

Non-Seizure Outcomes and Caregiver Burden in Patients With Dravet Syndrome and Lennox-Gastaut Syndrome Treated With Fenfluramine: Design of a Prospective, Multicentre, Non-Interventional Study

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OVERVIEW

QUESTION

How does fenfluramine treatment impact sleep disturbances, behaviour, activities of daily living (ADL), Clinical Global Impression-Improvement (CGI-I), and caregiver burden in patients with Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) during an ongoing real-world study (EP0239)?

STUDY OUTCOMES

Primary outcomes

Patient and caregiver sleep disturbances

- Change from baseline to month 6 in sleep outcomes assessed by:
 - PROMIS Paediatric Sleep Health Scale (patients)
 - PROMIS Adult Sleep Health Scale (caregivers)

Patient outcomes are proxy-reported by the caregivers. ADL, activities of daily living; BPM, Brief Problem Monitor; CBCL, Child Behaviour Checklist; CGI-I, Clinical Global Impression-Improvement; FROM, Family-Reported Outcome Measure; PedsQL, Paediatric Quality of Life Inventory; PROMIS, Patient-Reported Outcomes Measurement Information System.

CONCLUSIONS

- By prospectively evaluating sleep disturbances, behaviour, ADL, CGI-I, and caregiver burden in patients with DS and LGS, this ongoing study aims to provide real-world evidence on the holistic impact of fenfluramine treatment, including effects on seizure control and other relevant manifestations of developmental and epileptic encephalopathies.

INVESTIGATION

- Observational, multinational, prospective cohort study with a 6-month follow-up period.
- Enrolling ~60 patients with DS or LGS who have recently initiated fenfluramine, together with their primary adult caregivers.

Secondary outcomes

Patient's overall improvement

- CGI-I at month 6

Caregiver burden

- Monthly scores and change from baseline to month 6 on the FROM-16 scale

Patient and caregiver characteristics

- Demographics
- Patient clinical and treatment characteristics at baseline and month 6

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Background

- Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) are rare, severe developmental and epileptic encephalopathies (DEE) characterised by treatment-resistant seizures, neurodevelopmental delay, and substantial cognitive and behavioural impairments.^{2,3}
- Fenfluramine is approved for the treatment of seizures in patients (≥2 years of age) with DS and LGS in the European Union, United States, and many other countries worldwide.^{4,5}
- Poor sleep quality is common in children and adults with DS or LGS, and is a major determinant of health-related quality of life (HRQoL).^{6,7}
 - Sleep disturbances can have a substantial impact on the wellbeing and coping capacity of caregivers and the wider family.⁶
 - Epilepsy and sleep are closely interconnected, with seizures disrupting sleep and sleep deprivation potentially worsening seizure burden.⁸
- As the burden of DS and LGS is not limited by seizures and extends to other manifestations, treatment evaluation should encompass sleep disturbances, behaviour, HRQoL, and other DEE-related outcomes.

Objective

- To describe the design of an ongoing, prospective, multicentre, non-interventional study (EP0239) evaluating sleep disturbances, behaviour, and caregiver burden in patients with DS or LGS newly initiating fenfluramine treatment.

Methods

STUDY DESIGN

- EP0239/RWE0944 (REec; 0012-2026-OBS) is a multinational, prospective, observational cohort study enrolling patients (≥2 years of age) diagnosed with DS or LGS who have recently initiated fenfluramine, and their non-professional adult caregivers.
- Fenfluramine is prescribed according to standard local clinical practice.
 - All treatment decisions are made independently of the study protocol.
- Data are collected using standardised questionnaires completed by the same primary caregiver at baseline and throughout the 6-month follow-up period.
- There are no protocol-mandated site visits; informed consent may be obtained in person or remotely, and data are collected electronically.

PATIENT AND CAREGIVER ELIGIBILITY

Inclusion criteria

Patient

- ✓ Informed consent/assent
- ✓ Diagnosis of DS or LGS
- ✓ ≥2 years of age at fenfluramine initiation
- ✓ Decision to initiate fenfluramine made independently and prior to study enrolment
- ✓ New user of fenfluramine, with treatment initiated within 2 weeks after study enrolment

Caregiver

- ✓ Primary, non-professional adult caregiver (≥18 years of age) for the patient with DS/LGS
- ✓ Resides in the same household as the patient and is available to collect data during nighttime hours
- ✓ Willing and able to read, understand, and respond to study questions in local language at specified observation points

Exclusion criteria

Patient

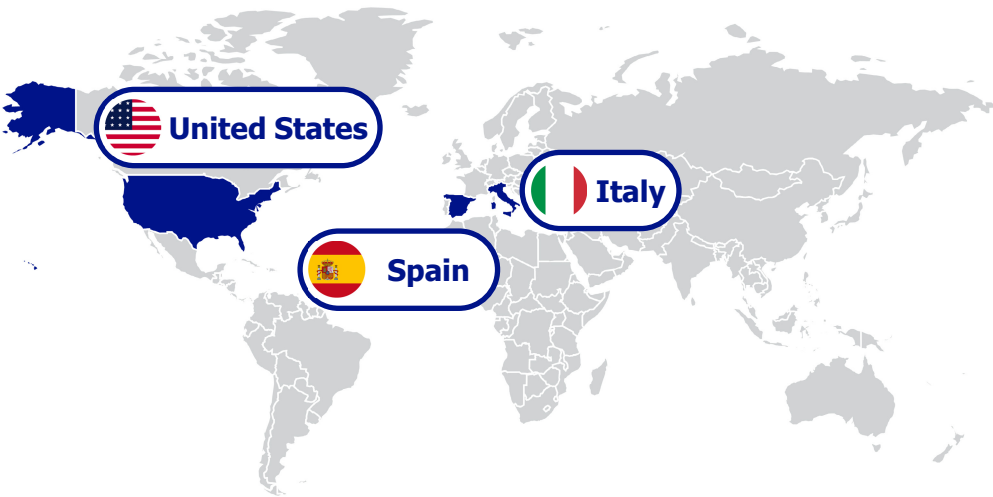
- ✗ Participation in an interventional study at enrolment

ENROLMENT

- Approximately 60 patient-caregiver dyads (~30 patients with DS and ~30 patients with LGS) are planned for enrolment.
- To minimise selection bias, sites are requested to systematically invite consecutive eligible patients newly initiating fenfluramine to participate in the study.

Planned results

PLANNED STUDY LOCATIONS



- Recruitment is currently ongoing at 30 sites.

ENDPOINTS

Primary and secondary outcomes

Objectives	Outcomes
Primary	
To describe patient and caregiver sleep disturbances	
Patients	Caregiver-reported monthly PROMIS Paediatric Sleep Health Scale scores, and change from baseline to month 6
Caregivers	Monthly PROMIS Adult Sleep Health Scale scores, and change from baseline to month 6
Secondary	
To describe the proportions of patients and caregivers reaching a minimum meaningful change improvement in sleep outcomes	
Patients	Change from baseline to month 6 in total PROMIS T-score of ≥5 ¹
Caregivers	Change from baseline to month 6 in total PROMIS T-score of ≥5 ¹
To describe patients' scores on instruments measuring behaviour and activities of daily living (caregiver-reported)	
Behaviour and emotional problems	Achenbach CBCL (1.5-5 years): total and subdomain scores at baseline and months 2, 4, and 6, and change from baseline to month 6 Achenbach CBCL (6-18 years): total score and subdomain scores at baseline and month 6, and change from baseline to month 6
Attentional, behavioural, and internalising problems	BPM (6-18 years): total score and subdomain scores at baseline and months 2, 4, and 6, and change from baseline to month 6
Physical, emotional, social, and school functioning	PedsQL Generic Core Scale: monthly total and domain scores and change from baseline to month 6
To assess patients' improvement relative to fenfluramine initiation	Caregiver-reported CGI-I at month 6
To characterise patients and caregivers at baseline and follow-up	Demographics, and patient clinical and treatment characteristics
To assess caregiver burden	Monthly scores and change from baseline to month 6 on the FROM-16 scale

BPM, Brief Problem Monitor; CBCL, Child Behaviour Checklist; CGI-I, Clinical Global Impression-Improvement; FROM, Family-Reported Outcome Measure; PedsQL, Paediatric Quality of Life Inventory; PROMIS, Patient-Reported Outcomes Measurement Information System.

CONCLUSIONS

- By systematically evaluating sleep disturbances and behaviour alongside caregiver burden, this ongoing study aims to provide prospective evidence on the impact of fenfluramine on a range of DEE manifestations in the real-world setting.

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