AAN 74th ANNUAL MEETING ABSTRACT

Media Contacts:
Renee Tessman, rtessman@aan.com, (612) 928-6137
Natalie Conrad, nconrad@aan.com, (612) 928-6164

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Abstract Title: Ecopipam in Children and Adolescents with Tourette Syndrome: Results from a Randomized, Double-Blind, Placebo-Controlled Phase 2b Study

Press Release Title: Experimental Drug Linked to Reduced Tics in Children, Teens with Tourette Syndrome: Study Suggests Medication Reduces Tics by 30%

Authors: Atul Mahableshwarkar[1], Jordan Dubow[2], Timothy Cuniff[2], Stephen Wanaski[2], Sarah Atkinson[3], David Kim[1], Meredith Miller[1], Donald Gilbert[4]

[1]Emalex Biosciences, [2]Paragon Biosciences, [3]ERG Clinical, [4]Cincinnati Children's Hospital Med. Ctr.

Objective: Evaluate the efficacy and safety of ecopipam in children and adolescents with Tourette syndrome (TS).

Background: Ecopipam, a first-in-class selective dopamine-1 (D1) receptor antagonist, is in clinical development for pediatric patients with TS. In prior Phase 2 studies in TS, ecopipam reduced tics in children and adults and demonstrated a history of low metabolic and movement-related adverse events.

Design/Methods: Patients with TS were randomized 1:1 to ecopipam or placebo for a 4-week titration, an 8-week maintenance, and a 1-week tapering period. The primary endpoint was mean change from Baseline (BL) to Week 12 for the Yale Global Tic Severity Score - Total Tic Score (YGTSS-TTS). Clinical Global Impression of Tourette Syndrome Severity (CGI-TS-S) was the key secondary endpoint. Safety and tolerability were evaluated at each study visit.

Results: 153 patients were randomized and 149 included in the modified intent-to-treat population (74 ecopipam, 75 placebo). A significant improvement (LS mean [SE] difference: -3.44 [1.35], 95% CI: -6.09, -0.79, p=0.011) in the YGTSS-TTS from BL to Week 12 was observed for ecopipam vs. placebo (30% reduction from BL to Week 12, effect size = 0.48). Similar results were observed among those ages 6 to 11 years (LS mean [SE] difference: -4.95 [2.50], 95% CI: -9.99, 0.10, p=0.054) and those ages 12 to 17 years (LS mean [SE] difference: -3.37 [1.58], 95% CI: -6.51, -0.24, p=0.035). Mean change from BL to Week 12 was significant for CGI-TS-S (p=0.001). Treatment-related AEs occurred in 26 (34%) patients with ecopipam and 16 (21%) with placebo, most commonly headache (9.2%), fatigue (6.6%), somnolence (6.6%), and restlessness (5.3%). No metabolic or movement-related AEs or treatment-related serious AEs occurred.

Conclusions: Among children and adolescents with TS, ecopipam significantly reduced motor and phonic tics with similar results between age groups and was safe and well-tolerated.

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