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Real-world evaluation of off-label fampridine use in cerebellar ataxia: safety and effectiveness in a tertiary hospital

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Background and importance

Cerebellar ataxias are a heterogeneous group of neurological disorders characterized by impaired coordination and balance. Pharmacological options are limited. Fampridine, a potassium channel blocker approved for gait improvement in multiple sclerosis, is being used off-label in ataxia despite limited clinical evidence. Real-world data are needed to clarify its therapeutic value and safety profile.

Aim and objectives

To evaluate the real-world effectiveness and safety of off-label fampridine in patients with cerebellar ataxia, describe their baseline characteristics, and identify potential predictors of treatment response.

Material and methods

A retrospective observational study included 30 patients with different forms of cerebellar ataxia who initiated off-label fampridine. Inclusion criteria were estimated glomerular filtration rate (eGFR) >50 mL/min and prescription made between October 2021 and October 2025, following internal committee approval. Follow-up mean time was 14 ± 12 months. Effectiveness was assessed using the Timed 25-Foot Walk Test (T25-FWT) at baseline and after 2 weeks of treatment. Safety outcomes included treatment discontinuation rate, duration of therapy, and adverse drug reactions (ADRs).

CHARACTERISTICS	N= 30
Age	64 +- 12,15 (SD)
Sex (male)	19 (63%)
Type of ataxia	
- Idiopathic	11 (36,7%)
- CANVAS (RFC1 mutation)	5 (16,7%)
- SCA27B (FGF14 expansion)	9 (30%)
- Episodic type 2	1 (3,3%)
- MSA-cerebellar	1 (3,3%)
- SPG7-related paraparesis	3 (10%)
Alcohol consumption	9(30%)
Nystagmus	20 (66,67%)

Table 1. Baseline characteristics

Results

Among 30 patients (mean age 64 ± 12 years; 63% male), 16 (53.3%) showed improvement in T25-FWT at 2-weeks, with a mean reduction of 2.4 seconds (range –0.7 to –9.0). Treatment was discontinued in 21 patients (70%), with a median (IQR) duration of 90 (30-271) days. ADRs occurred in 50% of patients, mainly insomnia, gastrointestinal discomfort, and dizziness.

CONCLUSION AND RELEVANCE

Fampridine may provide functional benefits in cerebellar ataxia, but its real-world use is limited by high discontinuation rates and frequent ADRs. Close monitoring and pharmacy-led review of off-label prescriptions are essential to ensure safe and effective therapy. Further controlled studies are needed to confirm these findings.