

# Real-world effectiveness and safety of fenfluramine in pediatric patients with refractory epileptic syndromes

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## Background

Dravet syndrome (DS) and Lennox-Gastaut syndrome (LG) are severe developmental and epileptic encephalopathies with limited therapeutic options. Fenfluramine has shown efficacy in DS and LGS, but evidence in other epileptic syndromes is limited.

## Aims

To evaluate the **real-world effectiveness** and **safety** of fenfluramine in pediatric patients with refractory epileptic syndromes.

## Material and methods

**Design:** Retrospective, single-center, observational.

**Population:** Patients with treated with fenfluramine



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**Data collected:** demographic, clinical and treatment variables



**Effectiveness:** seizure frequency and duration at 14-week follow-up

Adverse drug reactions recorded

Predictors of response explored with logistic regression

## Results

**N = 45**

62,5% male

Age: 8 years (IQR 5–11)

Dravet: 26,7%

Lennox-Gastaut: 31,1%

Other encephalopathies: **42,2%**



**51,1%** patients had more than 10 crisis per day

**Concomitant cannabidiol 29%**

## Effectivity

**55,6%**

Clinical response

**40%**

Non-DS/LG responders

**40%**

Previously treated with cannabidiol responders

**24,4%**

Discontinuations

72,7% due to lack of effectivity  
9,1% due to ADRs



## Fenfluramine dose

At initiation  $0,44 \pm 0.15$  mg/kg/day

At 14-weeks  $0.50 \pm 0.21$ mg/kg/day

## Safety

### Adverse Drug Reactions (ADRs)

**28.9%**  
Somnolence

**24.4%**  
Anorexia

**8.9%**  
Diarrhoea

**4.4%**  
Abdominal Pain

12 patients reported ADRs

**No significant predictors of response identified** (age, diagnosis, cannabidiol co-treatment)

## Conclusions

Fenfluramine appears **effective** and **safe** in the treatment of Dravet syndrome and Lennox–Gastaut syndrome and shows **promising results in other difficult-to-treat epileptic syndromes**. No predictors of response were identified, highlighting the need for larger multicenter studies to better define patient profiles and optimize therapy.

