Aim: This phase III, randomized, double-blind, active-comparator trial (NCT01362530) evaluated the safety and efficacy of aprepitant for the prevention of chemotherapy-induced nausea and vomiting (CINV) in children.


Methods: Patients 12-17 years (y) old undergoing highly/moderately emetogenic (guideline based) chemotherapy (chemo) received aprepitant capsule 125 mg + ondansetron before chemo (Day 1) + aprepitant capsule 80 mg (Days 2-3), OR placebo (Days 1-3) + ondansetron (Day 1). Patients <12 y old received aprepitant powder-for-suspension (PFS) 3 mg/kg (up to 125 mg) + ondansetron (Day 1) + aprepitant PFS 2 mg/kg (up to 80 mg) (Days 2-3), OR placebo (Days 1-3) + ondansetron (Day 1). Dexamethasone was added at the investigator’s discretion. Aprepitant and control regimens were compared for complete response (CR: no vomiting/retching/rescue medication) 25-120 hours (h) after chemo initiation (delayed phase; primary objective). The current analysis examined the CR in each phase (acute [0-24 h], delayed, overall [0-120 h]) across various subgroups including age, dexamethasone use, and use of very highly emetogenic chemo (VHEC).

Results: A total of 302 patients participated (ages 6 months to <2 y, n = 35; 2 to <6 y, n = 88; 6 to <12 y, n = 84; and 12 to 17 y, n = 95); 152 received the aprepitant regimen and 150 received control. During the delayed phase, the proportion of patients achieving CR varied across age groups within each regimen but was higher for aprepitant (range, 46.3%-55.6%) vs control (range, 10.4%-37.2%). A greater proportion of patients achieved CR with aprepitant vs control regardless of dexamethasone use (yes, 36.4% vs 21.4%; no, 56.5% vs 27.8%). In addition, CR rate was higher for aprepitant vs control in those receiving VHEC (42.4% vs 19.8%). Similar patterns were observed during the acute and overall phases. The aprepitant regimen was well tolerated; adverse events were similar between regimens and consistent with those in patients undergoing chemo.

Conclusions: The addition of aprepitant to a 5HT3-antagonist (± dexamethasone) as part of a 3-day regimen was well tolerated and effective for reducing CINV in pediatric patients with cancer, regardless of age, dexamethasone use, and use of VHEC.