Assessing the Impact of Specialty Pharmacists on Access and Adherence in ATTR Cardiomyopathy: A Quality Improvement Initiative

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BACKGROUND

- Transthyretin amyloid cardiomyopathy (ATTR-CM) is an increasingly recognized cause of heart failure with expanding disease-modifying treatment options, including TTR stabilizers and silencers.
- Access to these therapies is often challenged by complex prior authorization requirements, high costs, and specialty pharmacy coordination.
- As a URAC-designated Rare Disease Center of Excellence, MUSC Specialty Pharmacy developed specialized processes to facilitate therapy access and track patient outcomes.

OBJECTIVES

- The objective of this project is to evaluate the impact of specialty pharmacist involvement on access to TTR stabilizer therapies.

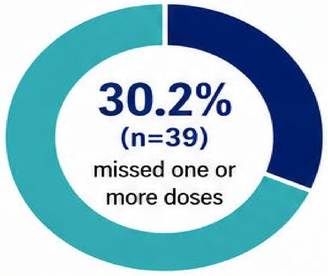
METHODS

- **Study design:** single-center, retrospective descriptive study evaluated data from Jan. 2025 to May 2026
- **Study sample**: ATTR-CM patients (N = 129) referred to the MSUC SP for TTR stabilizer therapy.
- **Outcome:**
  - **Prescription turnaround time** was defined by the interval between the date a prescription was received and the date the first shipment was scheduled
  - **Medication adherence** was reflected by the percentage of patient reported missing one or more doses of therapy
  - **Patient financial burden** was measured by the total amount of financial assistance utilized toward therapy copayment
- **Statistic Analysis**: descriptive statistics were used to analyze the data for this study

RESULTS

129 patients received transthyretin (TTR) stabilizer therapy through the MUSC Specialty Pharmacy during the study period.

MEDICATION ADHERENCE



Overall, **30.2%** (n=39) of patients reported missing one or more doses of therapy.

- Missed one or more doses (30.2%)
- No missed doses (69.8%)

BY DOSING FREQUENCY

TWICE-DAILY
45.9%
(17 of 37)
missed one or more doses

ONCE-DAILY
21.9%
(23 of 105)
missed one or more doses

FINANCIAL ASSISTANCE AND ACCESS

98.4%
(127 of 129)
of patients required financial assistance for treatment copayments.

- Manufacturer Copay Programs
- Third-Party Copay Assistance Foundations
- Internal Copay Assistance

A total of
$263,724.72
in copay savings was provided to eligible patients.

PHARMACY EFFICIENCY

The average prescription turnaround time for TTR stabilizer therapy was
2.01 days

PATIENT EXPERIENCE

100%
(129 of 129)
of patients reported improved quality of life and high satisfaction with the MUSC Specialty Pharmacy Rare Disease Management Program.

KEY TAKEAWAY

1. These data provide real-world evidence that dosing frequency should be weighed alongside efficacy when selecting TTR stabilizers, particularly for patients at risk of non-adherence.
2. Integrated financial navigation is not optional but essential — without copay support, treatment abandonment is a near-certainty for most patients given the cost of these therapies.
3. Specialty pharmacy programs with rapid turnaround and proactive adherence monitoring may help close the gap between clinical trial efficacy and real-world effectiveness in ATTR1.

DISCUSSION

- The higher rate of missed doses among patients on twice-daily TTR stabilizer therapy compared to once-daily therapy highlights dosing frequency as a modifiable barrier to adherence, which is critical given that these therapies slow but do not reverse disease progression.
- The near-universal need for financial assistance and over $263,000 in copay savings provided underscore that TTR stabilizer therapies are functionally inaccessible for most patients without integrated financial navigation
- A dedicated specialty pharmacy model achieved rapid prescription turnaround and 100% patient-reported satisfaction, demonstrating that centralized, multidisciplinary rare disease programs can effectively bridge the gap between drug availability and real-world treatment access.

CONCLUSION

- A dedicated specialty pharmacy rare disease management program can meaningfully support patients receiving TTR stabilizer therapy by addressing key barriers to treatment success.
- The reliance on financial assistance highlights an urgent need for systemic solutions to reduce the cost burden of these life-sustaining therapies, and the integrated financial navigation assistance through specialty pharmacy is essential to gaining access of therapy for patients.
- Future studies will evaluate the long-term clinical impact on hospitalization rates, disease progression, and medication adherence