



Study 1601 Phase 3 Top-Line Results

February 6, 2020



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Committed to developing and commercializing transformative therapies to improve the lives of patients and their families living with rare diseases.

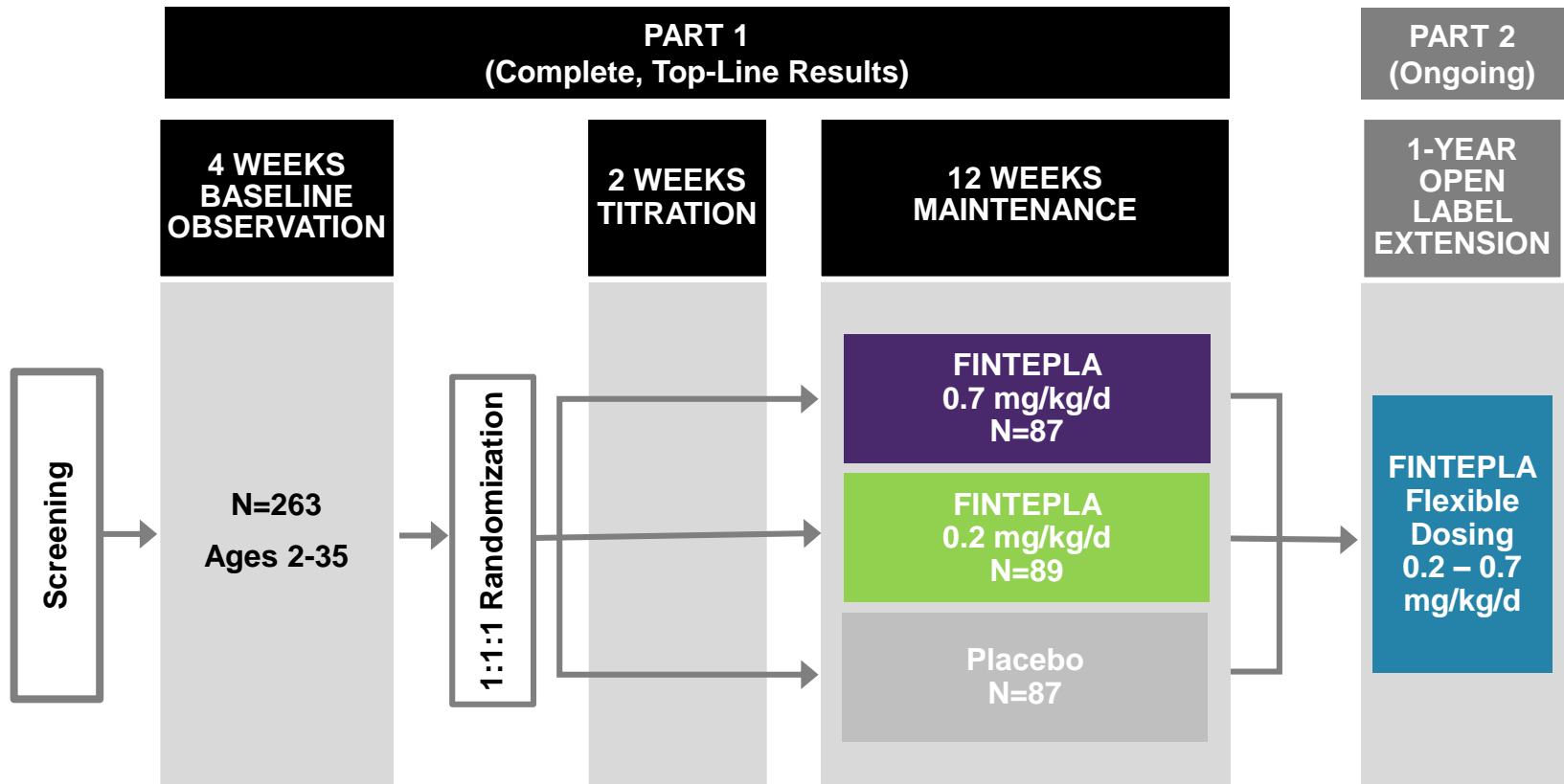
FINTEPLA Phase 3 Study 1601 Top-Line Results Lennox-Gastaut syndrome

Trial met primary endpoint of change in frequency of monthly drop seizures: FINTEPLA 0.7 mg/kg/day compared to placebo

Key secondary endpoint met: number of subjects with $\geq 50\%$ reduction in drop seizures: FINTEPLA 0.7 mg/kg/day group compared to placebo

FINTEPLA Phase 3 Study 1601 Design

Prospective, randomized, double-blind, placebo-controlled study of FINTEPLA in children and adults with LGS



Maximum Daily Dose of 26mg

Study 1601 Demographics

- Demographics were well balanced across dose groups
- The median baseline drop seizure frequency across all groups was 77 per 28 days
- Patients had previously received a median of 7 anti-epileptic drugs (AEDs) prior to entering the study

	Placebo (n=87)	FINTEPLA 0.2 mg/kg/d (n=89)	FINTEPLA 0.7* mg/kg/d (n=87)	All Patients (N=263)
Mean Age in years (SD) Median (min, max)	14.4 (7.71) 13.0 (2, 35)	13.4 (7.79) 13.0 (3, 35)	13.4 (7.28) 13.0 (2, 35)	13.7 (7.59) 13.0 (2, 35)
Age Group (years)				
2 - <6	9 (10.3%)	17 (19.1%)	12 (13.8%)	38 (14.4%)
6 - <12	23 (26.4%)	24 (27.0%)	25 (28.7%)	72 (27.4%)
12 - <18	29 (33.3%)	23 (25.8%)	25 (28.7%)	77 (29.3%)
18 - 35	26 (29.9%)	25 (28.1%)	25 (28.7%)	76 (28.9%)
% Female	47.1%	48.3%	37.9%	44.5%
US	47.1%	47.2%	44.8%	46.4%
Europe**	47.1%	48.3%	43.7%	46.4%
Mean Weight (kg) Median	43.9 38.7	42.4 41.0	42.4 39.0	42.8 39.0

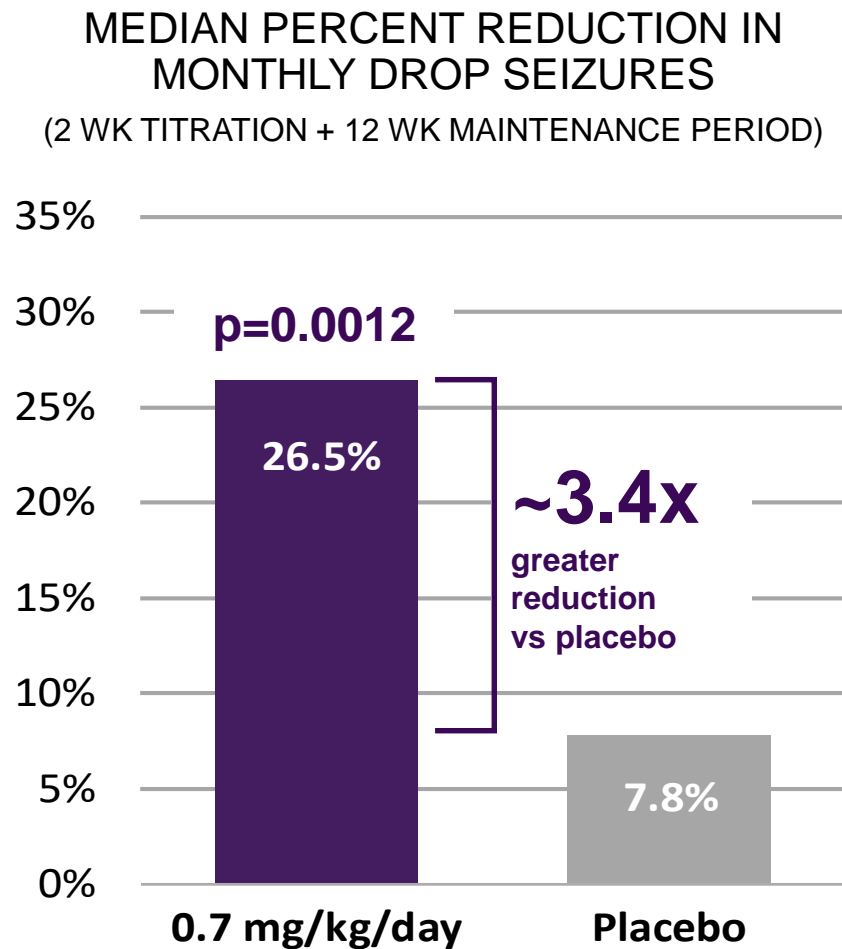
*0.7 mg/kg/day of fenfluramine base is equivalent to 0.8 mg/kg/day of fenfluramine hydrochloride salt

**Patients were also enrolled from Canada, Australia, and Mexico

Study 1601 Met Primary Efficacy Endpoint

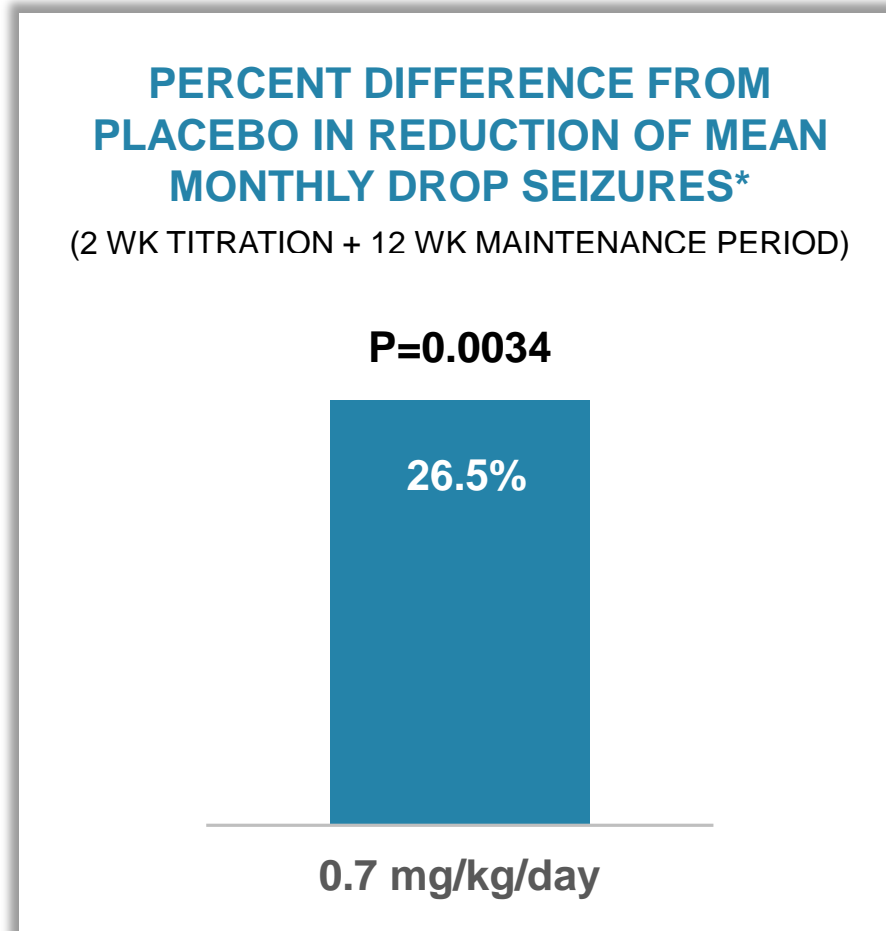
Study 1601 met primary endpoint demonstrating FINTEPLA, at a dose of 0.7 mg/kg/day, was superior to placebo as adjunctive therapy in the treatment of LGS based on median percent change in monthly frequency of seizures that result in drops ($p=0.0012$)

*FINTEPLA 0.7 mg/kg/day dose provided **~3.4x** greater median percent reduction in monthly drop seizures versus placebo*



p-value is treatment compared with placebo

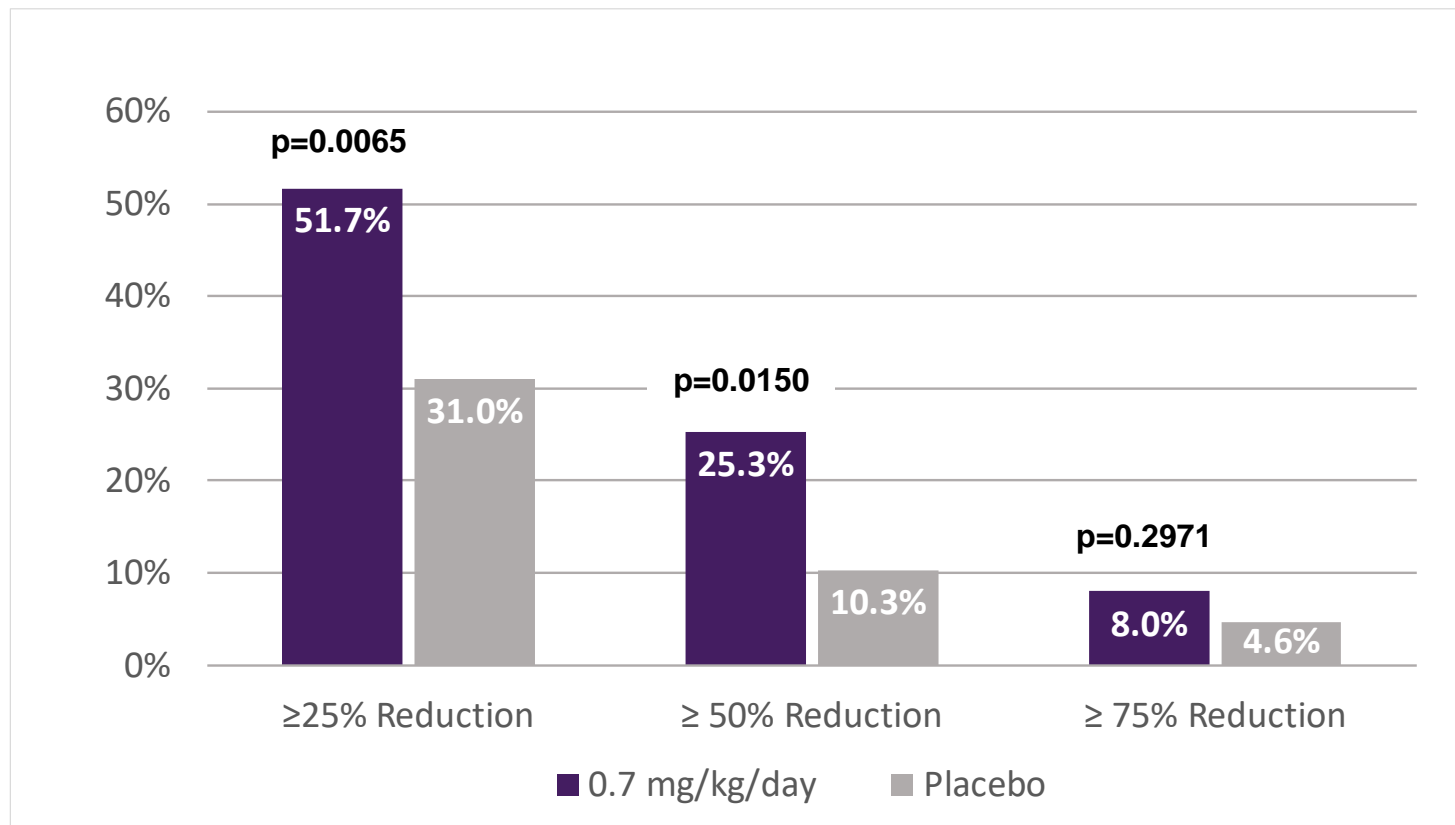
Study 1601 Mean Percent Seizure Reduction vs. Placebo



* Estimate based on a parametric analysis of a baseline-adjusted change in drop seizure frequency from baseline, 0.7 mg/kg/day vs. placebo
p-value is treatment compared with placebo group

Study 1601 Drop Seizure Responder Rates

PROPORTION OF PATIENTS WHO ACHIEVED $\geq 25\%$, $\geq 50\%$ AND $\geq 75\%$
REDUCTION IN MEAN MONTHLY DROP SEIZURES
(2 WK TITRATION + 12 WK MAINTENANCE PERIOD)

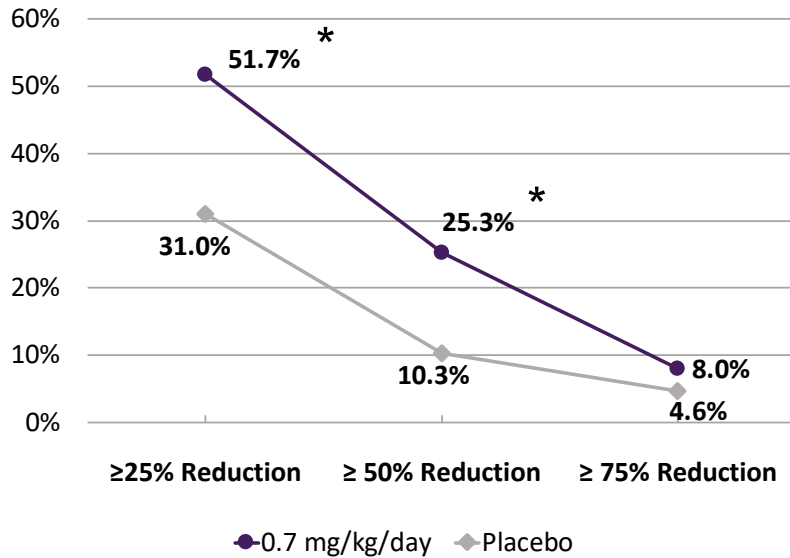


p-values are treatment compared with placebo
Comparisons based on a logistic regression model

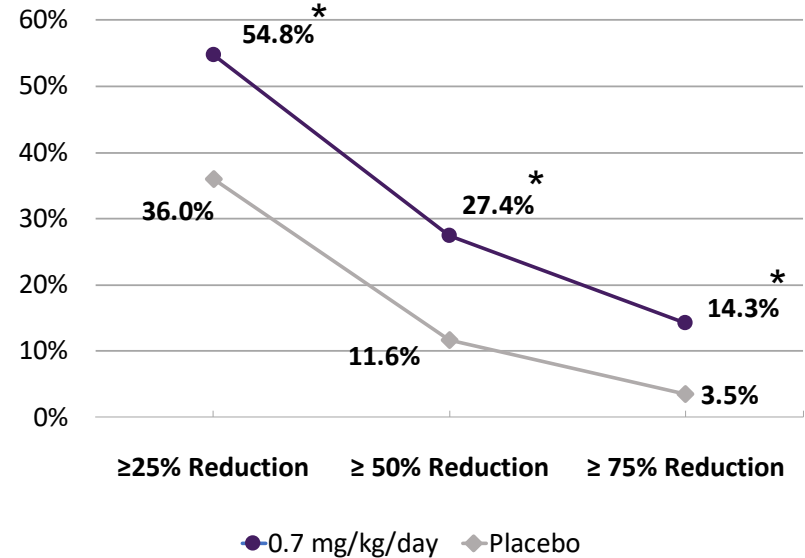
Study 1601 Maintenance Period Responder Rates

PROPORTION OF PATIENTS WHO ACHIEVED $\geq 25\%$, $\geq 50\%$, AND $\geq 75\%$ REDUCTION IN MEAN MONTHLY DROP SEIZURES MAINTENANCE COMPARED TO TITRATION + MAINTENANCE

Titration + Maintenance
(week 1-14)



Maintenance
(week 3-14)



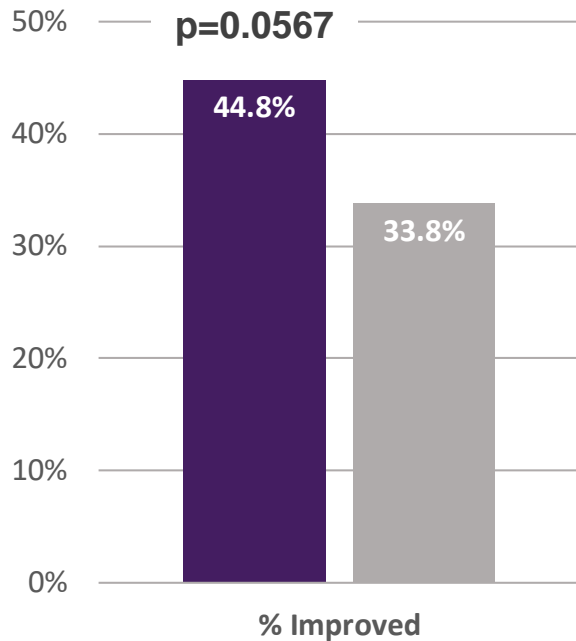
* $p < 0.05$

p-values are treatment compared with placebo

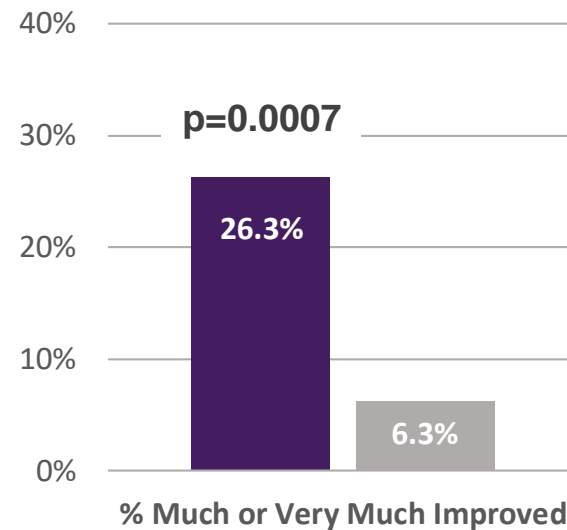
Study 1601 Clinical Global Impression - Improvement

PROPORTION OF PATIENTS WITH IMPROVEMENT IN CGI-I INVESTIGATOR RATING
(BASELINE VERSUS 2 WK TITRATION + 12 WK MAINTENANCE PERIOD)

PROPORTION OF PATIENTS
[1] Very Much Improved, [2] Much Improved
or [3] Minimally Improved



PROPORTION OF PATIENTS
[1] Very Much Improved or
[2] Much Improved

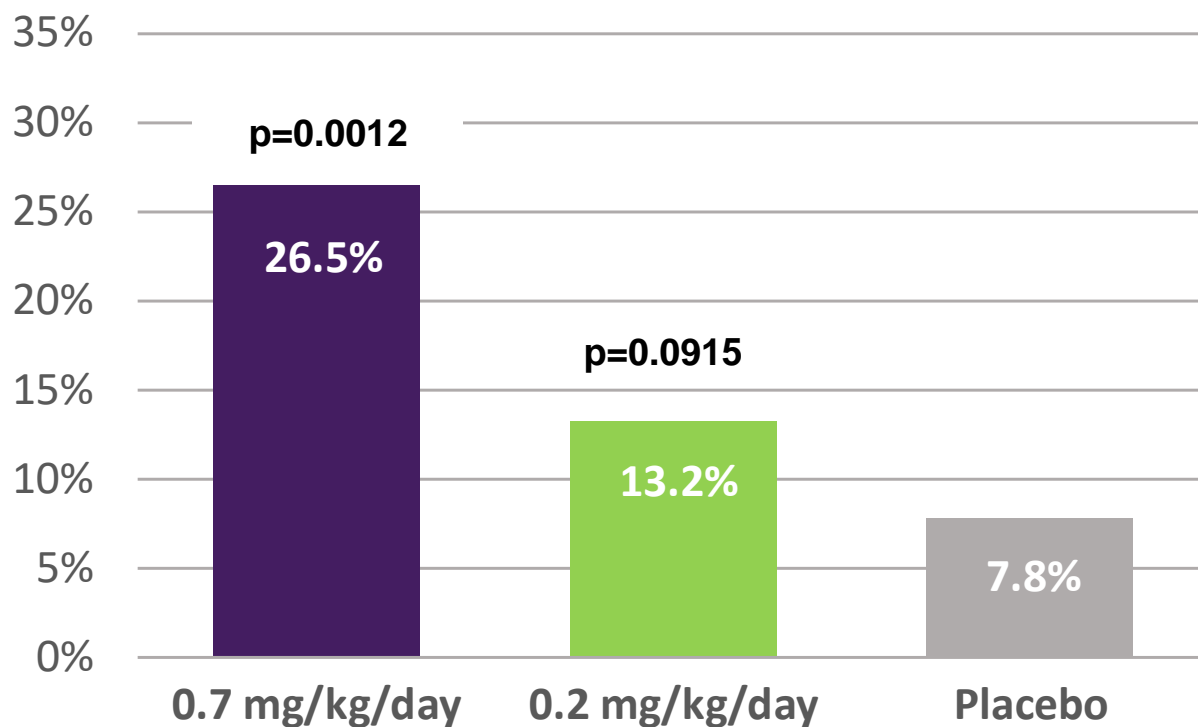


0.7 mg/kg/day Placebo

Study 1601 Median Drop Seizure Reduction - All Doses

MEDIAN PERCENT REDUCTION IN MONTHLY DROP SEIZURES

(2 WK TITRATION + 12 WK MAINTENANCE PERIOD)



p-values are treatment compared with placebo

Study 1601 Safety Results

- Generally well-tolerated with adverse events consistent with known safety profile of fenfluramine
- No cases of valvular heart disease or pulmonary arterial hypertension
- Six patients in the 0.7/mg/kg/d group had an AE that led to a discontinuation compared to four patients in the 0.2/mg/kg/d group and one patient in the placebo group
- One death during the trial caused by SUDEP in a patient in the 0.7/mg/kg/d/group assessed to be unrelated to study drug

	Placebo (n=87)	0.2 mg/kg/d (n=89)	0.7 mg/kg/d (n=87)
Number of Subjects With at Least One Treatment Emergent Adverse Event (AE)	79.3%	76.4%	89.7%
Number of Subjects With at Least One Serious Treatment Emergent Adverse Event (SAE)	4.6%	4.5%	11.5%
Most Common Adverse Events (>10%)	Placebo (n=87)	0.2 mg/kg/d (n=89)	0.7 mg/kg/d (n=87)
Decreased Appetite	13 (14.9%)	18 (20.2%)	32 (36.8%)
Diarrhea	4 (4.6%)	10 (11.2%)	11 (12.6%)
Vomiting	5 (5.7%)	12 (13.5%)	9 (10.3%)
Fatigue	11 (12.6%)	8 (9.0%)	16 (18.4%)
Pyrexia	11 (12.6%)	11 (12.4%)	9 (10.3%)
Seizure	5 (5.7%)	11 (12.4%)	5 (5.7%)
Somnolence	10 (11.5%)	10 (11.2%)	15 (17.2%)



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