Final Results From a Long-Term Open-Label Extension Study (Up to 4 Years): Tolerability of Fenfluramine and Global Functioning of Pediatric and Adult Patients With Dravet or Lennox-Gastaut Syndromes

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Introduction

- Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS) are rare, lifelong developmental and epileptic encephalopathies characterized by high seizure burden and cognitive and behavioral impairments[1–3]
- Fenfluramine differs from other antiseizure medications (ASMs) in its mode of action, as it targets the serotonergic (5-HT) system and the sigma-1 receptor[4] and is also associated with minimal risk for CYP450-related drug-drug interactions[5–7]
- Fenfluramine is currently approved in the US for the management of seizures associated with DS and LGS in patients ≥2 years old[8]; its safety and efficacy has been demonstrated in randomized controlled trials (RCTs)[9–12] and open-label extension (OLE) studies[13,14]

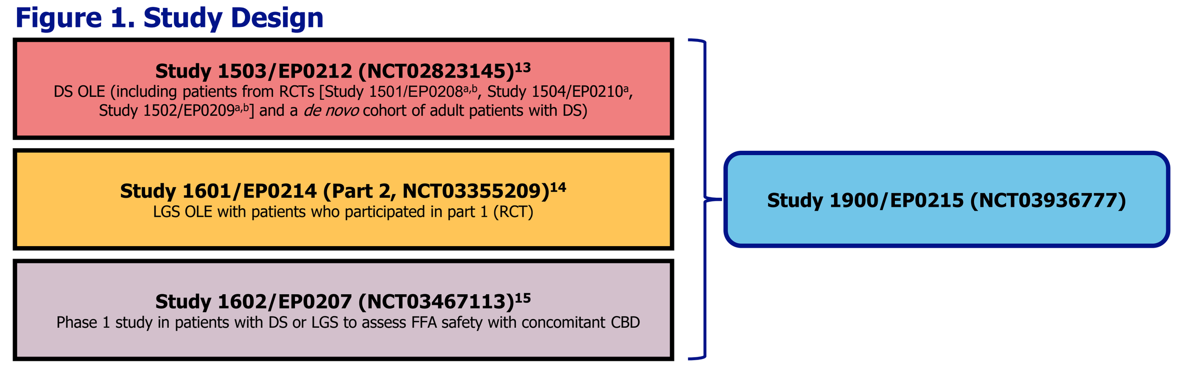
Objective

- Here we report the final long-term safety and global functioning analyses from an OLE study in pediatric and adult patients with DS or LGS treated with fenfluramine

Methods

- Patients who had participated in a prior fenfluramine open-label study were eligible to enroll in this international, multicenter OLE study (NCT03936777) (**Figure 1**)
- The latest fenfluramine dose from the previous study was continued; doses could then be flexibly titrated
  - Fenfluramine dose caps applied: maximum 0.7 mg/kg/day (26 mg/day) without stiripentol (STP) or 0.4 mg/kg/day (17 mg/day) if receiving concomitant STP
- ≥1 ASM was required to be given concomitantly with fenfluramine; dose changes in concomitant ASMs were permitted as clinically necessary
- The primary objective was to assess the long-term safety and tolerability of fenfluramine, including cardiovascular safety
  - Echocardiograms (ECHOs) were performed at baseline, then scheduled every 6 months (every 3 months in France) and required 6 months after the last fenfluramine dose
- A secondary objective was to evaluate patient global functioning using Clinical Global Impression–Improvement (CGI–I) ratings, globally and for specific domains, at last treatment visit relative to Clinical Global Impression–Severity (CGI–S) scores obtained at study baseline (entrance into this OLE) by caregiver and investigator
- Outcomes are presented by DS or LGS and by age group, where applicable; some age group analyses were conducted post hoc, as were analyses of mean daily dose and overall treatment exposure
- Descriptive statistics were used

Figure 1. Study Design



[a]Patients receiving any form of CBD were not eligible to participate in the DS RCTs.
[b]Concomitant stiripentol use not permitted in Study 1501/EP0208 and Study 1502/EP0209.
CBD, cannabidiol; DS, Dravet syndrome; FFA, fenfluramine; LGS, Lennox-Gastaut syndrome; OLE, open-label extension; RCT, randomized controlled trial.

Results

- A total of 412 patients were enrolled (DS: 265 [64.3%], LGS: 147 [35.7%]) and comprised the safety population; modified intent-to-treat (mITT) population comprised 410 patients
  - Respectively, 262 (63.6%) and 143 (34.7%) patients enrolled from Study 1503 (DS OLE)[13] and Study 1601 (LGS OLE)[14]
  - Of 412 enrolled patients, 285 (69.2%) were pediatric (2 to <18 years old) and 127 (30.8%) were adult (≥18 years old)
- Most baseline characteristics were balanced between the DEE diagnoses (**Table 1**)
  - A higher proportion of patients with LGS (37.4%) vs those with DS (27.2%) enrolled as adults
  - In both DEE groups, a median of 3.0 ASMs had been previously tried and then continued concomitantly with fenfluramine (DS: range, 0–7; LGS: range, 0–6)
    - Of the patients with DS, 143 (54.0%) previously tried 3–4 ASMs and 23 (8.7%) previously tried ≥5 ASMs; 97 (66.0%) patients with LGS had previously tried 3–4 ASMs and 20 (13.6%) had previously tried ≥5 ASMs

Table 1. Baseline Characteristics

	DS n=265		LGS n=147		ALL N=412	
Age group	2 to <18 y n=193	≥18 y n=72	2 to <18 y n=92	≥18 y n=55	2 to <18 y n=285	≥18 y n=127
Median age at enrollment in Study 1900, y (range)	10 (3–17)	20 (18–33)	10 (3–17)	22 (18–37)	10 (3–17)	21.0 (18–37)
Male sex, n (%)	109 (56.5)	35 (48.6)	48 (52.2)	29 (52.7)	157 (55.1)	64 (50.4)
Median weight, kg[a] (range)	29.9 (13.6–88.2)	56.5 (35.0–101.6)	31.8 (12.8–78.8)	57.3 (27.1–90.8)	30.5 (12.8–88.2)	57.1 (27.1–101.6)
Median number of prior and concomitant ASMs[b] (range)	3 (0–6)	3 (1–7)	3 (0–6)	3 (1–6)	3 (0–6)	3 (1–7)

[a]Weight available in 283/285 pediatric and 124/127 adult patients. [b]Prior and concomitant ASMs are those started before Visit 1 and still ongoing at Visit 1.
ASMs, antiseizure medications; DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome.

Overview

QUESTION

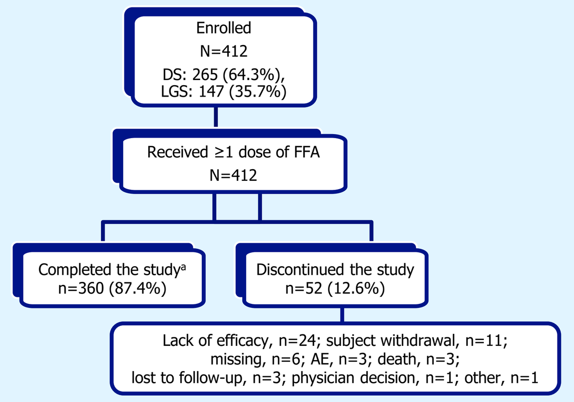
What are the long-term safety and global functioning outcomes in pediatric and adult patients with DS or LGS who continued treatment with FFA in an OLE study (NCT03936777) after participating in a previous open-label study?

INVESTIGATION

- Patients with DS and LGS were enrolled from:
  - Study 1503/EP0212 (DS OLE): NCT02823145
  - Study 1601/EP0214 (part 2, LGS OLE): NCT03355209
  - Study 1602/EP0207 (DS/LGS, phase 1 study assessing FFA safety with concomitant CBD)
- Initial FFA doses in this study were those continued from the previous study; patients received ≥1 concomitant ASM
- The primary objective was to assess long-term FFA safety and tolerability
- Secondary objectives included evaluation of patient global functioning via last visit CGI–I ratings relative to CGI–S at baseline (entrance) of this OLE

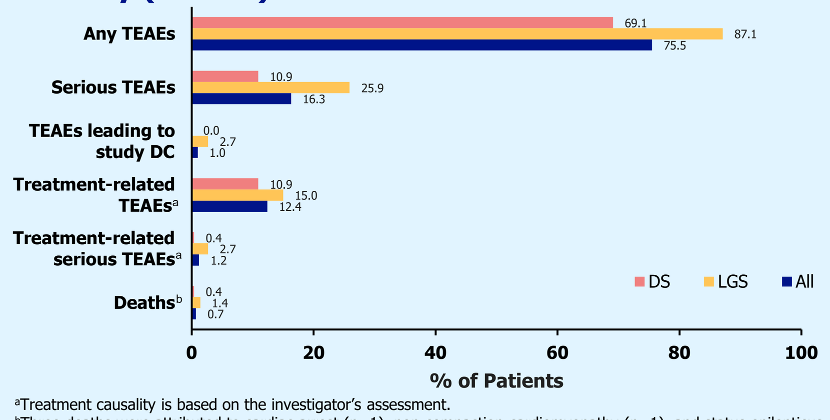
RESULTS

Patient Disposition



[a]"Completed the study" refers to completing 36 months of treatment (or 24 months in Denmark) or transitioning to commercial drug.

Safety (N=412)



[a]Treatment causality is based on the investigator's assessment.
[b]Three deaths were attributed to cardiac arrest (n=1), non-compaction cardiomyopathy (n=1), and status epilepticus (n=1); none were deemed by the investigator to be related to FFA.

Median FFA exposure, days (range)	Mean (SD) FFA daily dose, mg/kg/day
729.5 (8–1544)	0.5 (0.2)

- TEAEs reported in ≥10% of all patients included:
  - Coronavirus infection (85/412, 20.6%)
  - Seizure (63/412, 15.3%)
  - Pyrexia (54/412, 13.1%)
  - Nasopharyngitis (49/412, 11.9%)
- TEAEs were reported at similar rates among pediatric and adult groups
- There were no reports of VHD or PAH in this OLE study

Patient Global Functioning

	CGI–I Rating
1	Very much improved
2	Much improved
3	Minimally improved
4	No change
5	Minimally worse
6	Much worse
7	Very much worse

Ratings of 1, 2, or 3 are referred to as "Improved"

CGI–I Global Ratings of "Improved or No Change" (From Baseline CGI–S Ratings) at Last Treatment Visit

	Caregiver	Investigator
DS (n=264)	251/258 (97.3%)	251/258 (97.3%)
LGS (n=146)	122/143 (85.3%)	125/143 (87.4%)
All (N=410)	373/401 (93.0%)	376/401 (93.8%)

- Since patients entering this OLE study were already receiving FFA from a previous study, CGI–I ratings of 1–4 or "improved or no change" were reported to highlight improvement or stability in patient global functioning while on FFA treatment
- Proportions of patients with CGI–I global ratings of "improved or no change" (since entering this OLE) were similar among pediatric and adult groups by DS or LGS

CONCLUSIONS

- Consistent with prior studies, the results of this OLE study further affirm the continued clinical benefit of long-term FFA use in both pediatric and adult patient populations with DS or LGS.

Abbreviations: AE, adverse event; CGI–I, Clinical Global Impression–Improvement; CGI–S, Clinical Global Impression–Severity; DC, discontinuation; DS, Dravet syndrome; FFA, fenfluramine; LGS, Lennox-Gastaut syndrome; OLE, open label extension; PAH, pulmonary arterial hypertension; TEAEs, treatment-emergent adverse events; VHD, valvular heart disease.

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- The most common baseline global CGI–S ratings were "markedly ill" (113 [27.4%]) as assessed by parent/caregiver and "moderately ill" (141 [34.2%]) by investigator
- On CGI–S, more adults were rated "markedly ill", "severely ill", or "among the most extremely ill" by both caregiver and investigator (57.5%, 48.8%) vs pediatric patients (47.4%, 35.4%)
  - This is consistent with ratings for the CGI–S subdomains of cognition and behavior
- The most commonly used ASMs, started before entry into this OLE and continued concomitantly with fenfluramine while in the study, were valproate (all forms, n=280, 68.0%), clobazam (n=233, 56.6%), and diazepam (n=138, 33.5%)
  - At any point in this OLE study, 77/412 (18.7%) patients received concomitant CBD (any form), of whom 45/77 (58.4%) were those with LGS
- Of the 412 enrolled patients, 360 (87.4%) completed the study or transitioned to commercial product, and 52 (12.6%) discontinued the study (disposition by DEE and age group in **Table 2**)

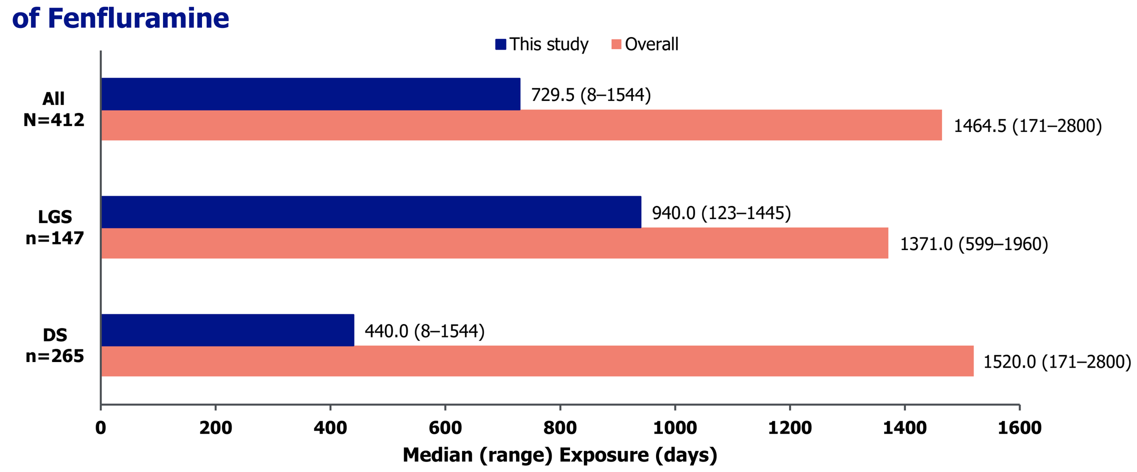
Table 2. Study Disposition, FFA Exposure, and FFA Daily Dose by DEE and Age Group

	DS n=265		LGS n=147		All N=412	
Age group	2 to <18 y n=193	≥18 y n=72	2 to <18 y n=92	≥18 y n=55	2 to <18 y n=285	≥18 y n=127
Completed the study[a], n (%)	186 (96.4)	71 (98.6)	65 (70.7)	38 (69.1)	251 (88.1)	109 (85.8)
Discontinued, n (%)	7 (3.6)	1 (1.4)	27 (29.3)	17 (30.9)	34 (11.9)	18 (14.2)
Reason for discontinuation, n (%)						
Adverse event	0	0	2 (2.2)	1 (1.8)	2 (0.7)	1 (0.8)
Death[b]	1 (0.5)	0	1 (1.1)	1 (1.8)	2 (0.7)	1 (0.8)
Lack of efficacy	4 (2.1)	0	13 (14.1)	7 (12.7)	17 (6.0)	7 (5.5)
Lost to follow-up	0	0	1 (1.1)	2 (3.6)	1 (0.4)	2 (1.6)
Physician decision	0	0	1 (1.1)	0	1 (0.4)	0
Subject withdrawal	1 (0.5)	1 (1.4)	5 (5.4)	4 (7.3)	6 (2.1)	5 (3.9)
Other	0	0	0	1 (1.8)	0	1 (0.8)
Missing[c]	1 (0.5)	0	4 (4.3)	1 (1.8)	5 (1.8)	1 (0.8)
Mean daily dose (SD), mg/kg/day	0.5 (0.2)	0.4 (0.1)	0.6 (0.1)	0.4 (0.1)	0.5 (0.2)	0.4 (0.1)
FFA exposure in this study, days Median (range)	449.0 (8–1544)	370.0 (182–1431)	993.0 (189–1443)	894.0 (123–1445)	730.0 (8–1544)	722.0 (123–1445)

[a]Completed means completed 36 months of treatment (24 months in Denmark) or transitioned to commercial fenfluramine. [b]3 deaths were attributed to cardiac arrest (n=1), non-compaction cardiomyopathy (n=1), and status epilepticus (n=1); none were deemed by the investigator to be related to FFA. [c]Six patients with "missing" disposition data were incorrectly reported as having completed the study but had not completed 36 months of treatment (or 24 months in Denmark), so have no reported reason for discontinuation.
DEE, developmental and epileptic encephalopathy; DS, Dravet syndrome; FFA, fenfluramine; LGS, Lennox-Gastaut syndrome; SD, standard deviation; y, years.

- Mean fenfluramine (SD) daily dose throughout this OLE study: 0.5 (0.2) mg/kg/day
  - In both DEE groups, mean daily dose was lower in adult (0.4 [0.1] mg/kg/day) vs pediatric patients (0.5 [0.2] mg/kg/day), **Table 2**
- Median fenfluramine exposure in this study was 2 years (729.5 days [range, 8–1544]; **Figure 2**)
  - Median exposure in patients with DS was 440.0 days (range, 8–1544) and in patients with LGS was 940.0 days (123–1445)
  - Exposure in either DEE was higher in pediatric patients (**Table 2**)

Figure 2. Treatment Exposure in This Study and Overall Exposure Since the Start of Fenfluramine



- Median overall exposure, including fenfluramine treatment in previous studies, was 1464.5 days (range, 171–2800), **Figure 2**
  - Median overall exposure in patients with DS was 1520.0 days (range, 171–2800) and in patients with LGS, 1371.0 days (range, 599–1960)

Table 3. Safety Summary by DEE and Age Group

	DS n=265		LGS n=147		All N=412	
Age group	2 to <18 y n=193	≥18 y n=72	2 to <18 y n=92	≥18 y n=55	2 to <18 y n=285	≥18 y n=127
Patients with any TEAEs, n (%)	134 (69.4)	49 (68.1)	80 (87.0)	48 (87.3)	214 (75.1)	97 (76.4)
Patients with serious TEAEs, n (%)	19 (9.8)	10 (13.9)	22 (23.9)	16 (29.1)	41 (14.4)	26 (20.5)
Patients with TEAEs leading to study DC[a], n (%)	0	0	2 (2.2)	2 (3.6)	2 (0.7)	2 (1.6)
Patients with treatment-related TEAEs[b], n (%)	20 (10.4)	9 (12.5)	14 (15.2)	8 (14.5)	34 (11.9)	17 (13.4)
Patients with treatment-related serious TEAEs[a], n (%)	0	1 (1.4)	1 (1.1)	3 (5.5)	1 (0.4)	4 (3.1)
Patients with TEAEs leading to death[c], n (%)	1 (0.5)	0	1 (1.1)	1 (1.8)	2 (0.7)	1 (0.8)
TEAEs reported in ≥10% of all patients, n (%)						
Coronavirus infection	30 (15.5)	13 (8.1)	20 (21.7)	22 (40.0)	50 (17.5)	35 (27.6)
Seizure	20 (10.4)	6 (8.3)	25 (27.2)	12 (21.8)	45 (15.8)	18 (14.2)
Pyrexia	30 (15.5)	9 (12.5)	9 (9.8)	6 (10.9)	39 (13.7)	15 (11.8)
Nasopharyngitis	27 (14.0)	7 (9.7)	10 (10.9)	5 (9.1)	37 (13.0)	12 (9.4)

[a]One patient did not have AE listed as the *primary* reason for discontinuation as noted in Table 2. [b]Treatment-related is based on the investigator's assessment. [c]Three deaths were attributed to cardiac arrest (n=1), non-compaction cardiomyopathy (n=1), and status epilepticus (n=1); none were deemed by the investigator to be related to FFA.
AE, adverse event; DC, discontinuation; DEE, developmental and epileptic encephalopathy; DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome; TEAE, treatment-emergent adverse event.

- Three patients discontinued the study due to an AE (**Table 2**); serious TEAEs were reported in 67 (16.3%) patients and 5 (1.2%) were deemed related to treatment by investigators (**Table 3**)
- Within each DEE, rates of TEAEs were comparable by age group
- At last visit, ≥7% and ≥10% reduction in body weight was reported in 22/390 (6/250 [DS], 16/140 [LGS]) and 12/390 (3/250 [DS], 9/140 [LGS]) patients, respectively
- There was no evidence of a clinically significant effect of fenfluramine on electrocardiogram parameters (QTcF) and there were no patients with pulmonary arterial hypertension (PAH; pulmonary arterial systolic pressure of >35mmHg)
- No cases of valvular heart disease (VHD) were reported
  - Three patients with mild aortic regurgitation (AR) met the definition for aortic valvulopathy; 2/3 patients had mild AR at study baseline (and at every ECHO recorded) and one patient (with trace AR at study baseline) had a single instance of transient mild AR at Month 18 of fenfluramine

Table 4. Proportion of Pediatric and Adult Patients with Last Visit CGI–I Global Ratings of "Improved or No Change" Relative to CGI–S at Baseline of This Study

	Age Group	Caregiver	Investigator
DS n=264	2 to <18 y, n=192	182/188 (96.8%)	182/188 (96.8%)
	≥18 y, n=72	69/70 (98.6%)	69/70 (98.6%)
LGS n=146	2 to <18 y, n=91	79/91 (86.8%)	82/91 (90.1%)
	≥18 y, n=55	43/52 (82.7%)	43/52 (82.7%)
All N=410	2 to <18 y, n=283	261/279 (93.5%)	264/279 (94.6%)
	≥18 y, n=127	112/122 (91.8%)	112/122 (91.8%)

CGI–I, Clinical Global Impression–Improvement; CGI–S, Clinical Global Impression–Severity; DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome.

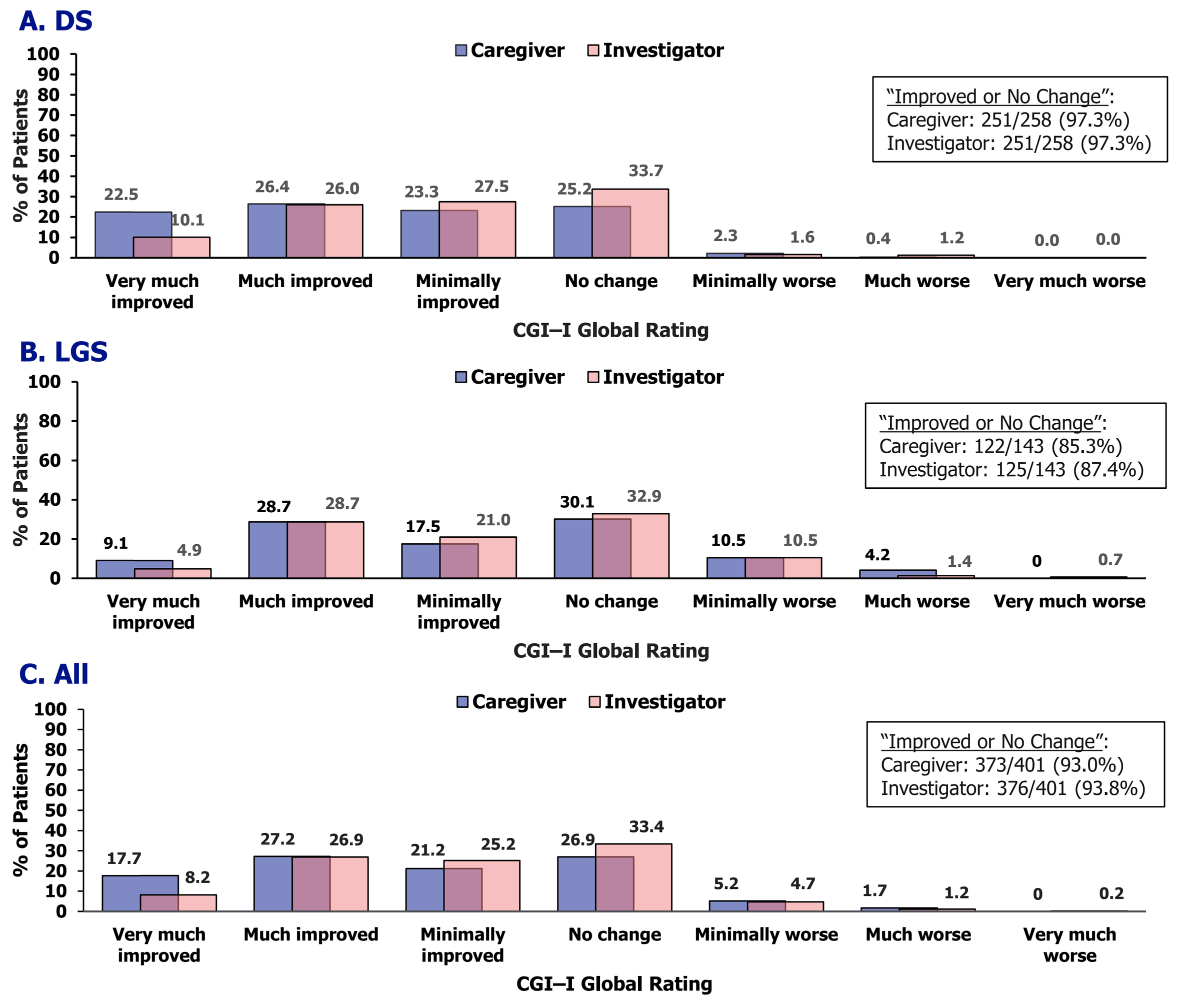
- On caregiver and investigator CGI–I global evaluation, most patients were rated as "improved or no change" relative to the rating at the entrance of this study (already on fenfluramine) (**Figure 3**); assessments by caregiver and investigator were generally aligned within the DEEs
- CGI–I global ratings of "improved or no change" between age groups within each DEE were similar (**Table 4**)
- Ratings of "improved or no change" by caregiver and investigator in CGI–I subdomains were generally comparable (**Table 5**)

Table 5. Proportion of Last Visit CGI–I Subdomain Ratings of "Improved or No Change" Relative to CGI–S at Baseline of This Study

		Caregiver	Investigator
DS n=264	Cognition	249/258 (96.5%)	250/256 (97.7%)
	Behavior	226/258 (87.6%)	242/256 (94.5%)
	Motor abilities	236/258 (91.5%)	246/256 (96.1%)
LGS n=146	Cognition	131/143 (91.6%)	134/143 (93.7%)
	Behavior	123/143 (86.0%)	132/143 (92.3%)
	Motor abilities	128/143 (89.5%)	132/143 (92.3%)
All N=410	Cognition	380/401 (94.8%)	384/399 (96.2%)
	Behavior	349/401 (87.0%)	374/399 (93.7%)
	Motor abilities	364/401 (90.8%)	378/399 (94.7%)

CGI–I, Clinical Global Impression–Improvement; CGI–S, Clinical Global Impression–Severity; DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome.

Figure 3. Last Visit CGI–I Global Ratings Relative to CGI–S at Baseline of This OLE Study by Caregiver/Investigator and DEE



CGI–I, Clinical Global Impression–Improvement; CGI–S, Clinical Global Impression–Severity; DEE, developmental and epileptic encephalopathy; DS, Dravet syndrome; LGS, Lennox-Gastaut syndrome; OLE, open-label extension.

Conclusions

- In this final OLE study analysis of pediatric and adult patients with DS or LGS treated with fenfluramine up to 4 years, TEAEs were consistent with the known safety profile of fenfluramine; no new or unexpected safety signals and no cases of VHD or PAH were reported
- Global functioning, as assessed by CGI–I ratings that encompass both seizure and non-seizure outcomes, was improved or stable in over 90% of all patients receiving fenfluramine treatment; this suggests a sustained clinical benefit continuing from fenfluramine exposure in the previous open-label studies
  - Clinically meaningful improvement (global CGI–I ratings of "very much improved" or "much improved") was observed in over one-third of patients
  - Global and subdomain CGI–I ratings by caregiver and investigator were generally consistent
- Within each DEE, safety outcomes and patient global functioning were generally comparable by age group
- Data from this OLE study (median fenfluramine exposure of 2 years) support the continued clinical benefit of fenfluramine and its positive risk/benefit profile in pediatric and adult patients with DS or LGS, as observed in prior studies

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