequency of CV-Related Hospitalizations[6]



*Average number of hospitalizations per patient over study period was 0.16

Figure 6: Secondary Outcomes n=44



RW = Real World

Discussion

- Favorable prior authorization approval timelines, medication adherence and cardiac related hospitalization outcomes among tafamidis treated patients relative to published studies were observed in this cohort.
- The high adherence (98%) observed in this cohort exceeded both the real-world adherence benchmarks (89% and 93.6%) and the industry standard of 85%.
- The low annual CV-related hospitalizations may reflect the downstream benefit of earlier treatment initiation in these patients.
- These results suggest an integrated specialty pharmacy model with a dedicated PA team, and specialty clinical pharmacist led education and reassessments along with proactive financial assistance coordination may meaningfully reduce barriers to tafamidis access and initiation.

Limitations

- This analysis is from a single academic health system cohort which may limit generalizability to community or non-integrated specialty pharmacies.
- Differences in disease severity, comorbidity, socioeconomic factors and insurance types were not controlled for and may affect adherence and hospitalization outcomes

Conclusion

- The integrated health system specialty model facilitated timely prior authorization approvals. Patients serviced by the HSSP exhibited high medication persistence and sustained therapy continuation.

References

1. Maurer MS, Schwartz JH, Gundapaneni B, Elliott PM, Merlini G, Waddington-Cruz M, Kristen AV, Grogan M, Witteles R, Damy T, Drachman BM, Shah SJ, Hanna M, Judge DP, Barsdorf AI, Huber P, Patterson TA, Riley S, Schumacher J, Stewart M, Sultan MB, Rapezzi C; ATTR-ACT Study Investigators. Tafamidis Treatment for Patients with Transthyretin Amyloid Cardiomyopathy. N Engl J Med. 2018 Sep 13;379(11):1007-1016. doi: 10.1056/NEJMoa1805689. Epub 2018 Aug 27. PMID: 30145929.
2. Masri A, Chen H, Wong C, Fischer KL, Karam C, Gellad WF, Heitner SB. Initial Experience Prescribing Commercial Tafamidis, the Most Expensive Cardiac Medication in History. JAMA Cardiol. 2020 Sep 1;5(9):1066-1067. doi: 10.1001/jamacardio.2020.1738. PMID: 32936265; PMCID: PMC7301295.
3. Miller P, Elias P, Einstein AJ, Maurer MS, Ahmed GY, Poterucha TJ. Socioeconomic Disparities Are Associated With Delayed Access to Tafamidis in Transthyretin Cardiac Amyloidosis. Circ Heart Fail. 2024 Dec;17(12):e012075. doi: 10.1161/CIRCHEARTFAILURE.124.012075. Epub 2024 Nov 7. PMID: 39508097; PMCID: PMC11652229.
4. Attal S, Kemner J, Alvir J, Barth S, Schuessler S. Tafamidis 61 mg Patient Characteristics and Persistency? A Retrospective Analysis of German Statutory Health Insurance Data (IQVIA™ LRx). Cardiol Ther. 2024 Jun;13(2):369-378. doi: 10.1007/s40119-024-00365-6. Epub 2024 Apr 13. PMID: 38615093; PMCID: PMC11093959.
5. Kato T, Ines M, Minamisawa M, Benjumea D, Keohane D, Alvir J, Kim R, Chen Y, Peixoto T, Kent M, Wogen J, Ishii T, Crowley A, Sugino T, Izumiya Y. Tafamidis medication adherence and persistence in patients with transthyretin amyloid cardiomyopathy in Japan. ESC Heart Fail. 2024 Oct;11(5):2881-2888. doi: 10.1002/ehf2.14736. Epub 2024 May 23. PMID: 38783561; PMCID: PMC11424365.
6. Rozenbaum MH, Tran D, Bhambri R, Nativi-Nicolau J. Annual Cardiovascular-Related Hospitalization Days Avoided with Tafamidis in Patients with Transthyretin Amyloid Cardiomyopathy. Am J Cardiovasc Drugs. 2022 Jul;22(4):445-450. doi: 10.1007/s40256-022-00526-9. Epub 2022 Mar 30. PMID: 35353352; PMCID: PMC9270297.

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