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Background

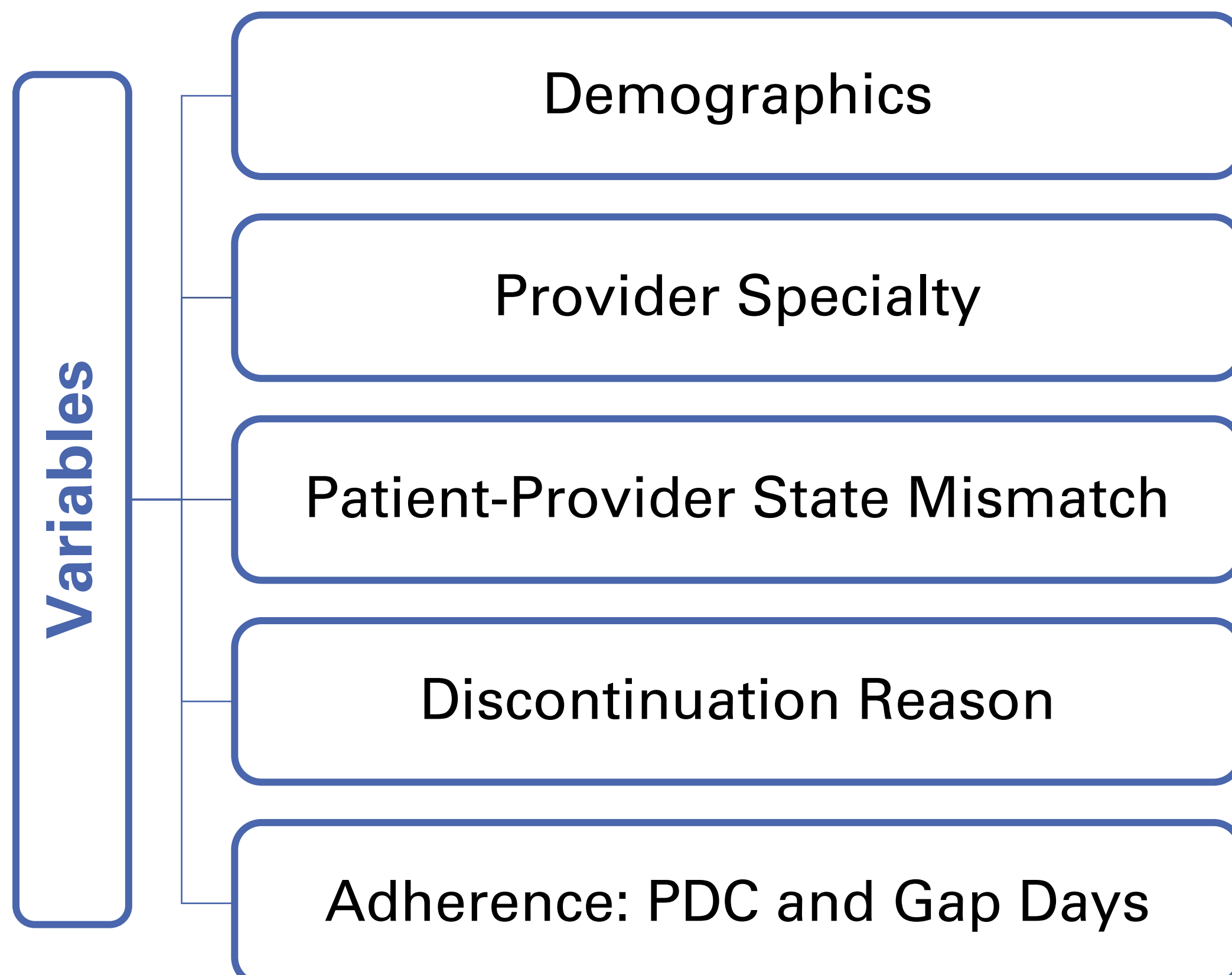
- Wilson disease (WD) is a rare, autosomal recessive disorder of copper metabolism leading to copper accumulation that primarily affects hepatic, neurologic or psychiatric systems¹
- Low adherence is associated with progressive disease and worse outcomes^{2, 3}
- Trientine tetrahydrochloride (TETA-4HCl, Cuvrior[®]) was FDA-approved in 2022 and launched commercially in April 2023
- TETA-4HCl is distributed directly to the patient by a single-source rare disease specialty pharmacy

Objective

To assess TETA-4HCl adherence as measured by proportion of days covered (PDC) and gap days

Methods

Inclusion Criteria: patients on therapy for ≥3 months from May 2023 (pharmacy launch) to December 2024



Data Source:

- Single source pharmacy database

Adherence Cohorts:

- 6 - <12 months on therapy
- ≥12 months on therapy

Figure 1: Study Population

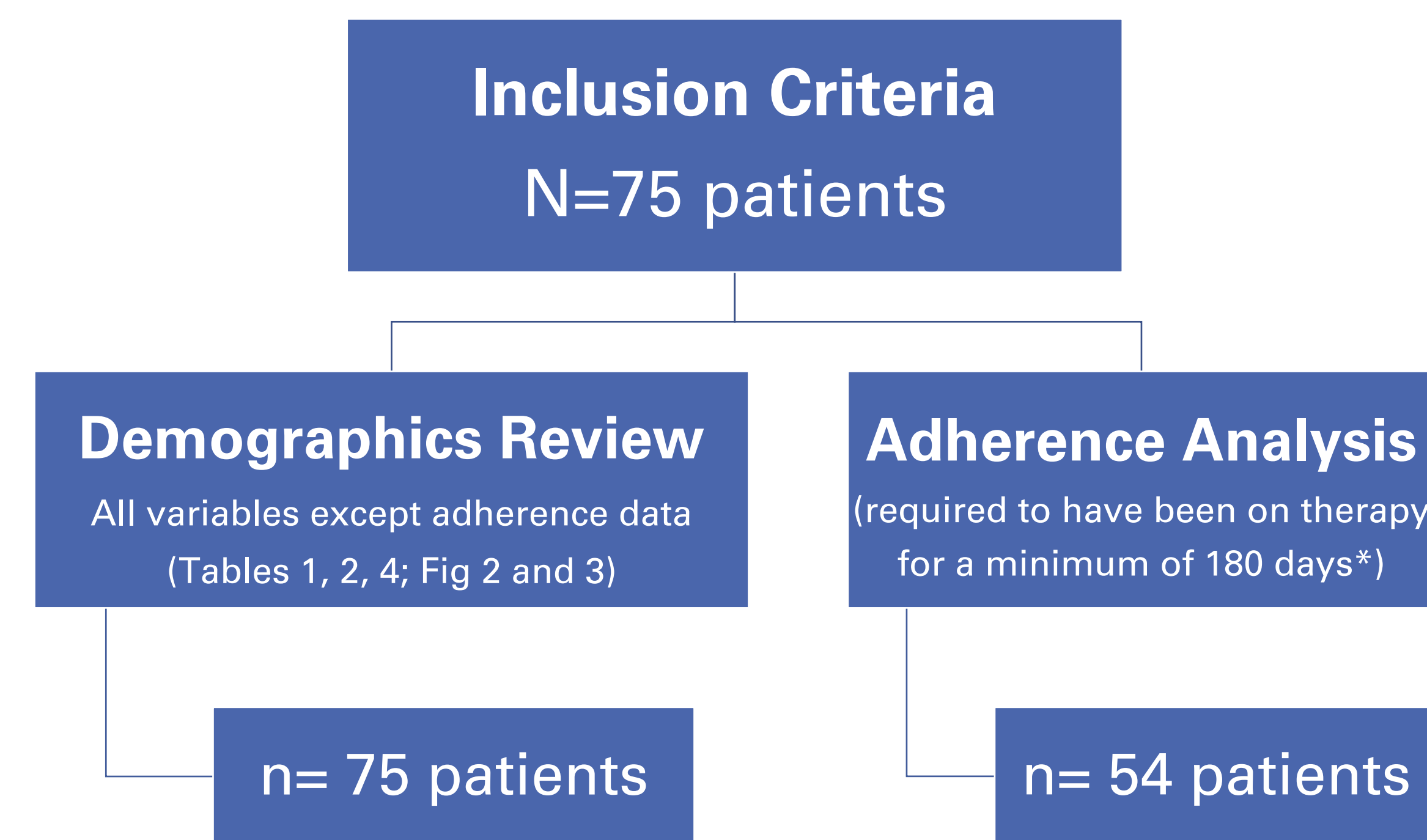


Table 1: Patient Demographics

Demographics	n (%)	Average Age (years)
Female	35 (47%)	35.4
Male	40 (53%)	37.2

Table 2: Provider Specialty

Hepatology: 49
Gastroenterology: 16
Neurology: 6
Other: 4

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Results

Figure 2: Patient Location Heat Map

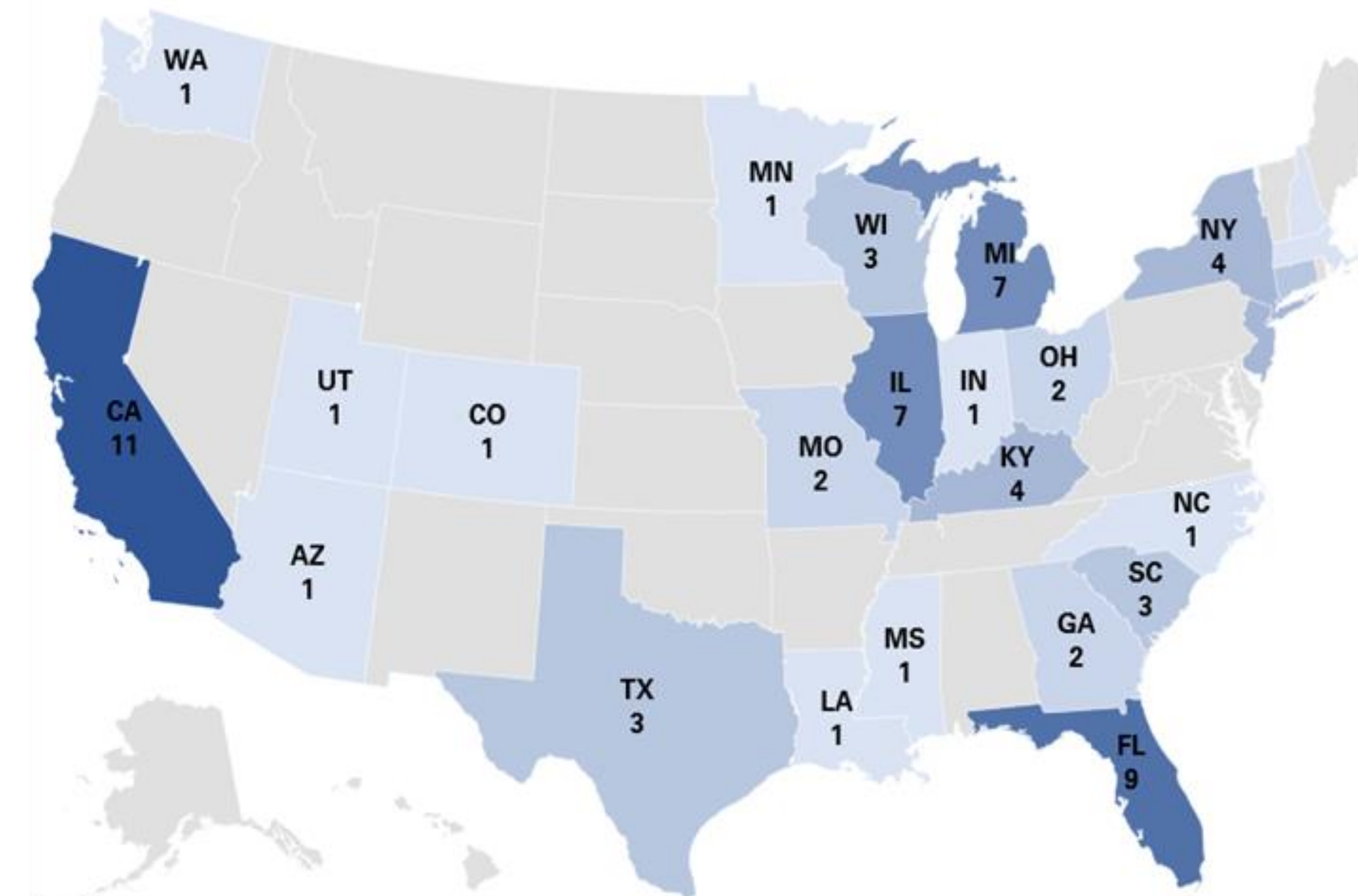


Figure 3: Patient-Provider State Mismatch
 Patient residence relative to provider office

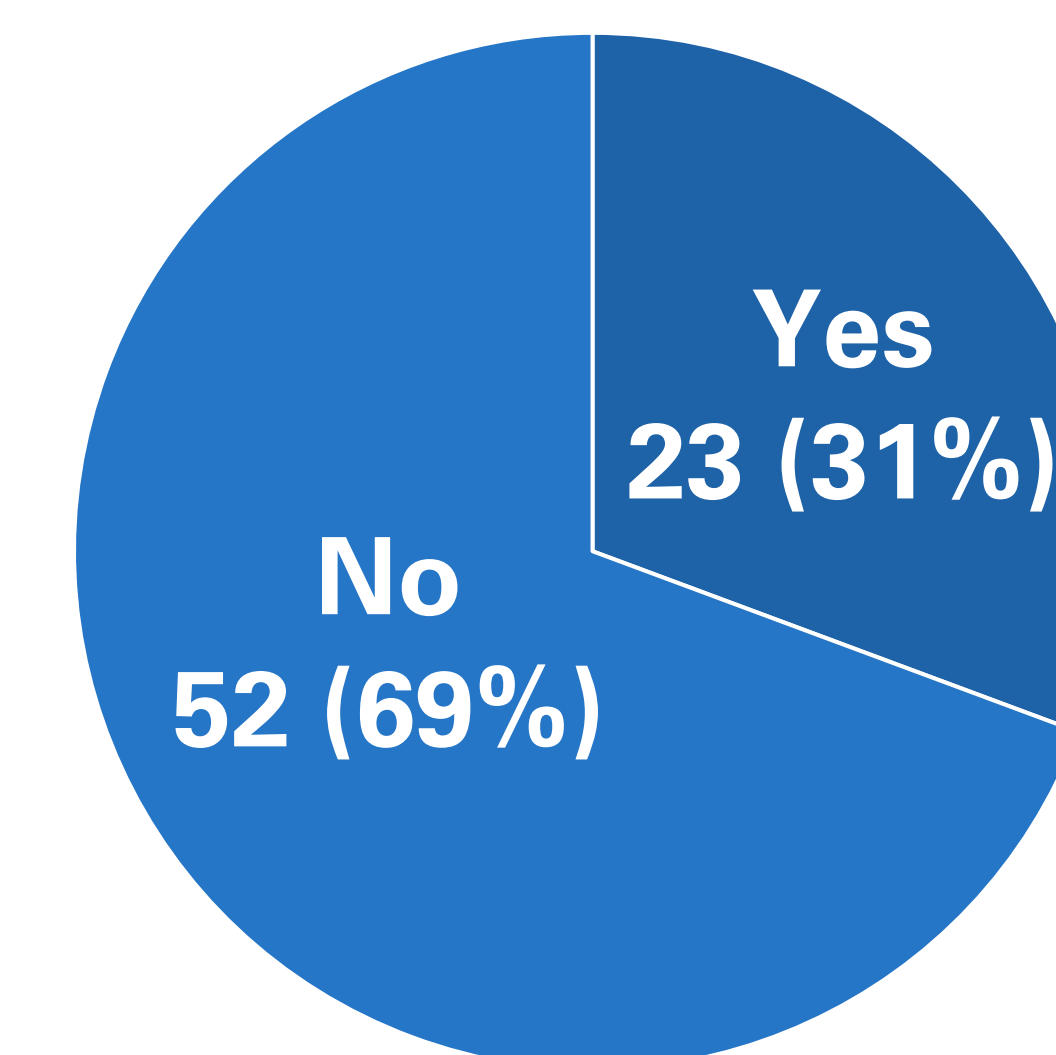


Table 3: Adherence Measures by Cohort

Adherence Variable	6 - <12 months on therapy (n= 22)	≥12 months on therapy (n= 32)
Average PDC	91%	87%
Average PDC ≥ 80%	82%	75%
Average fill count	10	16
Average gap days per shipment	2.0	2.8

PDC: proportion of days a patient has medication on hand over a given time; minimum industry standard is 180 days*

Gap days: length of days that a patient does not have medication on hand based on shipment history

Calculation: [number of days with drug on hand] / [number of days in a specified period]

Calculation: [medication shipment date] – [medication exhaust date]

Table 4: Discontinuations

6 patients discontinued overall	
3 patient/provider unreachable	3 due to provider order
No discontinuations due to an adverse event	

Conclusion

- TETA-4HCl is prescribed by predominantly hepatologists/gastroenterologists with ~1/3 of these scripts from practitioners in a different state which may be challenging for continued access to life-long medications
- Receiving TETA-4HCL from a single-source specialty pharmacy, demonstrate (i) favorable rates of adherence in first year and after 12 months as measured by PDC and gap days and (ii) a low discontinuation rate
- Simplifying access to treatment may have a positive impact on patient outcomes, and warrants further study

References

1. Ala A, Walker AP, Ashkan K, Dooley JS, Schilsky ML. Wilson's disease. Lancet. 2007
 2. Jacquelet E, Beretti J, De-Tassigny A, et al. Compliance with treatment in Wilson's disease: On the interest of a multidisciplinary closer follow-up. Rev Med Interne. 2018
 3. Treibich C, Lescher S, Sagaon-Teyssier L, Ventelou B. The expected and unexpected benefits of dispensing the exact number of pills. PLoS One. 2017