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Original article

Evaluation of long-term safety, tolerability, and behavioral outcomes with adjunctive rufinamide in pediatric patients (≥1 to <4 years old) with Lennox-Gastaut syndrome: Final results from randomized study 303

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ABSTRACT

Objective: Evaluate the long-term safety, tolerability, and behavioral effects of adjunctive rufinamide in pediatric patients (≥1 to <4 years old) with inadequately controlled seizures associated with Lennox-Gastaut syndrome (LGS).

Methods: Study 303 (ClinicalTrials.gov identifier NCT01405053) was a multicenter, randomized, open-label, Phase III trial. Patients were randomized (2:1) to oral suspension rufinamide (≤45 mg/kg/day) or any other investigator-chosen antiepileptic drug (AED) for a 2-year treatment period. Primary safety/tolerability assessments included monitoring of treatment-emergent adverse events (TEAEs) and serious TEAEs. Behavioral effects were assessed via the Child Behavior Checklist (CBCL) using the Total Problems score and change from baseline in CBCL Total Problems score. CBCL subscores were also evaluated.

Results: The Safety Analysis Set included 37 patients (rufinamide: $n = 25$; any other AED: $n = 12$). TEAE incidence was similar between the rufinamide (88.0%) and any-other-AED groups (83.3%); serious TEAE incidence was also similar between treatment groups

Abbreviations: AE, adverse event; AED, antiepileptic drug; CBCL, child behavior checklist; ECG, electrocardiogram; EEG, electroencephalogram; EXC, exclusion; INC, inclusion; LDS, language development survey; LGS, Lennox-Gastaut syndrome; max, maximum; min, minimum; PK, pharmacokinetic; QoLCE, quality of life in childhood epilepsy; SD, standard deviation; TEAE, treatment-emergent adverse event.

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